

# NOVELION THERAPEUTICS INC.

## FORM 10-K (Annual Report)

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UNITED STATES SECURITIES AND EXCHANGE COMMISSION  
WASHINGTON, DC 20549

FORM 10-K

(Mark One)

- ANNUAL REPORT PURSUANT TO SECTION 13 OR 15 (d) OF THE SECURITIES EXCHANGE ACT OF 1934  
For the fiscal year ended December 31, 2017
- TRANSITION REPORT PURSUANT TO SECTION 13 OR 15 (d) OF THE SECURITIES EXCHANGE ACT OF 1934  
For the transition period from \_\_\_\_\_ to \_\_\_\_\_

Commission File Number: 000-17082

**Novelion Therapeutics Inc.**

(Exact Name of Registrant as Specified in Its Charter)

British Columbia, Canada  
(State or Other Jurisdiction of  
Incorporation or Organization)

98-0455702  
(IRS Employer  
Identification Number)

c/o Norton Rose Fulbright  
1800 - 510 West Georgia Street, Vancouver, BC V6B 0M3 Canada

(Address of Principal Executive Offices, including Zip Code)

(877) 764-3131

(Registrant's telephone number, including area code)

SECURITIES REGISTERED PURSUANT TO SECTION 12(b) OF THE ACT:

<u>Title of each class</u>	<u>Name of each exchange on which registered</u>
Common shares, without par value	The NASDAQ Global Select Market

SECURITIES REGISTERED PURSUANT TO SECTION 12(g) OF THE ACT:

None

Indicate by check mark if the registrant is a well-known seasoned issuer, as defined in Rule 405 of the Securities Act. Yes  No

Indicate by check mark if the registrant is not required to file reports pursuant to Section 13 or 15(d) of the Act. Yes  No

Indicate by check mark whether the registrant (1) has filed all reports required to be filed by Section 13 or 15(d) of the Securities Exchange Act of 1934 during the preceding 12 months (or for such shorter period that the registrant was required to file such reports), and (2) has been subject to such filing requirements for the past 90 days. Yes  No

Indicate by check mark whether the registrant has submitted electronically and posted on its corporate Website, if any, every Interactive Data File required to be submitted and posted pursuant to Rule 405 of Regulation S-T (§ 232.405 of this chapter) during the preceding 12 months (or for such shorter period that the registrant was required to submit and post such files). Yes  No

Indicate by check mark if disclosure of delinquent filers pursuant to Item 405 of Regulation S-K (§ 229.405 of this chapter) is not contained herein, and will not be contained, to the best of registrant's knowledge, in definitive proxy or information statements incorporated by reference in Part III of this Form 10-K or any amendment to this Form 10-K.

Indicate by check mark whether the registrant is a large accelerated filer, an accelerated filer, a non-accelerated filer, a smaller reporting company or emerging growth company. See the definitions of "large accelerated filer," "accelerated filer," "smaller reporting company," and "emerging growth company" in Rule 12b-2 of the Exchange Act.

Large accelerated filer	<input type="checkbox"/>	Accelerated filer	<input checked="" type="checkbox"/>
Non-accelerated filer	<input type="checkbox"/> (Do not check if a smaller reporting company)	Smaller reporting company	<input type="checkbox"/>
Emerging growth company	<input type="checkbox"/>		

If an emerging growth company, indicate by check mark if the registrant has elected not to use the extended transition period for complying with any new or revised financial accounting standards provided pursuant to Section 13(a) of the Exchange Act.

Indicate by check mark whether the registrant is a shell company (as defined in Rule 12b-2 of the Exchange Act). Yes  No

The aggregate market value of the voting and non-voting common equity held by non-affiliates of the registrant as of June 30, 2017 was approximately \$149,963,502, based upon the closing price on the NASDAQ Select Global Market reported for such date.

The number of shares outstanding of the registrant's Common Stock as of March 12, 2018 was 18,703,204.

### **DOCUMENTS INCORPORATED BY REFERENCE**

The registrant intends to file a definitive Proxy Statement for its 2018 Annual Meeting of Shareholders no later than April 30, 2018. Portions of such definitive Proxy Statement are incorporated by reference into Part III of this Annual Report on Form 10-K (Annual Report) in accordance with instruction G(3) to Form 10-K.

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**FORM 10-K**  
**TABLE OF CONTENTS**

**PART I**

Item 1.	<a href="#">Business</a>	<a href="#">2</a>
Item 1A.	<a href="#">Risk Factors</a>	<a href="#">45</a>
Item 1B.	<a href="#">Unresolved Staff Comments</a>	<a href="#">87</a>
Item 2.	<a href="#">Properties</a>	<a href="#">87</a>
Item 3.	<a href="#">Legal Proceedings</a>	<a href="#">87</a>
Item 4.	<a href="#">Mine Safety Disclosures</a>	<a href="#">90</a>

**PART II**

Item 5.	<a href="#">Market for Registrant’s Common Equity, Related Shareholder Matters and Issuer Purchases of Equity Securities</a>	<a href="#">91</a>
Item 6.	<a href="#">Selected Financial Data</a>	<a href="#">93</a>
Item 7.	<a href="#">Management’s Discussion and Analysis of Financial Condition and Results of Operations</a>	<a href="#">96</a>
Item 7A.	<a href="#">Quantitative and Qualitative Disclosures About Market Risk</a>	<a href="#">114</a>
Item 8.	<a href="#">Consolidated Financial Statements and Supplementary Data</a>	<a href="#">116</a>
Item 9.	<a href="#">Changes in and Disagreements With Accountants on Accounting and Financial Disclosure</a>	<a href="#">154</a>
Item 9A.	<a href="#">Controls and Procedures</a>	<a href="#">154</a>
Item 9B.	<a href="#">Other Information</a>	<a href="#">155</a>

**PART III**

Item 10.	<a href="#">Directors, Executive Officers and Corporate Governance</a>	<a href="#">157</a>
Item 11.	<a href="#">Executive Compensation</a>	<a href="#">157</a>
Item 12.	<a href="#">Security Ownership of Certain Beneficial Owners and Management and Related Shareholder Matters</a>	<a href="#">157</a>
Item 13.	<a href="#">Certain Relationships and Related Transactions, and Director Independence</a>	<a href="#">157</a>
Item 14.	<a href="#">Principal Accounting Fees and Services</a>	<a href="#">157</a>

**PART IV**

Item 15.	<a href="#">Exhibits, Financial Statement Schedules</a>	<a href="#">158</a>
Item 16.	<a href="#">Form 10-K Summary</a>	<a href="#">158</a>
	<a href="#">Signatures</a>	<a href="#">164</a>

## Background

On November 29, 2016, Novelson Therapeutics Inc. ("Novelson") (formerly known as "QLT Inc." or "QLT") completed its acquisition of Aegerion Pharmaceuticals, Inc. ("Aegerion"), through the merger ("the Merger") of its indirect, wholly-owned subsidiary Isotope Acquisition Corp. ("MergerCo") with and into Aegerion, pursuant to an Agreement and Plan of Merger (as amended, the "Merger Agreement"), dated as of June 14, 2016, among Novelson, Aegerion and MergerCo. As a result of the Merger, Aegerion became an indirect wholly-owned subsidiary of Novelson. The former stockholders of Aegerion received shares of Novelson as consideration in connection with the Merger. As of November 29, 2016, after giving effect to the Merger, the pre-Merger shareholders of QLT collectively owned approximately 68% and the pre-Merger stockholders of Aegerion owned approximately 32% of our outstanding common shares.

The Merger has been accounted for as a business combination in which Novelson was considered the acquirer of Aegerion. As such, the consolidated financial statements of Novelson are treated as the historical financial statements of the combined companies, with the results of Aegerion being included beginning on November 29, 2016.

For periods prior to the closing of the Merger, the discussion in this Annual Report relates solely to the historical business and operations of QLT. Certain portions of this Annual Report may contain information that may no longer be material to our business related to Aegerion's historical operations. Any comparison of pre-Merger Aegerion revenues and operations with ours may not be helpful to an understanding of our results for the fiscal year ended December 31, 2017 or future periods.

All references in this Annual Report to "we," "us," "our," the "Company," "QLT" and "Novelson" refer to Novelson and its consolidated subsidiaries. For periods following the closing of the Merger, such references include Aegerion. As described more fully in this Annual Report, following the Merger, Novelson holds the rights to zuretinol and engages in other activities, as set forth herein, Aegerion continues to develop and commercialize lomitapide and metrelptin, and each maintains its respective ownership of or licenses covering intellectual property related to such products and remains party to the regulatory filings and approvals for such products.

## Trademarks

Novelson<sup>®</sup>, Aegerion<sup>®</sup>, JUXTAPID<sup>®</sup>, LOJUXTA<sup>®</sup>, MYALEPT<sup>®</sup> and MYALEPTA<sup>®</sup> are registered trademarks of Novelson or Aegerion. All other trademarks referenced in this Annual Report are the property of their respective owners.

## PART I

**The following discussion contains forward-looking statements. Actual results may differ significantly from those expressed or implied in the forward-looking statements. See "Cautionary Note Concerning Forward-Looking Statements" in "Item 7 - Management's Discussion and Analysis of Financial Condition and Results of Operations" of this Annual Report. Factors that might cause future results to differ materially from those expressed or implied in the forward-looking statements include, but are not limited to, those discussed in "Item 1A-Risk Factors" and elsewhere in this Annual Report.**

### Item 1. Business.

#### Overview

We are a biopharmaceutical company dedicated to developing new standards of care for individuals living with rare diseases. We, through Aegerion, have two commercial products:

- Lomitapide is marketed in the United States ("U.S.") under the brand name JUXTAPID (lomitapide) capsules ("JUXTAPID"). JUXTAPID is approved in the U.S. as an adjunct to a low-fat diet and other lipid-lowering treatments, including low-density lipoprotein ("LDL") apheresis where available, to reduce low-density lipoprotein cholesterol ("LDL-C"), total cholesterol ("TC"), apolipoprotein B ("apo B") and non-high-density lipoprotein cholesterol ("non-HDL-C") in adult patients with homozygous familial hypercholesterolemia ("HoFH"). Lomitapide is approved in the European Union ("EU"), under the brand name LOJUXTA (lomitapide) hard capsules ("LOJUXTA") for the treatment of adult patients with HoFH, as well as in Japan, Canada, and a limited number of other countries. In December 2016, Aegerion launched JUXTAPID as a treatment for HoFH in Japan. Aegerion receives sales milestones and royalties on net sales of LOJUXTA in the EU and certain other jurisdictions from Amryt Pharma plc ("Amryt"), to whom Aegerion out-licensed the rights to commercialize LOJUXTA in those jurisdictions in December 2016. Lomitapide is also sold, on a named patient sales basis, in Brazil and in a limited number of other countries outside the U.S. where such sales are

permitted before regulatory approval in such country as a result of the approval of lomitapide in the U.S. or the EU. We plan to file for regulatory approval for lomitapide for the treatment of HoFH in Brazil in 2018.

- Metreleptin, a recombinant analog of human leptin, is marketed in the U.S. under the brand name MYALEPT (metreleptin) for injection ("MYALEPT"). MYALEPT is approved in the U.S. as an adjunct to diet as replacement therapy to treat the complications of leptin deficiency in patients with congenital or acquired generalized lipodystrophy ("GL"). In December 2016, we submitted a marketing authorization application ("MAA") to the European Medicines Agency ("EMA") to seek approval for metreleptin in the EU, under the brand name MYALEPTA, as replacement therapy to treat complications of leptin deficiency in patients with GL and in a subset of patients with partial lipodystrophy ("PL"). In February 2018, we presented at an oral hearing of the EMA's Committee for Medicinal Products for Human Use ("CHMP"). Based on the CHMP's feedback at and after the hearing, we have requested a two-month "clock stop" of the CHMP's review to allow for the preparation of responses and additional data focused, primarily, on the PL patient subset. Subject to our ability to address the CHMP's feedback during the "clock stop" period, we anticipate that the CHMP will issue its opinion in the second quarter of 2018 and the European Commission ("EC") will issue its decision in mid-2018. We plan to file for regulatory approvals for metreleptin in GL and, subject largely to whether we receive EMA approval of any PL subset indication, such PL subset in other key markets, including Brazil in late 2018. We offer metreleptin through expanded access programs in countries where permitted by applicable regulatory authorities and under applicable laws, and generate revenues in certain markets where named patient sales are permitted based on the approval of metreleptin in the U.S. We plan to initiate, by late 2018, a phase 2 trial assessing metreleptin in hypoleptinemic metabolic disorder ("HMD"), a low leptin mediated metabolic disease, subject to approval of our protocol and statistical plan by applicable regulatory authorities. We also plan to continue to explore new opportunities for metreleptin to treat certain other low-leptin mediated metabolic diseases, and are reviewing options for raising capital to fund such opportunities, along with later-stage studies in HMD, upon which such opportunities are largely dependent.

We also have one product candidate, zuretinol acetate ("zuretinol"), an oral synthetic retinoid in development for the treatment of inherited retinal disease ("IRD") caused by underlying mutations in retinal pigment epithelium protein 65 ("RPE65") and lecithin: retinol acyltransferase ("LRAT") genes, comprising Leber Congenital Amaurosis ("LCA") and Retinitis Pigmentosa ("RP"). Following a comprehensive pipeline review, we are evaluating options for out-licensing zuretinol.

During the year ended December 31, 2017, net revenues from lomitapide and metreleptin were \$138.4 million, of which \$97.4 million was derived from prescriptions for lomitapide and metreleptin written in the U.S., and \$41.0 million was derived from prescriptions written for and royalties on sales of lomitapide and metreleptin outside the U.S. As of December 31, 2017, we had approximately \$55.4 million in cash and cash equivalents. We have approximately \$325.0 million principal amount of 2.0% convertible senior notes due August 15, 2019 (the "Convertible Notes"). To resolve certain Department of Justice ("DOJ") and Securities and Exchange Commission ("SEC") investigations regarding Aegerion's U.S. commercial activities and disclosures related to JUXTAPID, Aegerion is, among other obligations, required to pay approximately \$40.1 million in civil penalties, restitution and settlement amounts (plus interest) over three years. See the "Management's Discussion and Analysis of Financial Condition and Results of Operations - Liquidity and Capital Resources" section and the "Legal Proceedings" section of this Annual Report for further information.

In the near-term, we expect that the majority of revenues will continue to be derived from sales of JUXTAPID and MYALEPT in the U.S. We also expect to generate revenues from (i) sales in those countries outside the U.S. in which we have or expect to receive marketing approval, are able to obtain pricing and reimbursement approval at acceptable levels, and elect to commercialize the products, and (ii) sales of both products in a limited number of other countries where they are, or may in the future be, available on a named patient sales basis as a result of approvals in the U.S. or EU. We expect that in the near-term, our largest sources of revenues after the U.S., on a country-by-country basis, will be, sales of JUXTAPID in Japan and named patient sales of both of our products in Brazil. We have had, and expect to continue to have, named patient sales of metreleptin in Brazil, Colombia, Argentina, and a select number of other key markets, including France and Turkey. We expect net revenues from named patient sales to fluctuate significantly quarter-over-quarter given that named patient sales are derived from unsolicited requests from prescribers. In some countries, including Brazil, orders for named patient sales are for multiple months of therapy, which can lead to fluctuation in sales depending on the ordering pattern.

We expect that our near-term efforts will be focused on the following:

- continuing to sell JUXTAPID as a last-line treatment for adult HoFH patients in the U.S. despite the availability of PCSK9 inhibitor products, which have had a significant adverse impact on sales of JUXTAPID, and gaining market acceptance in the other countries, including Japan, where lomitapide is approved and being commercialized, or may in the future receive approval and be commercialized;

- reviewing our holding and capital structure with a view towards optimizing our assets and improving our balance sheet;
- managing our costs and expenses to better align with our revenues, while supporting approved products in a compliant manner;
- continuing to support patient access to and reimbursement for our products in the U.S. without significant restrictions, particularly given the availability of PCSK9 inhibitor products in the U.S., which has adversely impacted reimbursement of JUXTAPID, and given the considerable number of eligible JUXTAPID patients in the U.S. who are on Medicare Part D and the significant percentage of such patients who may not be able to afford their out-of-pocket co-payments for our products, given that prior sources of financial support for some of the patients through patient assistance programs operated by independent charitable 501(c)(3) organizations may no longer be available;
- continuing to support sales of lomitapide as a treatment for HoFH in Brazil on a named patient sales basis, particularly in light of local economic challenges, ongoing government investigations, an ongoing court proceeding reviewing the regulatory framework for named patient sales in Brazil, and recently implemented regulatory requirements for named patient sales which have added complexity to the process for named patient sales in Brazil and we believe have led a significant number of patients to discontinue therapy with lomitapide, and continuing to support named patient sales in other key countries where such sales are permitted, despite the availability of PCSK9 inhibitors on a named patient sales basis in such countries;
- initiating clinical development of metreleptin in HMD, and exploring potential new opportunities for metreleptin in additional indications, including certain other low-leptin mediated metabolic diseases, assuming we raise capital to fund such opportunities;
- building and maintaining market acceptance for MYALEPT in the U.S. for the treatment of complications of leptin deficiency in GL patients, and supporting named patient sales of metreleptin in GL in Brazil, particularly in light of local economic challenges, ongoing government investigations, an ongoing court proceeding reviewing the regulatory framework for named patient sales in Brazil, and recently implemented regulatory requirements for named patient sales which have added complexity to the process for named patient sales in Brazil and we believe have led a significant number of patients to discontinue therapy with metreleptin, and supporting such sales in other key countries, including Turkey and France, where such sales are permitted;
- gaining regulatory, pricing and reimbursement approvals to market our products in countries in which the products are not currently approved and/or reimbursed or for new indications, including obtaining approval of the MAA seeking marketing approval of metreleptin in the EU as a treatment for complications of leptin deficiency in GL patients and a subset of PL, and seeking approval of metreleptin in Brazil and other key markets as a treatment for complications of leptin deficiency in GL patients, and subject largely to whether we receive EMA approval of any PL subset indication, PL subset patients;
- preparing for the launch of metreleptin in Europe as a treatment for complications of leptin deficiency in GL patients and a subset of PL, in the event we obtain regulatory, pricing and reimbursement approvals in the EU for metreleptin;
- minimizing the number of patients who are eligible to receive but decide not to commence treatment with our products, or who discontinue treatment, by supporting activities such as patient support programs, to the extent permitted in a particular country;
- Aegerion complying with the various agreements and judgments entered into with the DOJ and SEC in September 2017, and subsequently, and Aegerion's criminal sentencing in January 2018, including the payment of approximately \$40.1 million in civil penalties, restitution and settlement amount (plus interest) over three years, and three-years of criminal probation, a civil settlement agreement with the DOJ, separate civil settlement agreements with multiple U.S. states, a final judgment entered in connection with a complaint filed by the SEC, a three-year deferred prosecution agreement with the DOJ (the "DPA"), a five-year corporate integrity agreement with the Office of Inspector General of the Department of Health and Human Services (the "CIA"), and a pending civil consent decree with the U.S. Food and Drug Administration ("FDA") and the DOJ relating to the JUXTAPID Risk Evaluation and Mitigation Strategy ("REMS") program, and managing other ongoing government investigations and related matters pertaining to its products;
- engaging in possible further development efforts related to our existing products, and assessing, and possibly acquiring, potential new product candidates targeted at rare diseases where we believe we can leverage our infrastructure and expertise;

- continuing to reinforce a culture of compliance, ethics and integrity throughout Novilion, Aegerion and their subsidiaries; and
- defending challenges to the patents or our claims of exclusivity for our products in the U.S., including against potential generic submissions with the FDA with respect to lomitapide, and expanding the intellectual property portfolio for our products.

### **Investigations and Legal Proceedings**

As noted above, Aegerion has been the subject of certain investigations and other legal proceedings (some of which remain ongoing), including investigations of Aegerion’s marketing and sales activities of JUXTAPID by the DOJ and the SEC, an investigation by federal and state authorities in Brazil to determine whether there have been violations of Brazilian laws related to the sales of JUXTAPID, and a putative class action lawsuit alleging certain misstatements and omissions related to the marketing of JUXTAPID and the Company’s financial performance in violation of the federal securities laws (the “Class Action Litigation”). Aegerion entered into agreements (the “Settlement”) with the DOJ and the SEC in September 2017 that required Aegerion, in addition to paying certain penalties and Settlement amounts, to plead guilty to two misdemeanor misbranding violations of the Food, Drug and Cosmetics Act and to enter into a three-year DPA with regard to a charge that it engaged in a conspiracy to violate the Health Insurance Portability and Accountability Act (“HIPAA”). Aegerion was sentenced by the U.S. District Court on January 30, 2018 after the judge accepted Aegerion’s guilty criminal plea. Under the terms of the Settlement, including the sentence, Aegerion is required to pay approximately \$40.1 million in aggregate penalties, plus interest, over three years, including \$7.2 million of restitution, a civil penalty of \$4.1 million to be paid to the SEC pursuant to an SEC Judgment, and \$28.8 million (including \$2.7 million designated for certain states), to be paid pursuant to the Civil Settlement Agreement, which is a significant financial burden given Aegerion’s financial condition. Aegerion made an initial payment to the DOJ on February 12, 2018, and an initial payment to certain states on February 15, 2018. On February 20, 2018, the DOJ filed a stipulation of dismissal in the civil qui tam matter. The FDA Consent Decree remains subject to approval by a U.S. District Court Judge. Aegerion also settled the Class Action Litigation for \$22.3 million. The insurance carriers agreed to cover \$22.0 million of this amount, with Aegerion responsible for the remainder of \$0.3 million. See Part I, Item 3 - “*Legal Proceedings*” for further information regarding these investigations and legal proceedings.

### **Recent Corporate and Securities Transactions**

*New Loan Agreement.* On March 15, 2018, Aegerion entered into a loan and security agreement (the “New Loan Agreement”) with affiliates of Broadfin Capital, LLC (“Broadfin Capital”) and Sarissa Capital Management LP (“Sarissa Capital”) and, together with Broadfin Capital, the “Lenders”), pursuant to which the Lenders have made a single-draw term loan to Aegerion in an aggregate amount of \$20.0 million (the “New Loan”), secured by substantially all of Aegerion’s assets, including a pledge of 66% of its first-tier foreign subsidiaries’ equity interests, and substantially all of the intellectual property and related rights in respect of MYALEPT and JUXTAPID, subject to certain exceptions. Interest on the New Loan accrues at 9.0% per annum. The term loan made pursuant to the New Loan Agreement matures on the earliest of (i) August 1, 2019, (ii) 30 days prior to the maturity date of the Convertible Notes, (iii) the date that any restructuring or recapitalization of all or substantially all of the Convertible Notes, including any exchange offer or similar transaction, is substantially consummated, and (iv) upon acceleration of the obligations under the New Loan Agreement. Concurrently with the execution of the New Loan Agreement, Novilion, Aegerion and the Lenders entered into a subordination agreement to subordinate the New Loan to the obligations of Aegerion to Novilion under the Amended and Restated Senior Loan Agreement (defined below), which is secured by the same collateral as the New Loan.

In connection with the New Loan Agreement, the Lenders were issued warrants to purchase 1,818,592 Novilion common shares. The warrants have an exercise price equal to \$4.40 per share, representing the volume weighted average price of Novilion common shares for the 20 trading days ending March 14, 2018, and have a term of four years.

In connection with Novilion’s business combination with Aegerion in 2016, Novilion entered into a secured loan facility with Aegerion (the “Senior Loan Agreement”). Since the consummation of the business combination, Aegerion has continued to borrow pursuant to the terms of the Senior Loan Agreement. In connection with the entry into the New Loan Agreement, on March 15, 2018, Aegerion and Novilion entered into an amended and restated senior secured term loan agreement (the “Amended and Restated Senior Loan Agreement”), which amends and restates the Senior Loan Agreement. The loans and interest paid in kind under the Amended and Restated Senior Loan Agreement (collectively, the “Senior Loan”) will continue to accrue interest at the rate of 8.0% per annum. Following an event of default and so long as an event of default is continuing, the interest rate would increase by 3.0% per annum. Interest will accrue and compound quarterly in arrears and is not to be payable in cash until July 1, 2019, the maturity date of the Senior Loan. The Senior Loan may be prepaid, in whole or in part, by Aegerion at any time without



premium or penalty. As of March 15, 2018, the aggregate principal amount outstanding under the Senior Loan Agreement (and that will be outstanding under the Amended and Restated Senior Loan Agreement) was approximately \$38.1 million, which includes all accrued interest paid in kind.

*Merger Transaction with Aegerion.* On June 14, 2016, we entered into the Merger Agreement with Aegerion, pursuant to which on November 29, 2016 our indirect wholly-owned subsidiary, Isotope Acquisition Corp., merged with and into Aegerion, with Aegerion surviving as our wholly-owned subsidiary. Upon completion of the Merger, on November 29, 2016, each outstanding share of Aegerion common stock was converted into a right to receive 1.0256 Novelon (pre-Consolidation) common shares and Aegerion's common stock was cancelled and delisted from the NASDAQ Global Select Market ("NASDAQ").

Pursuant to the Merger Agreement, we also issued certain warrants to the pre-closing shareholders of Novelon. These warrants (the "Merger Agreement Warrants") were exercisable for up to an aggregate of 11,301,791 Novelon common shares at an exercise price of \$0.05 per share if Aegerion's disclosed DOJ and SEC investigations or Aegerion's Class Action Litigation were settled for amounts in excess of a certain negotiated threshold. Given that the settlements of these matters did not exceed the negotiated threshold, the Merger Agreement Warrants have been cancelled subsequent to December 31, 2017 and will not be exercisable for any shares. Refer to Note 13 - *Share Capital* in the Notes to Consolidated Financial Statements included in this Annual Report for further details.

The aggregate consideration delivered to the former holders of Aegerion common stock in connection with the Merger was approximately 6,060,288 Novelon common shares. Shareholders of Novelon immediately prior to the Merger, including the participants in the private placement pursuant to the Unit Subscription Agreement (described below), owned approximately 68% of the outstanding Novelon common shares upon completion of the Merger and stockholders of Aegerion immediately prior to the Merger owned approximately 32% of the outstanding Novelon common shares upon completion of the Merger.

*Private Placement.* Also on June 14, 2016, we entered into a unit subscription agreement (the "Unit Subscription Agreement") with the investors party thereto (the "Investors"). Pursuant to the Unit Subscription Agreement, immediately prior to the Merger, the Investors acquired units, for \$8.80 per unit, on a post-Consolidation basis, consisting of (i) 2,472,727 Novelon common shares, which includes up to 568,181 Novelon common shares issuable upon exercise of fully paid-up warrants, and (ii) warrants (the "Unit Subscription Agreement Warrants") which were exercisable for up to an aggregate of 2,644,952 Novelon common shares at an exercise price of \$0.05 per share on the same terms and conditions as the Merger Agreement Warrants (collectively with the Merger Agreement Warrants, the "Warrants"). As with the Merger Agreement Warrants, these warrants have been cancelled subsequent to December 31, 2017 because the settlements of the matters described above did not exceed the negotiated threshold. The aggregate consideration paid under the Unit Subscription Agreement was approximately \$21.8 million.

*Share Consolidation.* On December 16, 2016, we completed a one-for-five (1:5) consolidation (the "Consolidation") of all of our issued and outstanding common shares, without par value, for shareholders of record as of December 16, 2016, resulting in a reduction in the issued and outstanding common shares from 92,653,562 to 18,530,323 as of that date. Each shareholder's percentage ownership in Novelon and proportional voting power remained unchanged after the Consolidation, except for minor changes resulting from the treatment of fractional shares. In connection with the Consolidation, the conversion rate of the Convertible Notes was automatically adjusted from 24.9083 common shares per \$1,000 principal amount of such Convertible Notes to 4.9817 common shares per \$1,000 principal amount of such Convertible Notes.

*Aralez Investment and Distribution.* On December 7, 2015, QLT entered into an Amended and Restated Share Subscription Agreement (the "Amended and Restated Subscription Agreement") with Tribute Pharmaceuticals Canada Inc. ("Tribute"), POZEN Inc. ("POZEN"), Aralez Pharmaceuticals plc, (formally known as Aguono Limited) ("Aralez Ireland") and certain other investors for the purpose of returning capital to QLT's shareholders in either Aralez Shares (approximately 0.13629 of an Aralez Share for each common share of the Company) or cash, subject to pro-rata (the "Aralez Distribution"), up to a maximum of \$15.0 million funded pursuant to the terms of the Backstop Agreement as described below.

In connection with the Aralez Distribution, on June 8, 2015, QLT entered into a share purchase agreement (as amended, the "Backstop Agreement") with Broadfin Healthcare Master Fund, Ltd. ("Broadfin") and the JW Partners, LP, JW Opportunities Fund, LLC and JW Opportunities Master Fund, Ltd. (together the "JW Parties"), pursuant to which Broadfin and the JW Parties agreed to purchase up to \$15.0 million of the Aralez Shares from QLT at \$6.25 per share. This arrangement provided QLT's shareholders with the opportunity to elect to receive, in lieu of Aralez Shares, up to an aggregate of \$15.0 million in cash, subject to proration among the shareholders.

Upon consummation of the merger of Tribute and POZEN (the "Aralez Merger") on February 5, 2016, we purchased 7,200,000 Aralez Shares (representing 10.1% of the issued and outstanding Aralez Shares), for an aggregate price of \$45.0 million. We held the Aralez Shares from February 5, 2016 to April 5, 2016 (the "Distribution Date") and the Aralez Shares were marked-

to-market. As a result, we recognized a \$10.7 million loss during the fiscal year ended December 31, 2016, to reflect the change in value from the acquisition date to the Distribution Date.

*Terminated Merger Transaction.* On June 8, 2015, QLT entered into an Agreement and Plan of Merger (as amended and restated on each of July 16, 2015 and August 26, 2015) (the "InSite Merger Agreement") with InSite Vision Incorporated, a Delaware corporation ("InSite"). On September 15, 2015, the InSite Merger Agreement was terminated by InSite and InSite paid QLT a termination fee of \$2.7 million. Refer to Note 4 - *Terminated Merger Transaction* in the Notes to Consolidated Financial Statements included in this Annual Report for further details.

## Marketed Products

As noted above, lomitapide and metreleptin are products that have been and continue to be developed and commercialized by our subsidiary Aegerion. All references to "we", "us", "our" and the like in this Annual Report in relation to lomitapide and metreleptin are references to the activities and plans of Aegerion or subsidiaries of Aegerion.

### Lomitapide

Lomitapide, a small molecule microsomal triglyceride transfer protein ("MTP") inhibitor, or MTP-I, is currently approved and marketed in a number of countries, including the U.S., Japan, Israel, Canada, and Colombia, under the brand name JUXTAPID, and in the EU under the brand name LOJUXTA, as an adjunct to a low-fat diet and other lipid lowering treatments, to reduce LDL-C in adult patients with HoFH.

### HoFH

HoFH is a serious, rare genetic disease that impairs the function of the receptor responsible for removing LDL-C (bad cholesterol) from the blood. An impairment of low density lipoprotein receptor ("LDL-R") function results in significant elevation of blood cholesterol levels.

Cholesterol is a naturally occurring molecule that is transported in the blood. The liver and the intestines are the two main sites where cholesterol is packaged and released within the body. The liver synthesizes cholesterol, and provides the body's intrinsic supply. The intestines are the conduit through which cholesterol enters the body for metabolism. The delivery of cholesterol to peripheral cells in the body provides necessary sources of cellular energy and cell structure. However, excess levels of cholesterol in the blood, also known as hypercholesterolemia, can be the source of significant diseases in humans. HoFH is most commonly caused by genetic mutations in both alleles of the LDL-R gene, but can also be caused by mutations in other genes. To date, more than 1,700 mutations have been identified that can impair the function of the LDL-R gene, with some mutations leading to a total lack of LDL-R activity and others leading to significantly reduced activity in the LDL-R gene. As a result of elevated levels of LDL-C, HoFH patients very often develop premature and progressive atherosclerosis, a narrowing or blocking of the arteries (usually in combination with arterial thrombosis), and are at very high risk of experiencing premature cardiovascular events, such as heart attack or stroke.

There are no universally accepted criteria for the diagnosis of HoFH. Diagnosis is typically made clinically, using the following criteria:

- significantly elevated LDL-C cholesterol levels (treated or untreated);
- physical signs, which may include the presence of cutaneous xanthomas, Achilles tendon thickening, xanthelasma and/or corneal arcus;
- limited response to statins that is not attributed to statin intolerance or to another identifiable cause (usually dependent on functional LDL receptors), or limited and/or inadequate response to a PCSK 9 inhibitor;
- evidence of premature cardiovascular disease (often in the second and third decade of life); and
- a positive history of high cholesterol and/or premature cardiovascular disease, consistent with having familial hypercholesterolemia ("FH") on both sides of the family.

HoFH is a rare form of FH and not all patients with the above characteristics will be HoFH patients. Genetic testing may be performed to make a diagnosis of HoFH, but is not routinely used in the U.S. because it has not been widely available, and because genetic testing can fail to detect certain defects given the large number of possible mutations and the number of genes

that could be involved, as described above. HoFH patients may have the same defect on both copies of the same gene or may have different defects, one inherited from each parent, on the same gene or defects inherited from each parent on two different genes each affecting the function of the LDL-R. A 2013 article in the European Heart Journal ("EHJ"), as well as a 2015 article from the American Heart Association ("AHA"), estimate that current genetic tests may fail to positively detect 10% to 40% of patients with FH. As a result, most physicians in the U.S. and in many other countries use clinical findings and family history on both sides to make a clinical diagnosis of HoFH. Although not widely used, HoFH may also be diagnosed through an assessment of LDL-R function in cultured skin fibroblasts.

Physicians treating patients with hypercholesterolemia, including HoFH, are highly focused on lowering levels of LDL-C in their patients. In the U.S., for example, organizations such as the National Cholesterol Education Program ("NCEP"), the American Heart Association, and the American College of Cardiology have emphasized aggressive management of LDL-C. NCEP guidelines currently recommend that patients at high risk of experiencing a heart attack achieve LDL-C levels of 100 mg/dL or lower through lifestyle changes and drug therapy as appropriate based on their starting levels. International guidelines for adult patients at high risk of experiencing a heart attack, such as those published in the International Journal of Cardiology and the Canadian Journal of Cardiology, and guidelines published in the EHJ in 2014 ("2014 EHJ HoFH Guidelines") that are specific to HoFH and guidelines published by the Japan Atherosclerosis Society FH Clinical Practice Guidelines Committee in 2017 support LDL-C treatment targets for such patients as low as 70 mg/dL or lower. The American College of Cardiology and the American Heart Association released guidelines in 2013 for patients at high risk of cardiovascular disease caused by atherosclerosis that are focused first on lifestyle changes and statin therapy. The 2014 EHJ HoFH Guidelines made similar recommendations regarding lifestyle changes and statin therapy for the treatment of HoFH and also recommended the use of LDL apheresis, in which cholesterol is removed from the body through mechanical filtration, and the use of other adjunctive treatments, such as lomitapide and mipomersen, for HoFH patients who are within the indication for such products (adults for lomitapide). More recent guidance, such as "The Agenda for Familial Hypercholesterolemia: A Scientific Statement from the American Heart Association" in 2015, added PCSK9 inhibitor treatment for HoFH patients as a recommended treatment. In February 2017, the American Association of Clinical Endocrinologists and American College of Endocrinology published guidelines for management of dyslipidemia and prevention of atherosclerosis. Patients in this "extreme risk" category, including men aged 55 years and younger and women aged 65 years and younger who have established cardiovascular disease accompanied by familial hypercholesterolemia, have an LDL-C goal of <55 mg/dL.

The clinical approach taken with HoFH patients typically involves an aggressive treatment plan to reduce lipid levels as much as possible through dietary modifications and a combination of available lipid-lowering drug therapies. Current therapies approved for reducing LDL-C levels in HoFH patients include statins, cholesterol absorption inhibitors, PCSK9 inhibitors and JXTAPID. With the introduction of PCSK9 inhibitors in the U.S. in 2015, healthcare professionals began trying most new adult HoFH patients on a PCSK9 inhibitor product before trying JXTAPID, and switching some existing JXTAPID patients to a PCSK9 inhibitor product, because such products, in comparison to JXTAPID, have fewer side effects, are significantly less expensive, and do not require that patients follow a special low-fat diet. Because many of these therapies, including statins and PCSK9 inhibitor products, act by increasing the activity of LDL-R, HoFH patients, given their impaired LDL-R function, or lack of function, often have an inadequate response to standard therapies. For example, high dose statin therapies that typically produce 46% to 55% reductions in LDL-C levels in the broad hypercholesterolemic patient population, on average, produce a 10% to 25% reduction in patients with HoFH. Also, in a study published in the Lancet journal in 2015, PCSK9 inhibitor evolocumab produced a mean reduction of LDL-C of 23% in HoFH patients at 12 weeks. However, a subset of HoFH patients with very low or no LDL-R function did not respond to evolocumab treatment. Patients with HoFH who are unable to reach their recommended target LDL-C levels on drug therapy are sometimes treated using LDL apheresis. Although levels of LDL-C are reduced using apheresis, there is a rapid rebound (usually after approximately four days). Because apheresis provides only temporary reductions in LDL-C levels, it must be repeated frequently. In addition, except in many countries in the EU, apheresis is not readily available, particularly in the U.S., due to the limited number of treatment centers that perform this procedure.

#### *Status in the U.S. and the EU*

In December 2012, the FDA approved JXTAPID as an adjunct to a low-fat diet and other lipid-lowering treatments, including LDL apheresis where available, to reduce LDL-C, total cholesterol, apo B and non-HDL-C in adult patients with HoFH. Aegerion launched JXTAPID in the U.S. in January 2013. The FDA has granted seven years of orphan drug exclusivity from the date of approval for JXTAPID in the U.S. in the treatment of HoFH, expiring in December 2019. The U.S. prescribing information for JXTAPID specifies that the safety and effectiveness of lomitapide have not been established in patients with hypercholesterolemia who do not have HoFH, including those with heterozygous familial hypercholesterolemia ("HeFH"), or in pediatric patients, and that the effect of lomitapide on cardiovascular morbidity and mortality has not been determined.

In July 2013, Aegerion received marketing authorization for LOJXTA in the EU as an adjunct to a low-fat diet and other lipid-lowering medicinal products with or without LDL apheresis in adult patients with HoFH. Despite the prevalence rate,

lomitapide does not have orphan medicinal product exclusivity in the EU for the treatment of HoFH because the EMA views the relevant condition, for orphan drug purposes, to include both HoFH and HeFH. The Summary of Product Characteristics ("SmPC") approved by the EC for LOJUXTA states that genetic confirmation of HoFH should be obtained whenever possible, and that other forms of primary hyperlipoproteinemia and secondary causes of hypercholesterolemia (e.g., nephrotic syndrome, hypothyroidism) must be excluded. The SmPC also specifies that the effect of lomitapide on cardiovascular morbidity and mortality has not been determined. Because Aegerion was unable to obtain commercially acceptable pricing and reimbursement approvals for LOJUXTA in several of the key markets of the EU, Aegerion elected to cease commercialization of LOJUXTA in the EU and, in December 2016, entered into a license agreement with Amryt under which Amryt was granted an exclusive right to develop and commercialize LOJUXTA in the European Economic Area ("EEA"), Switzerland, Turkey and certain Middle Eastern and North African territories, including Israel. Under the license agreement, Aegerion maintains the marketing authorizations for LOJUXTA; however, Amryt is responsible for ongoing regulatory and post-marketing obligations and commitments for LOJUXTA. Amryt is also required to pay Aegerion certain sales-related milestone payments and royalties on net product sales in the licensed territories.

The prescribing information for lomitapide in the U.S. and the EU warns physicians that lomitapide can cause hepatotoxicity as manifested by elevations in transaminases and increases in hepatic fat, and that physicians are recommended to measure alanine aminotransferase ("ALT"), aspartate aminotransferase ("AST"), alkaline phosphatase, and total bilirubin before initiating treatment and then to measure ALT and AST regularly during treatment. During the first year of treatment, physicians must conduct a liver-related test prior to each increase in the dose of lomitapide or monthly, whichever occurs first. After the first year, physicians are required to perform these tests every three months and before increases in dose. The prescribing information in the EU provides further recommendations for monitoring for hepatic steatohepatitis/fibrosis and the risk of progressive liver disease, including annual imaging for tissue elasticity, and measuring of biomarkers and/or scoring methods in consultation with a hepatologist.

Because of the risk of hepatotoxicity, JUXTAPID is available in the U.S. only through a REMS program, referred to as the JUXTAPID REMS program. The FDA approved significant modifications to the JUXTAPID REMS program on January 3, 2017 (which modifications have been implemented). The goal of the JUXTAPID REMS program, as modified, is to mitigate the risk of hepatotoxicity associated with the use of JUXTAPID by ensuring that: (a) prescribers are educated about the approved indication for JUXTAPID, the risk of hepatotoxicity associated with the use of JUXTAPID, and the need to monitor patients during treatment with JUXTAPID as per product labeling; (b) JUXTAPID is dispensed only to patients with a clinical or laboratory diagnosis consistent with HoFH; and (c) patients are informed about the risk of hepatotoxicity associated with the use of JUXTAPID and the need for baseline and periodic monitoring. The FDA's approval letter for the modified REMS program also specified that an authorized generic drug under JUXTAPID's New Drug Application ("NDA") must have an FDA-approved REMS program prior to marketing.

The originally approved JUXTAPID REMS program consisted of Elements To Assure Safe Use ("ETASU"), an implementation system, a communication plan and a timetable for submission of assessments of the JUXTAPID REMS program. It also required healthcare professionals who prescribe JUXTAPID and pharmacies that dispense JUXTAPID to be certified, and that JUXTAPID must only be dispensed to patients with evidence or other documentation of safe-use conditions. The ETASU in the modified JUXTAPID REMS program approved by the FDA on January 3, 2017 has been significantly enhanced and requires, among other things, that healthcare professionals and pharmacies complete a recertification process in order to continue prescribing and dispensing JUXTAPID; that healthcare professionals, once certified, counsel existing and new JUXTAPID patients on the goals of the JUXTAPID REMS program; and that healthcare professionals and their patients sign a form acknowledging that this counseling has taken place and that the patient understands the goals of the JUXTAPID REMS program. The modified JUXTAPID REMS program also requires that JUXTAPID prescriptions be written on an updated prescription authorization form. In order to prevent potential interruptions in therapy for existing JUXTAPID patients during the transition to the modified JUXTAPID REMS program, the FDA granted a 180-day extension to the original deadline, to December 28, 2017, for existing patients receiving therapy with JUXTAPID. This extension process allowed for existing JUXTAPID patients to continue to receive therapy with JUXTAPID while their prescriber completed recertification and conducted the patient counseling component of the JUXTAPID REMS program.

We have completed the implementation of the modifications to the JUXTAPID REMS program, including the requirements concerning recertification of current JUXTAPID prescribers and pharmacies and completion of the new patient prescriber acknowledgment form and new prescription authorization form for each existing and new JUXTAPID patient. However, we may lose JUXTAPID patients temporarily or permanently, or add new adult HoFH patients at a slower than expected pace, as a result of the enhancements to the modified JUXTAPID REMS program, as described above, for a variety of reasons, including: the inability to certify healthcare professionals who may want to put new patients on JUXTAPID, on a timely basis or at all; the failure of new healthcare professionals and/or new patients to meet the patient counseling requirements and sign and submit the patient acknowledgment form, as required, on a timely basis or at all; and that the enhanced education concerning the goals of the JUXTAPID REMS program, and related documentation, may cause healthcare professionals to stop or delay treatment with

JUXTAPID, or try alternative therapies for adult HoFH patients before starting or continuing JUXTAPID treatment. Any failure to comply with the pending consent decree that Aegerion entered into with the FDA related to the JUXTAPID REMS program as part of the settlement of the SEC and DOJ investigations may also have an effect on the FDA's requirements for the JUXTAPID REMS program.

Similarly, in the EU, we have adopted risk management plans to help educate physicians on the safety information for LOJUXTA and appropriate precautions to be followed by healthcare professionals and patients.

#### *Status outside the U.S. and the EU*

In September 2016, JUXTAPID was approved by the Ministry of Health, Labor and Welfare ("MHLW") in Japan for patients with HoFH. Approval in Japan was based on a Phase 3 study we conducted to evaluate the safety and efficacy of JUXTAPID to reduce LDL-C levels in nine adult Japanese HoFH patients. The results of the Phase 3 study were consistent with the known safety and efficacy profile of JUXTAPID. In November 2016, the MHLW approved pricing of JUXTAPID and in December 2016 we launched JUXTAPID in Japan. HoFH is listed as an intractable disease in Japan, and as part of that designation, reimbursement is mandated and patients register with the government to receive comprehensive treatment benefits, including apheresis. According to the 2014 MHLW Japanese Intractable Diseases Information Centers Listing, there are approximately 160 patients registered as diagnosed with HoFH in Japan. JUXTAPID has received orphan drug designation in the treatment of HoFH from the MHLW, which provides ten years of exclusivity. In April 2017, JUXTAPID was approved by Argentina's National Administration of Drugs, Foods and Medical Devices ("ANMAT") for patients with HoFH, and we plan to launch JUXTAPID in Argentina in 2018.

Lomitapide has also been approved as an adjunct treatment for adult patients with HoFH in other countries outside the U.S. and EU, including Colombia, Mexico, Canada, Israel, Argentina, Norway, Iceland, Liechtenstein, Taiwan and South Korea. INVIMA, the regulatory agency responsible for reviewing marketing authorization applications in Colombia, has also granted JUXTAPID five years of post-approval data exclusivity. The indications and prescribing information, including risk information, for lomitapide in these countries are generally comparable to those in the U.S.

Lomitapide is subject to risk management plans in each country in which it is approved outside the U.S. and the EU, except Israel, and such plans require the approval of regulatory authorities prior to reimbursement approval and marketing. The goal of the risk management plans is to help educate physicians on the safety information for lomitapide and appropriate precautions to be followed by healthcare professionals and patients.

We plan to file for marketing approval of lomitapide in Brazil in 2018. A regulation was approved in Brazil in December 2017 providing for special procedures concerning the registration of new drugs for the treatment, diagnosis or prevention of rare diseases; we are assessing the expected impact of this regulation on our expected submissions for marketing approval of our products in Brazil. We may also file for marketing approval in other countries where, in light of the potential size of the market and other relevant commercial and regulatory factors, it makes business sense to do so. To obtain marketing approval and commercialize JUXTAPID where approved, we must establish, and comply with, numerous and varying regulatory requirements of other countries regarding safety and efficacy and governing, among other things, clinical trials, pricing, promotion and distribution of the respective product.

We are also making lomitapide available in certain countries that allow use of a drug, on a named patient basis or under a compassionate use or other type of so-called expanded access program, before marketing approval has been obtained in such country. We charge for lomitapide for authorized pre-approval uses in some of the countries where it is available under an expanded access program, to the extent permitted by applicable law and local regulatory authorities. In 2017, the substantial majority of our revenues from named patient sales of lomitapide were derived from orders from Brazil, where patients have the right to bring legal action through the judicial system to seek access to unapproved drugs for which there are no therapeutic alternatives. We are also generating, or expect to generate, revenues from sales of lomitapide in several other countries on a named patient sales basis in the near-term. In some countries, including Brazil, orders for lomitapide on a named patient sales basis are for multiple patients and multiple months of therapy. We expect net product sales from named patient sales of lomitapide to fluctuate significantly quarter-over-quarter given that orders for named patient sales are typically for multiple months of therapy which can lead to fluctuation depending on the ordering pattern, government actions, including the ongoing investigations in Brazil, economic pressures and political unrest. In addition, the Brazilian Supreme Federal Court is currently discussing, in two ongoing lawsuits (unrelated to us), whether the government has an obligation to continue to provide, on a named patient sales basis, drugs that have not received regulatory and/or pricing and reimbursement approval in Brazil, such as lomitapide and metreleptin. Further, in October 2017, a new set of regulatory requirements governing products supplied on a named patient sales basis was published in Brazil, which has added complexity to the process for the purchase, on a named patient basis, of drugs which have not received regulatory and/or pricing and reimbursement approval in Brazil, such as lomitapide and metreleptin, which has, along with the ongoing court proceeding, resulted in delays in the receipt of orders from Brazil for existing lomitapide and metreleptin patients.

and we believe has led certain patients to discontinue therapy with our products. The result of the court proceeding, ongoing state and federal government investigations, a 2016 decision by the Brazilian pharmaceutical industry association that we violated its Code of Conduct, and negative coverage by Brazilian media could continue to significantly negatively affect product revenues from named patient sales of our products in Brazil. In certain countries where we charge for lomitapide during the pre-approval phase, we are able to establish the price for lomitapide, while in other countries we need to negotiate the price. In certain countries or under certain circumstances, we are providing lomitapide free of charge for permitted pre-approval uses and to the extent permitted by applicable law and local regulatory agencies.

### ***Clinical Development and Post-Marketing Commitments***

As part of our post-marketing commitments to the FDA for lomitapide, we completed a juvenile toxicology study in rodents to ascertain the impact, if any, of lomitapide on growth and development prior to initiating a clinical study of lomitapide in pediatric HoFH patients, and have submitted the results of this study to the FDA. In the first quarter of 2015, the FDA issued a Written Request for a study to evaluate lomitapide in pediatric HoFH patients, which, if completed as described, would provide for six months of pediatric exclusivity under the Federal Food, Drug, and Cosmetic Act ("FDCA"). In the second quarter of 2015, Aegerion decided to decline the FDA's Written Request regarding its planned study in pediatric HoFH patients, because it believed that the size and complexity of the requested trial created a considerable barrier to the feasibility of the study. Given that we have declined to conduct the study requested by the FDA, we will not be entitled to the six months of additional exclusivity available for conducting a study that is the subject of a Written Request issued by the FDA.

As part of Aegerion's post-marketing commitments to both the FDA and the EMA for lomitapide, we initiated an observational cohort study in 2014 to generate additional data on the long-term safety profile of lomitapide, the patterns of use and compliance and the long-term effectiveness of lomitapide in controlling LDL-C levels. Our commitment to the FDA is to target enrollment of 300 HoFH patients worldwide, and to study enrolled patients for a period of ten years. The EMA has required that all patients taking lomitapide in the EU be encouraged to participate in the study, and that the study period be open-ended. In connection with the license agreement with Amryt in December 2016, Amryt agreed to bear the costs of conducting this study in the EEA and other relevant territories. In the study, investigators will follow each patient to track malignancies, tumors, teratogenicity, hepatic effects, and gastrointestinal adverse reactions, events associated with coagulopathy, major adverse cardiovascular events and death. The EMA also required that a vascular imaging study be conducted to determine the impact of lomitapide on vascular endpoints, which Aegerion initiated in 2014 and is now the responsibility of Amryt pursuant to our license agreement with Amryt. In addition, we have completed certain drug-drug interactions studies and submitted the results to the EMA.

### ***Phase 3 Clinical Study***

Our Phase 3 clinical study of lomitapide evaluated the safety and effectiveness of lomitapide to reduce LDL-C levels in 29 adult patients with HoFH. The study was a multinational, single-arm, open-label, 78-week trial.

In the Phase 3 study, each patient's background lipid-lowering therapies were stabilized during a six-week run-in phase prior to dosing, and were maintained through at least the end of the 26-week efficacy phase. When added to the existing lipid-lowering therapy of the HoFH patients in the study, lomitapide reduced LDL-C by an average of 40% at week 26 in the intent-to-treat population with last observation carried forward for the patients who discontinued prematurely, and reduced LDL-C by an average of 50% for the 23 patients who completed the study through week 26. Also, approximately 65% of all patients completing the study experienced LDL-C reductions of 50% to 93% from their baseline as measured at the end of week 26. After week 26, during the 52-week safety phase of the study, adjustments to concomitant lipid-lowering treatments were allowed. Average reductions in LDL-C were sustained during chronic therapy.

The most common adverse reactions in the Phase 3 study were gastrointestinal, reported by 27 (93%) of 29 patients. Adverse reactions, reported by greater than or equal to eight patients (28%) in the HoFH clinical trial, included diarrhea, nausea, vomiting, dyspepsia and abdominal pain. Other common adverse reactions, reported by five to seven (17-24%) patients, included weight loss, abdominal discomfort, abdominal distension, constipation, flatulence, increased ALT, chest pain, influenza, nasopharyngitis, and fatigue. Elevations in liver enzymes and hepatic (liver) fat were also observed. Ten of the 29 patients in the study had at least one elevation in liver enzymes greater than or equal to three times the upper limit of normal ("ULN"), including four patients who experienced liver enzymes greater than or equal to five times the ULN. During the clinical trial, liver enzyme elevations were managed through dose reduction or temporary discontinuation of dose. There were no clinically meaningful elevations of total bilirubin, international normalized ratio or alkaline phosphatase, which are other markers of potential harmful effects on the liver. Hepatic fat increased from a baseline of 1% to a median absolute increase of 6% at 26 and 78 weeks.

Nineteen of 23 patients who completed the 78-week pivotal study entered a Phase 3 long-term extension study, and continued lomitapide at their individualized maintenance dose, with 17 (89%) completing 126 weeks of treatment. The primary efficacy endpoint of the extension study was mean percent change in LDL-C from the patient's baseline, measured at the start of the original pivotal trial, to week 126. In the extension study, mean LDL-C levels were reduced by 45.5% from baseline at week 126. Similar mean percent reductions were observed for apo B, non-HDL-C, and total cholesterol. The adverse reaction profile observed in the extension study was consistent with that observed during the pivotal trial.

### ***Estimated Prevalence of HoFH***

There is no patient registry or other method of establishing with precision the actual number of patients with HoFH in any geography. Medical literature has historically reported the prevalence rate of HoFH as one person in 1,000,000, based on an estimated prevalence rate for HeFH of one person in 500. Analysis of HoFH prevalence has been evolving in recent years culminating in published medical literature that suggests that the actual prevalence of both HeFH and HoFH may be significantly higher than the historical estimates. For example, in 2014, the European Atherosclerosis Society ("EAS") Consensus Panel on FH published an article citing research that would result in an estimate of the prevalence of HoFH in the range of between one person in 300,000 and one person in 160,000 or 3.33 persons per million to 6.25 persons per million, which is consistent with estimates that can be derived from other publications from the last few years. The FDA cited this estimate in its review of PCSK9 inhibitor products in June 2015. There is no guarantee that the prevalence of HoFH is higher than, or even as high as, the current medical literature suggests or is even higher than reported in the historical literature. Given that JUXTAPID is a last-line treatment for adult HoFH patients, the market for JUXTAPID may be significantly smaller than the prevalence of HoFH suggested by current and historical medical literature.

We believe that the prevalence rate of HoFH in countries outside the U.S. is likely to be consistent with the prevalence rate in the U.S.; however, we expect that our net product sales in countries outside the U.S. are likely to be lower than in the U.S. given the pricing and reimbursement process and approval levels in such countries compared to the U.S. and significant political and economic pressures to reduce healthcare costs in certain ex-U.S. countries, resulting in lower approved prices, pricing controls, reimbursement restrictions and caps on patients treated and/or drug expenditures, the more widespread availability of apheresis, in certain countries, like Japan, and the possibility that genotyping may be required in some countries, reducing the number of patients diagnosed with HoFH.

### **Metreleptin**

Metreleptin, a recombinant analog of human leptin, is marketed in the U.S. under the brand name MYALEPT. MYALEPT is approved in the U.S. as an adjunct to diet as replacement therapy to treat the complications of leptin deficiency in patients with congenital or acquired GL. In December 2016, we submitted an MAA to the EMA to seek approval for metreleptin in the EU, under the brand name MYALEPTA, as replacement therapy to treat complications of leptin deficiency in patients with GL and in a subset of patients with PL. In January 2018, we submitted our responses to questions raised by the EMA in its Day 180 list of questions, and in February 2018, we presented at an oral hearing of the CHMP. Based on the CHMP's feedback at and after the hearing, we have requested a two-month "clock stop" of the CHMP's review to allow for the preparation of responses and additional data focused, primarily, on the PL patient subset. Subject to our ability to address the CHMP's feedback during the "clock stop" period, we anticipate that the CHMP will issue its opinion in the second quarter of 2018 and the EC will issue its decision in mid-2018.

Leptin was initially discovered in 1994, and in the over two decades since, the role of leptin in human physiology has been further studied and understood. In addition to its central role in the regulation of energy homeostasis and glucose and fat metabolism, leptin has diverse effects on various physiologic functions, including the regulation of neuroendocrine function, reproduction, vascular function, bone metabolism, and the immune system. We plan to initiate, by late 2018, a phase 2 trial assessing metreleptin in HMD, subject to approval of our protocol and statistical plan by applicable regulatory authorities. We also plan to continue to explore new opportunities for metreleptin to treat certain other low-leptin mediated metabolic diseases, and are reviewing options for raising capital to fund such opportunities, along with later-stage studies in HMD, upon which such opportunities are largely dependent.

### ***Lipodystrophy***

Lipodystrophy is a heterogeneous group of rare syndromes characterized by selective but variable loss of fat tissue. The loss of fat tissue in patients with lipodystrophy can range from partial to more generalized, and some patients have concomitant accumulation of excess fat tissue centrally. Because of the loss of fat tissue, levels of the fat cell secreted hormone leptin are very low. Leptin is a naturally occurring hormone derived from fat cells and an important regulator of energy, fat and glucose metabolism,

reproductive capacity, and other physiological functions. Circulating levels of leptin closely correlate with the amount of fat mass present.

Due to the lack of fat cells in individuals with lipodystrophy, energy can no longer be stored as fat in adipose tissues (fat cells) and fat accumulates in the muscles and organs such as the heart, liver, and pancreas causing lipotoxicity and end-organ damage. In addition, deposition of fat in these unusual locations leads to extreme insulin resistance and its associated complications, such as diabetes mellitus, hypertriglyceridemia, hepatic steatosis, polycystic ovary syndrome, and high blood pressure. These severe metabolic abnormalities are typically resistant to conventional therapies. As a result of the deficiency of leptin associated with lipodystrophy, patients experience significant fatigue as well as unregulated appetite. The voracious appetite itself significantly aggravates the metabolic abnormalities that these patients have, and further reduces the ability to successfully treat these metabolic abnormalities with conventional therapies.

### ***Generalized Lipodystrophy***

GL is characterized by a near complete lack of adipose tissue and, consequently, leads to early and significant morbidity and mortality. Differentiation of GL (versus PL) is made based on the anatomical distribution of fat loss, which is widespread in GL patients, and the younger age and greater rapidity of onset and severity of the metabolic abnormalities. The severe metabolic abnormalities associated with GL may result in premature diabetic nephropathy, retinopathy, cardiomyopathy, recurrent attacks of acute pancreatitis, hepatomegaly, and organ failure. These complications themselves increase morbidity and mortality due to their known long-term impacts.

### ***Partial Lipodystrophy***

PL is characterized by a less uniform loss of fat cells and with a later age of onset. There can be considerable heterogeneity in the extent of fat cell loss, levels of leptin, and degree of metabolic abnormalities. Within the spectrum of PL, there are a subset of patients with more severe disease presentation. In PL patients with relative or near complete leptin deficiency, the metabolic abnormalities and longer impact on disease progression can closely mirror that of patients with GL.

We have defined a subset of patients with PL who have clinically similar metabolic disturbances as those patients with GL and who demonstrated clinically significant improvements in metabolic parameters on metreleptin treatment in clinical studies. See *Phase 3 Clinical Studies* below. Specifically, this subset includes patients with lower leptin levels, and more advanced metabolic abnormalities.

### ***Status in the U.S.***

The FDA approved MYALEPT in February 2014, as an adjunct to diet as replacement therapy to treat the complications of leptin deficiency in patients with congenital or acquired GL. The U.S. prescribing information for MYALEPT specifies that the safety and effectiveness of MYALEPT for the treatment of complications of PL or for the treatment of liver disease, including nonalcoholic steatohepatitis ("NASH"), have not been established. MYALEPT is not indicated for use in patients with HIV-related lipodystrophy or in patients with metabolic disease without concurrent evidence of congenital or acquired GL.

MYALEPT has a boxed warning, citing the risk of anti-metreleptin antibodies with neutralizing activity and the risk of lymphoma. The consequences of neutralizing antibodies are not well characterized but could reduce how well the leptin found naturally in the body works or how well MYALEPT works. Lymphoma has been observed in acquired forms of lipodystrophy with or without metreleptin therapy. Since patients with acquired lipodystrophy typically have underlying autoimmune conditions that may predispose them to risk of lymphoma, a causal link to the use of metreleptin has not been established.

Because of the risk of neutralizing antibodies and the risk of lymphoma, MYALEPT is available in the U.S. only through a restricted program under a REMS program (referred to as the MYALEPT REMS program). Under the MYALEPT REMS program, we certify all qualified healthcare providers who prescribe MYALEPT and the pharmacies that dispense the medicine. The goals of the MYALEPT REMS program are to:

- educate prescribers about the risk of neutralizing antibodies and the risk of lymphoma associated with the use of MYALEPT; and
- restrict access to therapy with MYALEPT to patients with a clinical diagnosis consistent with GL.

In May 2017, we received feedback from the FDA that a prospective placebo-controlled study will be required to support a marketing application in the U.S. for the use of metreleptin to treat a subset of the PL indication, and we are evaluating next



steps. The FDA has granted seven years of orphan drug exclusivity for MYALEPT in the U.S. in the treatment of metabolic disorders secondary to lipodystrophy.

### ***Status outside the U.S.***

MYALEPT is currently approved only in the U.S. and Japan. Pursuant to an existing distribution agreement assigned to Aegerion as part of its purchase of metreleptin rights, Shionogi & Co., Ltd. ("Shionogi") has rights to market metreleptin in Japan, South Korea and Taiwan. Shionogi received marketing and manufacturing approval in Japan for metreleptin for lipodystrophy in March 2013.

There are currently no approved treatments for GL or PL in the EU. In December 2016, we filed an MAA with the EMA seeking approval for metreleptin as replacement therapy to treat complications of leptin deficiency in patients with GL and in a subset of patients with PL. If approved, metreleptin would be marketed in the EU under the brand name of MYALEPTA. Metreleptin was granted orphan designation by the EC for the treatment of Barraquer-Simons syndrome ("acquired PL"), Berardinelli-Seip syndrome ("congenital GL"), Lawrence syndrome ("acquired GL") and familial PL.

We also plan to file for regulatory approvals for metreleptin in GL and, subject largely to whether we receive EMA approval of any PL subset indication, such PL subset in other key markets, including Brazil in late 2018. In December 2017, a regulation was approved in Brazil providing for special procedures concerning the registration of new drugs for the treatment, diagnosis or prevention of rare diseases; we are assessing the expected impact of this regulation on our expected submissions for marketing approval of our products in Brazil.

When Aegerion acquired metreleptin from AstraZeneca in January 2015, a number of patients were receiving metreleptin therapy free of charge in certain countries outside the U.S. that allow use of a drug, under a compassionate use or other type of expanded access program, before marketing approval has been obtained in such country. Where permitted in accordance with applicable requirements, we have continued to make metreleptin available free of charge under such program, which has resulted in significant costs to us, given that we have more than 100 patients participating in this program; many of these patients are GL and PL subset patients who will be eligible for paid commercial therapy if we obtain regulatory, pricing and reimbursement approvals in the EU for metreleptin. In 2016, we began generating revenues from named patient sales of metreleptin in certain markets where such sales of metreleptin are possible and to the extent permitted by applicable law and local regulatory authorities. In particular, we are in the process of converting GL and PL patients currently in the expanded access program in France to a paid program of Autorisation Temporaire d'Utilisation (Temporary Authorization for Use). Metreleptin has also been approved for reimbursement by the Turkish Social Security Association ("SGK"), and we are providing metreleptin on a named patient basis there for GL patients, including congenital GL patients, and other subsets of lipodystrophy patients, including patients with congenital leptin deficiency, subject to individual assessment in response to unsolicited requests from clinicians. Further, we now supply paid product for individual patients in certain other markets and anticipate that further unsolicited requests from clinicians may follow in these countries and potentially other selected markets in the EU where there is a formal mechanism for named patient sales in place.

Outside of the EU, we have named patients sales of metreleptin in Brazil, Argentina, Colombia, and certain other markets. We expect net product sales from named patient sales of metreleptin to fluctuate significantly quarter-over-quarter given that orders for named patient sales are typically for multiple months of therapy which can lead to fluctuation depending on the ordering pattern, government actions, including the ongoing investigations in Brazil, economic pressures and political unrest. The result of the ongoing proceeding before the Brazilian Supreme Federal Court concerning the government's obligation to provide drugs on a named patient sales basis, the ongoing state and federal government investigations into Aegerion's activities in Brazil, a 2016 decision by the Brazilian pharmaceutical industry association that we violated its Code of Conduct, and negative coverage by Brazilian media could continue to significantly negatively affect product revenues in Brazil.

### ***Phase 3 Clinical Study***

The safety and efficacy of metreleptin for the treatment of metabolic disorders associated with lipodystrophy syndromes in pediatric and adult patients were evaluated in a long-term, open-label, single-arm study conducted at the National Institutes of Health (the "NIH"). The objective of the NIH trial was to evaluate the efficacy of metreleptin for improving metabolic disorders associated with acquired or inherited lipodystrophy. This investigator-sponsored study was initiated in August 2000.

A total of 107 patients ( $\geq 6$  months of age) with a clinical diagnosis of GL or PL, low baseline leptin levels (men  $< 8$  ng/mL, women  $< 12$  ng/mL), and at least one metabolic abnormality (diabetes mellitus, hypertriglyceridemia  $> 200$  mg/dL, fasting insulin levels  $> 30\mu$ U/mL) were enrolled in the NIH study. A total of 66 of the 107 patients had GL and 41 had PL, including 31 patients who were included in the PL subgroup of the studied patients, and who constitute the "PL subset" described in our MAA

currently being assessed by the EMA, i.e. those PL patients who have similar metabolic disturbances as patients with GL and who were defined as patients with baseline Hemoglobin A1c ("HbA1c")  $\geq 6.5\%$  and/or triglycerides  $\geq 5.65$  mmol/L. Given that the MAA review is ongoing, the PL indication, if approved by the EMA, may be distinct from the PL subset described in our MAA. Among the 66 patients with GL, 45 (68%) had congenital GL and 21 (32%) had acquired GL. Most patients in the PL subgroup had familial PL (27 patients, 87%); four patients (13%) had acquired PL.

Metreleptin was administered subcutaneously once or twice daily in a gender-dependent, weight-based protocol, with step-wise specified titration over the first two months of the study and subsequent dose adjustments based on clinical response. The co-primary efficacy endpoints in the NIH study were defined as:

- Actual change from baseline in HbA1c at month 12, and
- Percent change from baseline in fasting serum triglycerides at month 12.

Treatment with metreleptin led to substantial and sustained improvements in glycemic control and hypertriglyceridemia in patients with GL and in the PL subgroup.

The observed primary efficacy results in GL patients are as follows:

- A mean change from baseline to month 12 in HbA1c of -2.2%.
- A mean percent change from baseline to month 12 in triglycerides of -32.1%. The observed primary efficacy results in PL subgroup patients are as follows:
- A mean change from baseline to month 12 in HbA1c of -0.9%; and
- A mean percent change from baseline to month 12 in triglycerides of -37.4%.

In general, changes in fasting plasma glucose followed a similar pattern to changes in HbA1c.

Patients who met target decreases in both parameters were also assessed. In the GL group, 55% of patients achieved both an actual decrease in HbA1c of  $\geq 1\%$  and a  $\geq 30\%$  reduction in triglycerides at month 12; with over one-third of patients achieving the highest target reductions of a  $\geq 2\%$  actual decrease in HbA1c and a  $\geq 40\%$  reduction in triglycerides. These levels of reduction in baseline metabolic abnormalities were also observed in patients in the PL subgroup. In this subgroup, 30% of patients achieved both an actual decrease in HbA1c of  $\geq 1\%$  and a  $\geq 30\%$  reduction in triglycerides at month 12. Based on the overall mixed-model repeated measures analysis, which evaluates average levels of HbA1c and triglycerides across all visits, statistically significant decreases in HbA1c from baseline over all analysis visits was observed in the GL group and in the PL subgroup.

Median overall duration of metreleptin treatment was 49.9 months and 29.3 months in GL patients and in PL subgroup patients, respectively.

The most common adverse drug reactions ("ADRs") occurring in GL patients were weight decrease (reported by fifteen patients; 22.7%) and hypoglycemia (reported by eight patients; 12.1%), followed by decreased appetite, fatigue and neutralizing antibodies (each reported by four patients; 6.1%). The most common ADRs occurring in PL subgroup patients were hypoglycemia and fatigue (each reported by three patients; 9.7%), followed by alopecia (reported by two patients; 6.5%). Over the 14-year study duration, treatment-emergent deaths were reported in 4 (4%) of the 107 patients; treatment-emergent adverse events leading to death were consistent with the underlying morbidity of lipodystrophy and included renal failure, cardiac arrest (with pancreatitis and septic shock), progressive end-stage liver disease (chronic hepatic failure), and hypoxic-ischemic encephalopathy. None of the deaths was assessed as drug-related.

The presence of neutralizing antibodies in a small minority of patients did not result in clearly identified clinical sequelae. Two cases of peripheral T-cell lymphoma and one case of a localized anaplastic lymphoma kinase-positive anaplastic large cell lymphoma (a type of T-cell lymphoma) were reported, all in patients with acquired GL. Lymphoma is known to be associated with autoimmune disease. As the boxed warning for MYALEPT states, T-cell lymphoma has been reported in patients with acquired GL, both treated and not treated with MYALEPT. There was evidence of pre-existing lymphoma and/or bone marrow/hematologic abnormalities in the two patients with peripheral T-cell lymphoma prior to metreleptin therapy, and the third case of anaplastic large cell lymphoma occurred in the context of a specific chromosomal translocation.

## ***Clinical Development and Post-Marketing Commitments***

As part of the post-marketing commitments to the FDA for metreleptin, we have initiated a long-term, prospective, observational study (product exposure registry) in patients to evaluate serious risks related to the use of the product. The registry will attempt to enroll at least 100 new patients treated with metreleptin. Enrollment will close after five years or after 100 new patients have been enrolled, whichever occurs first. The registry will continue for ten years from the date of last patient's enrollment. We have also committed to the EMA, as part of our pediatric investigation plan ("PIP"), to conduct a study in GL patients under six years old to further evaluate the pharmacokinetics, efficacy and safety of metreleptin in this pediatric sub-population. In connection with the ongoing review of the metreleptin MAA and its expected approval, we anticipate the conduct of the following studies: a non-interventional, prospective, observational study (product exposure registry) of GL and PL subset patients initiating treatment with metreleptin, a post-approval efficacy study ("PAES") in PL patients, an in vitro study to assess the binding of metreleptin to proteins in serum, and a non-clinical tissue distribution study.

In addition, three programs are expected to expand the understanding of the immunogenicity of metreleptin. These programs consist of:

- the development, validation, and implementation of a ligand binding assay to supplement the neutralizing bioassay that tests for the presence of neutralizing antibodies in serum samples from patients with GL, which we have completed;
- testing all banked clinical samples from the GL clinical program for the presence of neutralizing antibodies against leptin using the ligand binding assay and to correlate neutralizing antibodies with clinical events, which we have completed and regarding which we are awaiting feedback from the FDA; and
- a prospective study to assess the immunogenicity of metreleptin in patients receiving metreleptin, which is in the planning phase and which we expect to initiate in 2018.

The presence of neutralizing antibodies will be assessed using both a validated cell-based assay and a validated ligand-binding assay in samples that are confirmed positive for binding antibodies to leptin. In addition, we are required to conduct certain studies related to the manufacture of metreleptin, including in order to validate new test methods, implement a risk-based reference standard program approach, and reassess product acceptance criteria with a larger data set from more manufactured batches. The three post-marketing commitments related to manufacturing metreleptin are completed or are on track for completion by their respective commitment deadlines. Finally, we have an ongoing commitment to assess spontaneous reports of serious risks related to the use of metreleptin, including the risk to exposed pregnancies and pregnancy outcomes, regardless of indication, for ten years from the date of approval of metreleptin in the U.S.

## ***Estimated Prevalence of GL and PL***

There is no patient registry or other method of establishing with precision the actual number of patients with GL and PL in any geography. The data to date suggest that the approximate prevalence of GL in the U.S. is slightly under one in 1,000,000 persons and for PL overall is three in 1,000,000 persons. Although the data are even more limited, the prevalence in the U.S. of a subset of more severe PL, one that is comparable to the PL subset indication that we included in our MAA, is estimated to be between approximately 0.5 and one in 1,000,000 persons. We believe that the prevalence rate of GL and PL, and correspondingly the PL subset, in countries outside the U.S. is likely to be consistent with the prevalence rate in the U.S. There is no guarantee, however, that our estimates are correct. The actual prevalence of GL, PL and the PL subset may be significantly lower than we expect. Ultimately, the actual size of the total addressable market in the U.S. and other key markets where metreleptin is sold, if approved, will be better understood only after we have substantial commercial history selling metreleptin.

## ***Clinical Development***

We plan to initiate, in late 2018, a phase 2 trial assessing metreleptin in HMD, subject to approval of our protocol and statistical plan by applicable regulatory authorities. We also plan to continue to explore new opportunities for metreleptin to treat certain other low-leptin mediated metabolic diseases, and are reviewing options for raising capital to fund such opportunities, along with later-stage studies in HMD, upon which such opportunities are largely dependent.

## ***Commercialization and Patient Support***

We market and sell JUXTAPID and MYALEPT through our Aegerion subsidiary. We believe that the key priorities for the successful commercialization of our products in the countries in which we have received marketing approval, and the preparation for commercialization in the countries in which our products may be approved, include:

- commercializing JUXTAPID and MYALEPT in the U.S. with a full-time employed sales force that leverages claims data analysis and other information to help identify potential GL and adult HoFH patients; and reorganizing and realigning our sales and medical organizations in support of key centers of excellence for both JUXTAPID and MYALEPT;
- educating and training healthcare providers about our products and the diseases they are approved to treat, including educating healthcare providers about JUXTAPID as a last-line treatment for adult HoFH patients in the U.S. after use of PCSK9 inhibitor products, which products have continued to have an adverse impact on long-term use of, and new prescriptions for, JUXTAPID; and gaining market acceptance in the other countries, including Japan, where lomitapide is approved and being commercialized, or may in the future receive approval and be commercialized;
- continuing to support patient access to and reimbursement for our products in the U.S. without significant restrictions, particularly given the availability of PCSK9 inhibitor products in the U.S., which has adversely impacted reimbursement of JUXTAPID, and given the considerable number of eligible JUXTAPID patients in the U.S. who are on Medicare Part D and the significant percentage of such patients who may not be able to afford their out-of-pocket co-payments for our products, given that prior sources of financial support for some of the patients through patient assistance programs operated by independent charitable 501(c)(3) organizations may no longer be available;
- gaining regulatory, pricing and reimbursement approvals to market our products in countries in which the products are not currently approved and/or reimbursed or for new indications, including obtaining approval of the MAA seeking marketing approval of metreleptin in the EU as a treatment for complications of leptin deficiency in GL patients and a subset of PL patients; and
- minimizing the number of patients who are eligible to receive but decide not to commence treatment with our products, or who discontinue treatment, by supporting activities such as patient support programs, to the extent permitted in a particular country.

The principal goals of our commercial strategy are to stabilize the number of patients on JUXTAPID, to support current and future potential for incremental sales of JUXTAPID and to grow sales of MYALEPT in the U.S., as well as prepare for launch in other jurisdictions in which we have submitted for approval of our products, such as MYALEPTA in the EU, if approved for sale.

In 2017, our commercial organization in the U.S. included a small contract sales force. In January 2018, we converted the contract sales force to full-time employees to create a full-time employee-based sales team. This team operates in four regions to educate and train healthcare providers who treat adult HoFH and GL patients about the safety and efficacy of JUXTAPID or MYALEPT, as applicable. The most frequent physician call points for our products are endocrinologists (including pediatric endocrinologists, with respect to MYALEPT), lipidologists, and cardiologists. We also implemented the use of de-identified claims data to more precisely target physicians who may have recently treated patients with adult HoFH or GL.

Outside the U.S., we mainly use country managers to market and sell our products where they are approved. In Japan, we sell JUXTAPID through a small contract sales force which is overseen by our country manager. The rights to commercialize LOJUXTA in the EU and certain other jurisdictions were out-licensed to Amryt in December 2016. In certain other countries outside the U.S., we have engaged, or plan to engage local distributors to conduct permitted commercial and pre-approval activities.

We also have a small marketing team that, along with third-party contractors, support our commercialization and disease awareness efforts in the countries in which our products are approved, and permitted educational and disease awareness activities in other parts of the world where we do business.

Another key aspect of our commercialization efforts is obtaining market access for our products, which primarily represents securing pricing and reimbursement approvals on acceptable levels, without the imposition of significant restrictions, such as caps, significant step edits or other similar measures, from private and government payers where our products are approved. In the U.S., we have a small team that supports our market access initiatives, which is primarily responsible for working with insurance plans, health maintenance organizations and other payers on securing reimbursement and formulary status for JUXTAPID and MYALEPT. This team also focuses on anticipating authorization and reauthorization requirements and educating physicians and office staff regarding criteria to continue coverage for our products. Outside the U.S., we support this effort primarily through the work of country managers and local third-party consultants. One of our main market access objectives, which is conducted in conjunction with our medical and health economics teams, is to strengthen the value proposition for JUXTAPID and MYALEPT for payers through the generation of critical market access studies to enhance patient, physician and payer knowledge of GL, HoFH, and, subject to approval in the EU, the PL subset and the real-world burden of these diseases.

We believe the pricing for our products in the U.S. is consistent with the level of pricing for other rare orphan drugs that treat diseases with comparable prevalence rates. The majority of payers in the U.S. are providing coverage for our products, and with respect to JUXTAPID, most payers in the U.S. have not required genotyping. Many payers in the U.S. have, however, imposed requirements, conditions or limitations as conditions to coverage and reimbursement for JUXTAPID as a result of the commercial availability of PCSK9 inhibitor products, which often include a requirement that HoFH patients have not achieved an adequate LDL-C response on PCSK9 inhibitor products before access to JUXTAPID is approved. For patients currently taking JUXTAPID, several U.S. pharmacy benefit managers ("PBMs") are using prior authorization requiring current JUXTAPID patients to "step through" the less expensive PCSK9 inhibitor product, and additional PBMs and payers may follow this practice. For MYALEPT, some U.S. payers require additional information, such as a leptin level test for patients, which may delay or otherwise impact reimbursement. The cost of JUXTAPID and MYALEPT in the U.S. may result in co-pay amounts for some patients that are prohibitive, and prevent these patients from being able to commence therapy on JUXTAPID or MYALEPT, respectively. We have a direct co-pay assistance program that provides support to eligible commercial patients for certain drug co-pays and co-insurance obligations for JUXTAPID. We also provide support to eligible commercial patients for certain drug co-pays and co-insurance obligations for MYALEPT treatment. We currently do not plan to provide financial support to patient assistance programs operated by independent charitable 501(c)(3) organizations in the U.S. that are permitted, subject to compliance with strict legal and regulatory requirements, to assist eligible HoFH and GL patients, as determined solely by the organization, with certain co-payments or co-insurance requirements for their drug therapies, which may include lomitapide or metreleptin. If we do elect to provide such financial support, we would not have control or input into the decisions of these organizations. We believe that investigations and enforcement actions by certain government agencies have caused a reduction in contributions to these third-party patient organizations, which may prevent these organizations from providing adequate financial assistance, including assistance with co-payment obligations, to individuals who would otherwise be unable to afford our products. As a result, Medicare Part D patients may not be able to afford their out-of-pocket co-payments for our products. In 2017, for example, we believe that a considerable number of JUXTAPID patients who were covered by Medicare Part D ceased treatment with JUXTAPID, due to reductions in contributions to patient assistance programs operated by independent charitable 501(c)(3) organizations, which resulted in prior sources of financial support for these patients being less available.

In the second quarter of 2017, to improve distribution efficiency, we signed a letter of intent for the distribution of JUXTAPID with the same specialty pharmacy that distributes MYALEPT in the U.S. The agreement was finalized in October 2017, and the transition of this distribution model was completed in November 2017. Our specialty pharmacy provides a comprehensive patient support program for each of JUXTAPID and MYALEPT in the U.S. Each program includes educational resources about the relevant product and disease; insurance verification and reimbursement support; monitoring and support of adherence; providing patients with information about potential sources of financial assistance; for JUXTAPID, nutritional counseling, and for MYALEPT, injection training; and a free drug program for certain eligible uninsured and underinsured patients. We also maintain a small internal patient support team to manage our specialty pharmacy and to supplement the patient support efforts of our specialty pharmacy.

## **Medical Affairs**

We have a medical affairs function in the U.S., the EU, Latin America, and Japan. The medical affairs function is responsible for conducting medical research, including maintaining product registries, performing retrospective reviews of clinical data, and conducting post-marketing clinical trials as needed, to support the value proposition of our products. The medical affairs function also supports independent medical education programs and investigator-initiated studies by providing financial grants in a number of medical and disease-related areas. The responsibilities of medical affairs personnel also include providing training and education to physicians through the dissemination of medical information and publications; providing support in connection with our post-approval clinical commitments, and organizing scientific and medical advisory boards to obtain input from experts and practitioners on a variety of medical topics relevant to our products and the diseases our products treat.

## **Manufacturing, Supply and Distribution**

We and our contract manufacturers are subject to the FDA's current Good Manufacturing Practices ("cGMP") regulations and other rules and regulations prescribed by regulatory authorities outside the U.S.

Lomitapide is a small molecule drug that is synthesized with readily available raw materials using conventional chemical processes. Hard gelatin capsules are prepared in 5 mg, 10 mg, 20 mg, 30 mg, 40 mg and 60 mg strengths. Metreleptin is a recombinant protein biologic that is produced using conventional fermentation, isolation, and purification processing techniques. The drug product is provided in nominal 10 mg vials that are reconstituted prior to injection. We are in the process of developing new presentations in nominal 2.5 mg and 5 mg vials. In order to provide metreleptin in these new presentations in the EU, assuming

we obtain approval of our MAA, we must, after approval, file variations with the EMA establishing that these new presentations are comparable to the current 10 mg vial presentation.

We currently have no manufacturing facilities, and limited personnel with manufacturing experience. We rely on contract manufacturers to produce drug substance for lomitapide and metreleptin and to produce drug product for commercial supplies and for our clinical trials. Aegerion has long-term supply agreements with lomitapide drug substance and drug product manufacturers. Aegerion also has long-term supply agreements with metreleptin drug substance and drug product manufacturers, which were assigned to Aegerion in connection with the acquisition of metreleptin in 2015. In December 2017, the contract manufacturer for metreleptin drug product informed Aegerion that it will terminate its supply contract in December 2019. Aegerion has a supply of safety stock and has a mitigation plan with another contract manufacturer for metreleptin drug product. Validation at the back-up manufacturer is ongoing. In February 2017, our current contract manufacturer for metreleptin drug product received a Warning Letter from the FDA citing significant violations of cGMP regulations at the manufacturing facility where metreleptin drug product is manufactured. In response, the manufacturer may make modifications to the line on which metreleptin drug product is filled. We have sufficient inventory of lomitapide and metreleptin drug substance to maintain a supply for more than one year.

In addition, our metreleptin drug product manufacturer also supplies bacteriostatic water for injection (“BWFI”), one of the approved diluents for reconstitution of metreleptin, which allows for use of a reconstituted vial of metreleptin for up to three days when stored appropriately. BWFI is only available for sale and approved for use in the U.S., and we or our contract manufacturers purchase it for supply with ex-US named patient sales and other expanded access distribution to reduce the number of metreleptin vials provided. Although BWFI is currently in shortage and on back order from our contract manufacturer, we have secured sufficient inventory to maintain a supply through late 2018 based on current demand. If in the future we exhaust our inventory of BWFI, ex-U.S. patients who obtain metreleptin via named patient sales or an expanded access program will need to use water for injection, a diluent which requires immediate use of product after reconstitution, and the discarding of any product remaining in the vial, which will likely result in more metreleptin vials being shipped and could decrease our revenues from named patient sales, increase our expenses, and impact our inventory of metreleptin and our agreements with our distributors. We do not have any other agreements in place for redundant supply or a second source for drug substance or drug product for either product.

In the U.S., lomitapide and metreleptin are distributed through a single specialty pharmacy that distributes the products directly to patients and, under limited circumstances, to other purchasers. Our specialty pharmacy takes title upon delivery of our products to such pharmacy. As noted above, in the second quarter of 2017, to improve distribution efficiency, we signed a letter of intent for the distribution of JUXTAPID with the same specialty pharmacy that distributes MYALEPT in the U.S. The agreement was finalized in October 2017 and the transition of this distribution model was completed in November 2017. Prior to the transition, the specialty pharmacy that distributed JUXTAPID did not take title to JUXTAPID; title transferred upon delivery of JUXTAPID to the purchaser. Our specialty pharmacy provides certain patient program support services, as set forth in the “*Commercialization and Patient Support*” section of this Annual Report. For named patient sales and other expanded access distribution, we use third-party providers to distribute our products either directly to the purchaser in the applicable country or to our local third-party distributor or service provider for such country.

#### **Financial Information about Segments and Geographic Areas**

We currently operate in one business segment, pharmaceuticals, and are focused on the development and commercialization of two commercial products. Financial information about this segment required herein is contained in the “*Consolidated Financial Statements and Supplementary Data*” section of this Annual Report, and the geographic information required herein is contained in Note 17 - *Segment Information* in the Notes to Consolidated Financial Statements and is incorporated herein by reference.

#### **Competition**

Our industry is highly competitive and subject to rapid and significant technological change. Our competitors and potential competitors include large pharmaceutical and biotechnology companies, specialty pharmaceutical and generic drug companies, academic institutions, government agencies and research institutions. All of these competitors currently engage in, have engaged in or may engage in the future in the development, manufacturing, marketing and commercialization of pharmaceuticals that compete with or may in the future compete with lomitapide, metreleptin, or other products or product candidates we may develop or acquire. Smaller or early stage companies may also prove to be significant competitors, particularly through collaborative arrangements with large, established companies. Academic institutions, government agencies and other public and private research organizations also are conducting research activities, seeking patent protection and may commercialize products on their own or through joint ventures. The existence of these products, or other products or treatments of which we are not aware, or products or treatments that may be developed in the future, may adversely affect the marketability of products developed by us. Key competitive factors affecting the commercial success of lomitapide, metreleptin, and any other products or product candidates that we develop

or acquire include efficacy, safety and tolerability profile, reliability, physician acceptance, level of generic competition, convenience of dosing, price and reimbursement.

The market for cholesterol-lowering therapeutics is large and competitive with many drug classes. Lomitapide is approved in the U.S., Japan, the EU and in certain other countries as an adjunct to a low-fat diet and other lipid-lowering treatments to reduce LDL-C in adult HoFH patients. As a treatment for HoFH, JUXTAPID competes in the U.S. and certain other countries with Kynamro. Developed by Ionis Pharmaceuticals and now marketed by Kastle Therapeutics, Kynamro is an antisense apolipoprotein B-100 inhibitor which is taken as a weekly subcutaneous injection. JUXTAPID also faces significant competition in the treatment of adult HoFH patients with a class of drugs known as PCSK9 inhibitors. In July 2015, Regeneron Pharmaceuticals, Inc. ("Regeneron") and Sanofi announced that the FDA had approved the biologics licensing application ("BLA") for their PCSK9 inhibitor candidate, alirocumab, for use in addition to diet and maximally tolerated statin therapy in adult HeFH patients and in patients with clinical atherosclerotic cardiovascular disease who require additional lowering of LDL-C. In September 2015, following the positive opinion of the CHMP of the EMA, the EC approved alirocumab for the treatment of adult patients with HeFH or mixed dyslipidemia as an adjunct to diet, either in combination with a statin, or statin with other lipid-lowering therapies in patients unable to reach their LDL-cholesterol goals with the maximally-tolerated statin, or alone or in combination with other lipid-lowering therapies for patients who are statin intolerant, or for whom a statin is contraindicated. The FDA approved Amgen Inc.'s ("Amgen") BLA for its anti-PCSK9 antibody, evolocumab, in August 2015, as an adjunct to diet and maximally tolerated statin therapy for the treatment of adults with HeFH or clinical atherosclerotic cardiovascular disease, who require additional lowering of LDL-C; and as an adjunct to diet and other LDL-lowering therapies for the treatment of patients with HoFH, who require additional lowering of LDL-C. In July 2015, the EC approved the marketing authorization of evolocumab for the same indication as alirocumab, and for the treatment for certain patients with high cholesterol, including patients aged 12 years and over with HoFH in combination with other lipid-lowering therapies. In January 2016, the MHLW in Japan approved evolocumab for the treatment of patients with FH or hypercholesterolemia who have high risk of cardiovascular events and do not adequately respond to statins, and in July 2016 the MHLW approved alirocumab for the same indication. The regulatory agencies responsible for reviewing marketing authorization applications in Brazil, Canada, Colombia, and Argentina have also approved evolocumab for the treatment of patients with HoFH. Other companies, including Eli Lilly & Co. and Alnylam Pharmaceuticals, Inc., in collaboration with The Medicines Company, are also developing PCSK9 inhibitor products.

The introduction of PCSK9 inhibitors in the U.S. and in other key markets has significantly negatively impacted sales of JUXTAPID and we expect continued pressure on sales of JUXTAPID. This impact on JUXTAPID sales results from several factors, including: healthcare professionals switching some existing JUXTAPID patients to a PCSK9 inhibitor product; healthcare professionals trying most new adult HoFH patients on a PCSK9 inhibitor product before trying JUXTAPID because such products, in comparison to JUXTAPID, have fewer side effects, are significantly less expensive, and do not require that patients follow a special low-fat diet; requirements imposed by insurance companies, managed care organizations and other private payers in the U.S. that HoFH patients demonstrate an inability to achieve an adequate LDL-C response on PCSK9 inhibitor products before access to JUXTAPID is approved; and the likelihood that prior authorization (which we believe is required by all U.S. payers) will encourage a switch of current JUXTAPID patients to the less expensive PCSK9 inhibitor product. Competitors for JUXTAPID may enjoy other competitive advantages if insurance companies, managed care organizations or other private payers in the U.S. impose other hurdles to access or other significant restrictions or limitations on reimbursement, or require switching of JUXTAPID patients to PCSK9 inhibitor products. We believe that many of the PCSK9 inhibitor switches from current JUXTAPID patients have been at the direction of the prescribing physician, and physicians may ultimately not decide to switch the adult HoFH patient back to JUXTAPID even if the patient does not reach a goal of LDL-C response while being treated with a PCSK9 inhibitor product, or otherwise prescribe JUXTAPID to patients. It is unknown how many adult HoFH patients, if any, may be switched back to JUXTAPID or the period of time in which this might take place.

The availability of PCSK9 inhibitor products in commercial markets outside of the U.S. is having similarly negative effects on sales, including named patient sales, of lomitapide outside the U.S., particularly in Brazil, Japan, Canada and Colombia, where PCSK9 inhibitor products have been approved by the regulatory authorities and are available commercially. If the continued negative impact of PCSK9 inhibitors is greater than we expect, it may make it more difficult for us to generate revenues and achieve profitability.

Potential future competition for JUXTAPID may include drugs and biologics currently in clinical development for the treatment of patients with HoFH. Development programs currently include other oral drug therapeutics, such as Gemphire Therapeutics' gemcabene which targets apolipoprotein C-III and is currently in Phase 3 development for the treatment of FH. Other potential therapies include gene therapies, such as REGENXBIO's LDL-receptor gene therapy which is currently in Phase 1/2 development for HoFH. Also, although there are no other MTP-I compounds currently approved by the FDA for the treatment of hyperlipidemia in humans, there may be other MTP-I compounds in development. If approved, novel drug or biologic therapies for the treatment of HoFH or FH could negatively impact the sales of JUXTAPID in the future.

In addition, in the EU, patients with HoFH who are unable to reach their recommended target LDL-C levels on conventionally used drug therapies are commonly treated using LDL apheresis, in which cholesterol is removed from the body through mechanical filtration. Although levels of LDL-C are reduced using apheresis, there is a rapid rebound. Because apheresis provides only temporary reductions in LDL-C levels, it must be repeated frequently, typically one or two times per month. The widespread use and availability of apheresis as a treatment for HoFH in the EU, combined with the lower cost of apheresis compared to LOJUXTA, made it more difficult for us to maintain a profitable business for LOJUXTA in the key markets of the EU. For example, in part because Aegerion was unable to obtain commercially acceptable pricing and reimbursement approvals for LOJUXTA in several of the key markets of the EU, Aegerion elected to cease commercialization in the EU, and in December 2016, entered into a license agreement with Amryt under which Amryt was granted an exclusive right to develop and commercialize LOJUXTA in the EEA, Switzerland, Turkey, and certain Middle Eastern and North African countries, including Israel.

MYALEPT is the first and only product approved in the U.S. for the treatment of complications of leptin deficiency in patients with GL. There are, however, a number of therapies approved to treat these complications independently that are not specific to GL. Certain clinical complications of GL, including diabetes and hypertriglyceridemia, may be treated with insulin and/or oral medications, such as metformin, insulin secretagogues, fibrates, or statins, but patients with GL often have an inadequate response to these therapies.

We are seeking regulatory approval of metreleptin as a treatment for GL and a PL subset in the EU and evaluating whether to seek regulatory approval of metreleptin for that PL subset in the U.S. and in other key countries where it makes business sense to do so. Akcea Therapeutics ("Akcea") is developing volanesorsen, an antisense therapy targeting apolipoprotein C-III, which is currently in Phase 3 development for patients with familial PL with diagnosed type 2 diabetes mellitus or hypertriglyceridemia, which, if approved and depending on the labeled indication, could potentially compete with metreleptin, if metreleptin is approved for the treatment of a PL subset.

We may also face future competition from companies selling generic alternatives of our products in countries where we do not have patent coverage, orphan drug status or another form of data or marketing exclusivity or where patent coverage or data or marketing exclusivity has expired or may, in the future, be challenged.

### **Patents, Trademarks and Proprietary Rights**

Our business relies on patents covering inventions licensed from third parties, and on other means to protect our technology, inventions and improvements that are commercially important to our business. Our policy is to file patent applications on a worldwide basis in those jurisdictions where we consider it beneficial, depending on the subject matter and our commercialization strategy.

Our success will depend significantly on our ability to:

- obtain and maintain patent, regulatory exclusivity and other proprietary protection for the products, technology, inventions and improvements we consider important to our business;
- defend our patents;
- preserve the confidentiality of our trade secrets; and
- operate without infringing the patents and proprietary rights of third parties.

### ***Patent Rights and Regulatory Exclusivity***

*Lomitapide.* Our lomitapide patent portfolio consists of eight issued U.S. patents and issued patents in Europe, Australia, South Korea, New Zealand and Japan and pending applications in the U.S., Japan, Canada, and India, all of which have been licensed to us in a specific field. A five-year patent term extension has been granted for our U.S. patent covering the composition of matter of lomitapide, originally scheduled to expire in early 2015, and will now expire in 2020. The non-U.S. patents directed to the composition of matter of lomitapide have expired. Our six method of use patents in the U.S. cover certain dosing regimens for lomitapide, with one such patent expiring in 2027 and the other five patents expiring in 2025. Two separate inter partes review ("IPR") petitions were filed with the Patent Trial and Appeal Board ("PTAB") of the U.S. Patent and Trademark Office in August 2015 by the Coalition for Affordable Drugs VIII L.L.C. ("CFAD") challenging the validity of two of these method of use patents. On March 6, 2017, the PTAB determined that the CFAD failed to show that the claims of these patents were invalid. Our non-U.S. patents, including the European Patent Office ("EPO") methods of use patent, expire in 2025. The EPO method of use patents may be eligible for up to about three years of supplemental protection in certain EPO countries, and we have applied for such



protection in the countries in which LOJUXTA is approved, on a country-by-country basis; in some countries supplemental protection has been granted to extend patent protection to July or August of 2028, while in other countries the application is still pending. An opposition was filed with respect to the EPO method of use patent, but has since been revoked.

We have obtained orphan drug exclusivity for JUXTAPID in the U.S. for the treatment of HoFH (which expires in December 2019). JUXTAPID's new chemical entity exclusivity expired on December 21, 2017, and, as such, since December 21, 2016, an Abbreviated New Drug Application ("ANDA") or 505(b)(2) NDA may be submitted for JUXTAPID if it contains a Paragraph IV certification of patent invalidity or non-infringement. If we instigate a suit against an ANDA or 505(b)(2) applicant for patent infringement within 45 days of receiving a Paragraph IV notice, the FDA is prohibited from approving the ANDA or 505(b)(2) application for a period of 30 months. The FDA may approve the proposed competitor product before the expiration of the 30-month stay if a court finds our patents invalid or not infringed or if the court shortens the period because the parties have failed to cooperate in expediting the litigation.

Moreover, if one or more ANDA filers were to receive approval to sell a generic or follow-on version of JUXTAPID, those competitor products could potentially be marketed as early as December 21, 2019 (the date on which JUXTAPID's orphan drug exclusivity ends) and JUXTAPID would become subject to increased competition at that time. The FDA's approval letter for the modified JUXTAPID REMS program, received on January 3, 2017, specified that an authorized generic drug under the JUXTAPID NDA must have an FDA-approved REMS program prior to marketing.

*Metreleptin*. Our metreleptin patent portfolio consists of two issued U.S. patents and issued patents in Europe, Canada, Israel, Australia, New Zealand, Mexico, China, South Korea and Japan, all of which have been licensed to us. The U.S. and non-U.S. patents directed to the composition of matter of metreleptin have expired. The patent family covering metreleptin methods of use is co-owned by Amgen, University of Texas and the NIH, and is licensed to us from Amgen. We have consent from each additional co-owner to the sublicense granted by Amgen and have also obtained a direct in-license from one of the other co-owners of this patent to its rights under the patent family. The U.S. method of use patent directed to treating lipoatrophy has been elected for a 1,445-day patent term extension that will extend the expiration date to 2027. The U.S. method of use patent directed to treating diabetes associated with lipoatrophy and the non-U.S. method of use patents issued in certain European countries, Canada, and Australia, and pending in Japan, expire in 2022. In the U.S., metreleptin qualifies for 12-year biologic exclusivity under the Biologics Price Competition and Innovation Act (the "BPCI Act"), which will expire in 2026. If approved by the EMA, metreleptin would be entitled to 10 years of market and orphan drug exclusivity in the EU.

#### ***Other Patents, Trademarks and Proprietary Rights***

In addition to patent protection, we also rely on trade secrets, proprietary know-how and continuing technological innovation to develop and maintain a competitive position in our product areas. We require our potential business collaborators, clinical investigators, sponsored researchers, employees and consultants who might have access to or be provided with proprietary information to sign confidentiality agreements. We have included information about risks and uncertainties relating to protection of our proprietary information in the "Risk Factors" section of this Annual Report.

We or Aegerion own registered trademarks including Novelion<sup>®</sup>, Aegerion<sup>®</sup>, JUXTAPID<sup>®</sup>, LOJUXTA<sup>®</sup>, MYALEPT<sup>®</sup> and MYALEPTA<sup>®</sup> in the U.S., EU and in other jurisdictions.

#### **Licensing**

##### ***Lomitapide***

##### ***University of Pennsylvania***

In May 2006, Aegerion entered into a license agreement with The Trustees of the University of Pennsylvania, ("UPenn") pursuant to which Aegerion obtained an exclusive, worldwide license from UPenn to certain know-how and a range of patent rights applicable to lomitapide. In particular, Aegerion obtained a license to certain patents and patent applications owned by UPenn relating to the dosing of MTP-I compounds, including lomitapide, and certain patents and patent applications and know-how covering the composition of matter of lomitapide that were assigned to UPenn by Bristol-Myers Squibb Company ("BMS") for use either as a monotherapy or with other dyslipidemic therapies to treat patients with HoFH. Aegerion also has the right to use lomitapide in the field of monotherapy or in combination with other dyslipidemic therapies for treatment of patients with other severe forms of hypercholesterolemia unable to come within 15% of NCEP's LDL-C goal on maximal tolerated oral therapy, as determined by the patient's prescribing physician, or with severe combined hyperlipidemia unable to come within 15% of NCEP's non-HDL-C goal on maximal tolerated oral therapy, as determined by the patient's prescribing physician, or with severe

hypertriglyceridemia unable to reduce triglycerides ("TG") <1,000 on maximal tolerated therapy. We refer to the patents and patent applications assigned by BMS to UPenn and licensed to Aegerion by UPenn as the BMS-UPenn assigned patents.

To the extent that rights under the BMS-UPenn assigned patents were not licensed to Aegerion under Aegerion's license agreement with UPenn or were retained by UPenn for non-commercial education and research purposes, those rights, other than with respect to lomitapide, were licensed by UPenn back to BMS on an exclusive basis pursuant to a technology donation agreement between UPenn and BMS. In the technology donation agreement, BMS agreed not to develop or commercialize any compound, including lomitapide, covered by the composition of matter patents included in the BMS-UPenn assigned patents in the field licensed to Aegerion exclusively by UPenn. Through Aegerion's license with UPenn, as provided in the technology donation agreement, Aegerion has the exclusive right with respect to the BMS-UPenn assigned patents regarding their enforcement and prosecution in the field licensed exclusively to Aegerion by UPenn.

The license from UPenn covers, among other things, the development and commercialization of lomitapide alone or in combination with other active ingredients in the licensed field. The license is subject to customary non-commercial rights retained by UPenn for non-commercial educational and research purposes. Aegerion may grant sublicenses under the license, subject to certain limitations. Aegerion is required to make royalty payments to UPenn at a range of royalty rates in the high single digits on net sales of lomitapide in countries where lomitapide has patent protection, and of any other products covered by the license (subject to a variety of customary reductions), and share with UPenn specified percentages of sublicensing royalties and certain other consideration that Aegerion receives under any sublicenses that Aegerion may grant. Aegerion made royalty payments to UPenn through November 29, 2016. There were no royalty payments made to UPenn during the period from November 30, 2016 to December 31, 2016. In 2017, Aegerion made \$3.1 million of royalty payments to UPenn and had \$0.7 million in royalties payable to UPenn as of December 31, 2017. Aegerion will be required to make development milestone payments to UPenn of up to an aggregate of approximately \$2.6 million if we decide to develop lomitapide for indications within the licensed field other than HoFH, and we achieve certain milestones in such development efforts. All such development milestone payments for these other indications are payable only once, no matter how many licensed products for these other indications are developed.

The license agreement with UPenn will remain in effect on a country-by-country basis until the expiration of the last-to-expire licensed patent right covering the product in the applicable country. Aegerion has the right to terminate this license agreement for convenience upon 60 days prior written notice to UPenn or for UPenn's uncured material breach of the license agreement. UPenn may terminate this license agreement for Aegerion's uncured material breach of the license agreement, uncured failure to make payments to UPenn or if Aegerion is the subject of specified bankruptcy or liquidation events.

### ***Metreleptin***

#### *Amgen Inc.*

In connection with Aegerion's acquisition of MYALEPT in January 2015, Aegerion acquired a license agreement between Amgen and Amylin Pharmaceuticals, Inc., dated February 7, 2006 (the "Amgen License") pursuant to which Aegerion obtained an exclusive worldwide license from Amgen to certain know-how and patents and patent applications covering the composition of matter and methods of use of metreleptin to develop, manufacture and commercialize a preparation containing metreleptin (the "Amgen Licensed Products").

As part of the Amgen License, Aegerion also obtained an exclusive sublicense of Amgen's exclusive rights to certain metreleptin-related patents and patent applications owned by the Rockefeller University and exclusively licensed to Amgen under a license agreement dated April 14, 1995, as amended (the "Rockefeller License") and an exclusive sublicense of Amgen's non-exclusive rights to certain metreleptin-related patents and patent applications owned by The Regents of the University of California and non-exclusively licensed to Amgen under a license agreement dated July 13, 2005 (the "UCSF License"). Amgen retains rights to conduct research, development, manufacturing and commercialization activities with respect to products other than the Amgen Licensed Products.

Aegerion may grant sublicenses under the licenses and sublicenses granted by Amgen, subject to certain limitations, including Amgen's right of first offer for any out-license, partnership, co-development, commercialization, co-promotion or similar agreement related to metreleptin or the Amgen Licensed Products, which expires in February 2021. Under this license agreement, Amgen must notify Aegerion of any potential third-party partnership regarding any intellectual property rights controlled by Amgen in the neurology field and Aegerion will have a right of first negotiation for any license, partnership, co-development, commercialization, co-promotion or similar agreement, which expires in February 2021.

Aegerion is required to make royalty payments to Amgen on net sales of each Amgen Licensed Product on a country-by-country basis (i) at a royalty rate in the low double digits where the Amgen Licensed Product has patent protection or market

exclusivity granted by a regulatory authority at the time of regulatory approval in the applicable country during the applicable royalty term, which runs on a country-by-country basis until the later of (a) the expiration of the last-to-expire valid claim covering an Amgen Licensed Product in the applicable country, (b) expiration of any market exclusivity granted by a regulatory authority, and (c) ten years from the date on which an Amgen Licensed Product is first sold to a third party in a country after regulatory approval for the Amgen Licensed Product has been granted in such country ("Amgen Royalty Term") or (ii) at a royalty rate in the mid-single digits to low double digits where the Amgen Licensed Product receives patent protection or market exclusivity following the time of regulatory approval in the applicable country, in either case subject to a variety of customary reductions.

Under the Amgen License, Aegerion is also required to directly meet certain payment obligations under the Rockefeller License and UCSF License. Aegerion is required to make royalty payments to Rockefeller University on net sales of each product with patent rights or know-how in the field of obesity genes, obesity gene products, and molecules that modulate or mediate their action and/or regulation on a country-by-country basis at a range of royalty rates in the low single digits depending on whether the product has an orphan product designation or not until the later to occur of expiration of (i) patent protection, (ii) any market exclusivity period granted in the applicable country, or (iii) any data exclusivity period in the applicable country (with certain limitations related to the number of units sold). In February 2015, Aegerion paid a one-time \$5.0 million milestone payment to Rockefeller University, which was due twelve months following the receipt of marketing approval for MYALEPT in the U.S. Aegerion is also required to pay to Rockefeller University a percentage in the low double digits of any upfront license fees or one-time fees Aegerion receives in consideration for any sublicense of the licensed rights. There are no material payment obligations outstanding under the UCSF License. Also, in connection with the acquisition of metreleptin, Aegerion entered into a letter agreement with AstraZeneca pursuant to which Aegerion agreed to make royalty payments payable by AstraZeneca and its affiliates to BMS with respect to net sales of metreleptin in the U.S. The time-based royalty rate ranges from mid-single digits to low double digits, increasing annually in years 2016 to 2019 from rates in the low single digits to low double digits, peaking in years 2019 to 2020 at a rate in the low double digits before decreasing in years 2022 through 2025 to rates in the high single digits to mid-single digits. The royalty obligation to BMS terminates in 2026.

The Amgen License will terminate upon the expiration of the last Amgen Royalty Term for any Amgen Licensed Product. Aegerion has the right to terminate the Amgen License for convenience upon 90 days prior written notice to Amgen or for Amgen's uncured material breach of the Amgen License, or becoming subject to specified bankruptcy or liquidation events. Amgen may terminate the Amgen License for Aegerion's uncured failure to make payments to Amgen or if Aegerion is the subject of specified bankruptcy or liquidation events.

Aegerion made royalty payments to Amgen, Rockefeller University and BMS related to the sales of MYALEPT through November 29, 2016. There were no royalty payments made to these parties from November 30, 2016 to December 31, 2016. In 2017, Aegerion made aggregate royalty payments of \$9.7 million to Amgen, Rockefeller University and BMS, and had \$2.9 million in aggregate royalties payable to these parties as of December 31, 2017.

*Shionogi & Co., Ltd.*

In connection with Aegerion's acquisition of MYALEPT in January 2015, Aegerion acquired a license agreement between Shionogi and Amylin Pharmaceuticals, Inc., dated July 8, 2009 pursuant to which Shionogi was granted an exclusive sublicense to the patent rights licensed under the Amgen License and the Rockefeller License to develop and commercialize the Amgen Licensed Products and know-how for use in the treatment of lipodystrophy in humans in Japan, South Korea and Taiwan (the "Shionogi Territory"). This license agreement does not provide Shionogi with manufacturing rights. Shionogi may grant further sublicenses under the license, subject to certain limitations.

The license agreement requires that Shionogi use commercially reasonable efforts to develop, obtain regulatory approvals for, and commercialize the Amgen Licensed Products in the Shionogi Territory. Shionogi is required to make royalty payments to Aegerion on net sales of each Amgen Licensed Product at a range of royalty rates in the mid-to high-single digits dependent on the amount of net sales. Shionogi made royalty payments to Aegerion related to sales of MYALEPT in Japan through November 29, 2016. During the period from November 30, 2016 to December 31, 2016, Aegerion did not receive any royalty payments from Shionogi. In 2017, the total amount of royalty payments received from Shionogi was \$0.1 million. Shionogi will be required to make milestone payments to Aegerion of up to an aggregate of approximately \$25.0 million if and when Shionogi achieves certain commercialization milestones. Such milestone payments are payable only once. Under the license agreement, Shionogi has also agreed to directly comply with the payment obligations under the Rockefeller License and Amgen License, as set forth under those agreements, relating to its activities under this license agreement.

The license agreement with Shionogi will terminate upon the expiration of the last Amgen Royalty Term for any Amgen Licensed Product with respect to which Shionogi has a license under this license agreement. Aegerion has the right to terminate this license agreement for Shionogi's uncured material breach of the license agreement, failure to make any payment due to

Aegerion, a procedural default, or becoming subject to specified bankruptcy or liquidation events. Shionogi may terminate this license agreement for Aegerion's uncured material breach of this license agreement, failure to make payments due to Shionogi, or if Aegerion is the subject of specified bankruptcy or liquidation events, or if Shionogi determines it is not feasible to develop, launch or sell the Amgen Licensed Products due to scientific, technical, regulatory or commercial reasons. Aegerion may also terminate this license agreement at any time without cause by exercising our buy-back option for a one-time fee to Shionogi equal to (i) a number in the low single digits times the amount of expenses and fees incurred by Shionogi in developing the Amgen Licensed Products plus (ii) an amount no more than a number in the mid-double digits times monthly net sales of the Amgen Licensed Products by Shionogi in the month the option is exercised.

## **Government Regulation**

Government authorities in the U.S., at the federal, state and local level, the EU, EU Member States, and in other countries extensively regulate, among other things, the research, development, testing, manufacture, quality control, approval, labeling, packaging, storage, record-keeping, promotion, advertising, distribution, post-approval monitoring and reporting, marketing and export and import of lomitapide and metreleptin. In the future, if we were to develop or acquire any other products, or any product candidates, they would also be subject to such regulations and oversight. Our products must be approved by the FDA through the NDA or the BLA process before they may be legally marketed in the U.S., and must be approved by foreign regulatory authorities via various procedures before they can be marketed in the applicable country, including the EMA or the regulatory authorities of the EU Member States before they can be placed on the market in the EU.

### ***U.S. Drug and Biologic Development Process***

In the U.S., the FDA regulates drugs under the FDCA and implementing regulations, and regulates biologics under both the FDCA and the Public Health Service Act ("PHSA"). The process of obtaining marketing approvals and the subsequent compliance with appropriate federal, state, local, and foreign statutes and regulations require the expenditure of substantial time and financial resources. Failure to comply with the applicable U.S. requirements at any time during the product development process, approval process or after approval, may subject a company to administrative or judicial sanctions. These sanctions could include, among other things, the FDA's refusal to approve pending applications, withdrawal of an approval, imposition of a clinical hold, issuance of warning letters and other types of enforcement-related letters, product recalls, product seizures, changes to the conditions surrounding marketing approval such as labeling changes or changes to a REMS program, total or partial suspension of production or distribution, injunctions, civil money penalties, fines, refusals of government contracts, debarment, restitution, disgorgement of profits, or civil or criminal investigations and penalties. Any agency or judicial enforcement action could have a material adverse effect on us.

The process required by the FDA before a drug or biologic may be marketed in the U.S. is extensive and generally involves the following:

- completion of preclinical laboratory tests, animal studies and formulation studies according to Good Laboratory Practices ("GLP") and other applicable regulations;
- submission to the FDA of an investigational new drug application ("IND"), which must become effective before human clinical trials may begin;
- performance of human clinical trials, including adequate and well-controlled trials, according to Good Clinical Practices ("GCP") to establish the safety and efficacy of the proposed drug for its intended use, or the safety, purity, and potency of a biological product;
- approval by an independent Institutional Review Board ("IRB"), representing each clinical site before each clinical trial may be initiated;
- submission to the FDA of an NDA or BLA;
- completion of registration batches and validation of the manufacturing process to show that we are capable of consistently producing quality batches of product;
- satisfactory completion of an FDA inspection of the manufacturing facility or facilities at which the product is produced to assess compliance with cGMP to assure that the facilities, methods and controls are adequate to preserve the drug's identity, strength, quality and purity; and

- FDA review and approval of the NDA or BLA.

Once a pharmaceutical candidate is identified for development it enters the preclinical testing stage. Preclinical tests include laboratory evaluations of product chemistry, toxicity and formulation, as well as animal studies, to assess the safety and quality of the product. Animal studies must be performed in compliance with the FDA's GLP regulations and the U.S. Department of Agriculture's Animal Welfare Act. Human clinical trials cannot commence until an IND application is submitted and becomes effective. An IND sponsor must submit the results of the preclinical tests, together with manufacturing information and analytical data, to the FDA as part of the IND. The sponsor also will include a protocol detailing, among other things, the objectives of the first phase of the clinical trial, the parameters to be used in monitoring safety and the effectiveness criteria to be evaluated, if the first phase lends itself to an efficacy evaluation. Preclinical testing may continue after the IND is submitted. The IND automatically becomes effective 30 days after receipt by the FDA, unless the FDA, within the 30-day time period, places the clinical trial on a clinical hold. In such a case, the IND sponsor and the FDA must resolve any outstanding concerns before the clinical trial can begin. Clinical holds also may be imposed by the FDA at any time before or during studies due to safety concerns or non-compliance, or other reasons.

All clinical trials must be conducted under the supervision of one or more qualified investigators. The conduct of clinical trials is subject to extensive regulation, including the FDA's bioresearch monitoring regulations and GCP requirements, which establish standards for conducting, recording data from, and reporting the results of, clinical trials, and are intended to assure that the data and reported results are credible and accurate, and that the rights, safety, and well-being of study participants are protected. These regulations include the requirement that all research subjects provide informed consent. Further, an IRB must review and approve the plan for any clinical trial before it commences at any institution. An IRB considers, among other things, whether the risks to individuals participating in the trials are minimized and are reasonable in relation to anticipated benefits. The IRB also approves the information regarding the trial and the consent form that must be provided to each trial subject or his or her legal representative, and continues to provide oversight of the study until it is completed. Additionally, companies sponsoring the clinical trials, investigators, and IRBs also must comply with regulations and guidelines for complying with the protocol and investigational plan, adequately monitoring the clinical trial, and timely reporting of adverse events. Foreign studies conducted under an IND must meet the same requirements that apply to studies being conducted in the U.S. Data from a foreign study not conducted under an IND may be submitted in support of an NDA or BLA if the study was conducted in accordance with GCP and the FDA is able to validate the data.

Each new clinical protocol must be submitted for FDA review, and to the IRBs for approval. Protocols detail, among other things, the objectives of the study, the primary and secondary endpoints of the study, dosing procedures, subject selection and exclusion criteria, and the parameters to be used to monitor subject safety.

Human clinical trials are typically conducted in three sequential phases that may overlap or be combined:

- *Phase 1* . The investigational drug is initially introduced into healthy human subjects, and tested for safety, dosage tolerance, absorption, metabolism, distribution and excretion. In the case of some products for severe or life-threatening diseases, especially when the product may be too inherently toxic to ethically administer to healthy volunteers, the initial human testing may be conducted in patients with the target diseases.
- *Phase 2* . This phase involves trials in a limited patient population to identify possible adverse effects and safety risks, to preliminarily evaluate the efficacy of the product for specific targeted diseases and to determine dosage tolerance and optimal dosage.
- *Phase 3* . This phase involves trials undertaken after preliminary evidence of effectiveness has been obtained and is intended to further evaluate dosage and clinical efficacy and safety of the drug, or the safety, purity, and potency of a biological product, in an expanded patient population, often at geographically dispersed clinical trial sites. These trials are intended to establish the overall risk/benefit ratio of the product, and to provide an adequate basis for product approval and product labeling.

Progress reports detailing developments associated with the clinical testing program must be submitted at least annually to the FDA, and safety reports must be submitted to the FDA and the investigators for serious and unexpected adverse events or animal test results that suggest a significant risk to human subjects. Phase 1, Phase 2, and Phase 3 testing may not be completed successfully within any specified period, if at all. The FDA or the sponsor may suspend or terminate a clinical trial at any time on various grounds, including a finding that the research subjects or patients are being exposed to an unacceptable health risk. Similarly, an IRB can suspend or terminate approval of a clinical trial at its institution if the clinical trial is not being conducted in accordance with the IRB's requirements or if the drug has been associated with unexpected serious harm to patients. Further, success in either preclinical studies or early-stage clinical trials does not assure success in later-stage clinical trials. Sponsors of certain interventional

clinical trials, except for Phase 1 trials, are required to submit certain clinical trial information for inclusion in the public clinical trial registry and results data bank maintained by the NIH, which are publicly available at <http://clinicaltrials.gov>.

Concurrent with clinical trials, companies usually complete additional studies in non-human models, and must also develop additional information about the chemistry and physical characteristics of the product, and finalize a process for manufacturing the product in commercial quantities in accordance with cGMP requirements.

The manufacturing process must be capable of consistently producing quality batches of the product candidate and, among other things, the manufacturer must develop methods for testing the identity, strength, quality and purity of the final product. Additionally, appropriate packaging must be selected and tested, and stability studies must be conducted to demonstrate that the product candidate does not undergo unacceptable deterioration over its shelf life.

### ***U.S. Review and Approval Processes***

The results of product development, preclinical studies and clinical trials, along with descriptions of the manufacturing process, analytical tests conducted on the chemistry of the product, proposed labeling, and other relevant information are submitted to the FDA as part of an NDA or BLA requesting approval to market the product. The submission of an NDA or BLA generally is subject to the payment of a user fee, although NDAs or BLAs for designated orphan drugs are exempt from this fee.

In addition, under the Pediatric Research Equity Act of 2007, as amended ("PREA"), an application or supplement to an application for a drug with certain novel features (e.g., new active ingredient, new indication, new dosage form) must contain data to assess the safety and effectiveness of the drug or biologic for the claimed indications in all relevant pediatric subpopulations, and to support dosing and administration for each pediatric subpopulation for which the drug is safe and effective. The FDA may grant deferrals or full or partial waivers for submission of this data. Unless otherwise required by regulation, PREA does not apply to any drug or biologic for an indication for which orphan designation has been granted.

The FDA conducts a preliminary review of all NDAs and BLAs submitted to ensure that they are sufficiently complete for substantive review before it accepts them for filing. The FDA may request additional information rather than accept an application for filing. In this event, the application must be resubmitted with the additional information. The resubmitted application also is subject to review before the FDA accepts it for filing. Once the submission is accepted for filing, the FDA begins an in-depth substantive review. The FDA is required to refer an NDA for a new chemical entity ("NCE") to an advisory committee for review, evaluation and recommendation as to whether the application should be approved and under what conditions, or explain why such review is not necessary. Other NDAs or BLAs may also be referred to an advisory committee for evaluation and recommendation. The FDA is not bound by the recommendation of an advisory committee, but it frequently follows such recommendations. The approval or licensure process is lengthy and difficult, and the FDA may refuse to approve an NDA or BLA if the applicable regulatory criteria are not satisfied or may require additional clinical or other data and information. Even if such data and information are submitted, the FDA ultimately may decide that the application does not satisfy the regulatory criteria for approval or licensure. Data obtained from clinical trials are not always conclusive; and the FDA may interpret data differently than we interpret the same data. The FDA may issue a complete response letter, which generally outlines the deficiencies in the submission and may require additional clinical or other data or impose other conditions that must be met in order to secure final approval of the application. The FDA reviews an application to determine, among other things, whether a drug is safe and effective for its intended use, or whether a biologic is safe, pure, and potent, and whether its manufacturing is cGMP-compliant to assure and preserve the product's identity, strength, quality and purity. Before approving an NDA or BLA, the FDA will typically inspect the facility or facilities where the product is manufactured. In addition, the FDA often will conduct a biosearch monitoring inspection of the clinical trial sites involved in conducting pivotal studies to ensure data integrity and compliance with applicable GCP requirements.

Applications receive either standard or priority review. A product representing a major advance in treatment or treatment where no adequate therapy exists may receive priority review. Under the goals and policies agreed to by the FDA under the Prescription Drug User Fee Act ("PDUFA"), FDA has ten months in which to complete its initial review of a standard new molecular entity ("NME") NDA or original BLA and six months for a priority review NME NDA, BLA, or efficacy supplement. The FDA does not always meet its PDUFA goal dates and in certain circumstances the PDUFA goal date may be extended. In addition, products studied for their safety and effectiveness in treating serious or life-threatening illnesses and which provide meaningful therapeutic benefit over existing treatments, may receive accelerated approval. In that situation, the product may be approved on the basis of adequate and well-controlled clinical trials establishing that the product has an effect on a surrogate endpoint that is reasonably likely to predict clinical benefit or on the basis of an effect on a clinical endpoint other than survival or irreversible morbidity. As a condition of approval, a sponsor of a drug or biologic receiving accelerated approval must perform post-marketing studies to validate the surrogate endpoint or otherwise confirm the effect of the product on a clinical endpoint, and the product may be subject to accelerated withdrawal procedures. Priority review and accelerated approval do not change the standards for approval, but may expedite the approval process for certain products.

If a product receives marketing approval, the approval may be significantly limited to specific diseases, dosages or patient populations, or the indications for use may otherwise be limited, which could restrict the commercial value of the product. In addition, the FDA may impose distribution and use restrictions and other limitations on labeling and communication activities with respect to an approved product via a REMS program, to mitigate serious risks, which could include Medication Guides, patient package inserts, physician communication plans, and/or ETASU. ETASU may include restricted distribution methods, patient registries and other risk minimization tools. Because of the risk of liver toxicity, JUXTAPID is available in the U.S. only through a REMS program, which program was modified and approved by the FDA on January 3, 2017. The goal of the modified JUXTAPID REMS program, as discussed earlier in this “*Business*” section of this Annual Report, is to mitigate the risk of hepatotoxicity associated with the use of JUXTAPID by ensuring that: (a) prescribers are educated about the approved indication for JUXTAPID, the risk of hepatotoxicity associated with the use of JUXTAPID, and the need to monitor patients during treatment with JUXTAPID as per product labeling; (b) JUXTAPID is dispensed only to patients with a clinical or laboratory diagnosis consistent with homozygous familial hypercholesterolemia; and (c) patients are informed about the risk of hepatotoxicity associated with the use of JUXTAPID and the need for baseline and periodic monitoring. The ETASU in the modified JUXTAPID REMS program approved by the FDA on January 3, 2017 has been significantly enhanced and imposes significant requirements on healthcare professionals, pharmacies, and patients. See the “*Business-Lomitapide*” section of this Annual Report for further information regarding the modified JUXTAPID REMS program. MYALEPT is also subject to a REMS program, due to the risks of serious adverse sequelae, as a result of the development of anti-metroleptin antibodies that neutralize endogenous leptin and/or MYALEPT, and the risk of lymphoma. The MYALEPT REMS program aims to educate prescribers about these risks and to restrict access to MYALEPT by requiring prescriber certification, pharmacy certification, and prescriber attestation that each patient has a diagnosis consistent with GL. The REMS programs for each product restrict distribution and sales of our products and impose ongoing implementation requirements that could be burdensome or costly.

#### *The Hatch-Waxman Act, Marketing Exclusivity in the U.S. and Patent Term Restoration*

The Drug Price Competition and Patent Term Restoration Act of 1984, or the Hatch-Waxman Act, established two abbreviated approval pathways for drug products that are in some way follow-on versions of already approved NDA products.

*Generic Drugs* . A generic version of an approved drug is approved by means of an ANDA through which the sponsor demonstrates that the proposed product is identical or bioequivalent to the approved, brand-name drug, referred to as the Reference Listed Drug (“RLD”). Generally, an ANDA must contain data and information showing that the proposed generic product and RLD have the same active ingredient(s), in the same strength and dosage form, to be delivered via the same route of administration; are intended for the same uses; have the same labeling; and are bioequivalent. An ANDA need not independently demonstrate the proposed product’s safety and effectiveness; rather the proposed product’s safety and effectiveness are inferred from the fact that the product is demonstrated to be the same as, and bioequivalent to, the RLD, which the FDA previously found to be safe and effective. These drugs are commonly referred to as “generic equivalents” to the RLD, and under state law they typically can be substituted by pharmacists under prescriptions written for the RLD.

*505(b)(2) NDAs* . If a product is similar to, but not the same as, an already approved product (and thus is not eligible for submission of an ANDA), it may be submitted for approval via an NDA under FDCA section 505(b)(2). Like an ANDA, a 505(b)(2) application is permitted to rely on the FDA’s finding that the RLD is safe and effective to the extent the products share similar features, but the sponsor must submit its own product-specific safety and effectiveness data to support any differences between the proposed and reference products.

*RLD Patents* . An NDA sponsor must identify to the FDA any patents that claim the drug substance, drug product or method of using the drug. These patents are among the information about the product that is listed in the FDA publication, Approved Drug Products with Therapeutic Equivalence Evaluations (the “Orange Book”). The sponsor of an ANDA or 505(b)(2) application seeking to rely on an approved product as the RLD must make one of several certifications regarding each listed patent, and these certifications can affect the timing of approval of the ANDA or 505(b)(2) application. For example, a “Paragraph III” certification is the sponsor’s statement that it will wait for the patent to expire before obtaining approval for its product. A “Paragraph IV” certification is submitted to obtain approval of the ANDA or 505(b)(2) application before expiration of a listed patent and is an assertion that the patent is invalid, unenforceable or not infringed by the new product.

#### *Marketing Exclusivities in the U.S.*

The Hatch-Waxman Act provides periods of regulatory exclusivity for products that would serve as RLDs for an ANDA, or 505(b)(2) application. If a drug is an NCE, generally meaning that the FDA has not previously approved any other new drug containing the same active moiety, which is the molecule or ion responsible for the action of the drug substance, there is a period of five years from the product’s approval during which the FDA may not accept for filing any ANDA or 505(b)(2) application for

a drug with the same active moiety. However, an ANDA or 505(b)(2) NDA may be submitted after four years if it contains a Paragraph IV certification of patent invalidity or non-infringement. According to the Orange Book, JUXTAPID's NCE exclusivity expired on December 21, 2017, which means that an ANDA or 505(b)(2) NDA may be submitted for JUXTAPID.

A product that is not an NCE may qualify for three years of marketing exclusivity following approval, when the application contains new clinical investigations, other than bioavailability studies, conducted or sponsored by the applicant and deemed by the FDA to be essential to the approval of the application. Three-year exclusivity is often available for changes to a previously-approved drug product, such as new indications, strengths or dosage forms. This exclusivity period does not preclude filing or review of an ANDA or 505(b)(2) application; rather, the FDA is precluded from granting final approval to the ANDA or 505(b)(2) application until three years after approval of the RLD. In addition, this three-year exclusivity covers only the conditions of use associated with the new clinical investigations and, as a general matter, does not prohibit the FDA from approving ANDAs or 505(b)(2) NDAs for generic versions of the original, unmodified drug product.

Once the FDA accepts for filing an ANDA or 505(b)(2) application containing a Paragraph IV certification, the applicant must within 20 days provide notice to the RLD NDA holder and patent owner that the application with patent challenge has been submitted, and provide the factual and legal basis for the applicant's assertion that the patent is invalid, unenforceable or not infringed. If the NDA holder or patent owner files suit against the ANDA or 505(b)(2) applicant for patent infringement within 45 days of receiving the Paragraph IV notice, the FDA is prohibited from approving the ANDA or 505(b)(2) application for a period of 30 months from the date of receipt of the notice. If the RLD has NCE exclusivity and the notice is given and suit filed during the fifth year of exclusivity, the 30-month stay is extended so that it does not expire until 7 ½ years after approval of the RLD. The FDA may approve the proposed product before the expiration of the 30-month stay if, within that time period, the patent involved expires, the parties settle the lawsuit or a court finds the patent invalid or not infringed or if the court shortens the period because the parties have failed to cooperate in expediting the litigation.

Pediatric exclusivity is another type of exclusivity available in the U.S. under Section 505A of the FDCA. Pediatric exclusivity, if granted, provides an additional six months to an existing exclusivity period, including orphan drug exclusivity, or delay in approval resulting from certain patent certifications. This six-month exclusivity, which runs from the end of other exclusivity protection or patent delay, may be granted based on the voluntary completion of a pediatric trial or trials and submission of pediatric data that fairly responds to an FDA-issued "Written Request" for such a trial or trials. The data need not show the product to be safe or effective in the pediatric population studied; rather if the clinical trial is deemed to fairly respond to the FDA's request, the additional protection is granted. If reports of requested pediatric studies are submitted to and accepted by the FDA within statutory time limits, any periods of regulatory exclusivity or Orange Book-listed patent protections that cover the drug (other than a 30-month stay) are extended by six months. This is not a patent term extension, but it effectively extends the regulatory period during which the FDA cannot approve an ANDA or 505(b)(2) application owing to regulatory exclusivity or listed patents. Pediatric exclusivity, however, cannot extend any regulatory exclusivity or patent protection if the FDA makes its determination that the pediatric studies fairly respond to the Written Request later than nine months prior to the expiration of the exclusivity or patent protection period. In the first quarter of 2015, the FDA issued a Written Request for a study to evaluate lomitapide in pediatric HoFH patients, which, if completed as described, would provide for six months of pediatric exclusivity under the FDCA. In the second quarter of 2015, we decided to decline the FDA's Written Request regarding the study in pediatric HoFH patients, because we believe that the size and complexity of the requested trial created a considerable barrier to the feasibility of the study. Given that we have declined to conduct the study requested by the FDA, we will not be entitled to the six months of additional exclusivity available for conducting a study that is the subject of a Written Request issued by the FDA.

#### *Patent Term Restoration*

The Hatch-Waxman Act established a patent restoration term of up to five years as compensation for patent term lost during product development and the FDA regulatory review process. The maximum period of restoration is five years and cannot extend the remaining term of a patent beyond a total of 14 years from the product's approval date. The patent term restoration period is generally one-half the time between the effective date of an IND, and the submission date of an NDA, plus the time between the submission date of an NDA and the approval of that application. Only one patent applicable to an approved drug is eligible for the extension, and the extension must be applied for prior to expiration of the patent and within 60 days of the NDA approval. The United States Patent and Trademark Office ("PTO"), in consultation with the FDA, reviews and approves the application for any patent term extension or restoration. A five-year patent term extension has been granted for our U.S. patent covering the composition of matter of lomitapide, extending the patent term to 2020 from the originally scheduled expiration of early 2015. With respect to metreleptin, the U.S. method-of-use patent directed to treating lipotrophy has been elected for a 1,445-day patent term extension that will extend its expiration date to 2027.



The BPCI Act, enacted in 2010 as part of the Patient Protection and Affordable Care Act, as amended by the Health Care and Education Reconciliation Act (as amended, the "Healthcare Reform Act"), authorizes the FDA to license a biological product that is biosimilar to, and possibly interchangeable with, a PHSA-licensed reference biological product through an abbreviated pathway. The BPCI Act establishes criteria for determining that a product is biosimilar to an already-licensed biologic (a reference product), and establishes a process by which an abbreviated BLA for a biosimilar product is submitted, reviewed and approved. The BPCI Act provides periods of exclusivity that protect a reference product from biosimilar competition. Under the BPCI Act, innovator manufacturers of original reference products are granted twelve years of exclusive use before biosimilar versions of such products can be licensed for marketing in the U.S. This means that the FDA may not approve an application for a biosimilar version of a reference product until twelve years after the date of approval of the reference product (with a potential six-month extension of exclusivity if certain pediatric studies are conducted and the results reported to FDA), although a biosimilar application may be submitted four years after the date of licensure of the reference product. Additionally, the BPCI Act establishes procedures by which the biosimilar applicant must provide information about its application and product to the reference product sponsor, and by which information about potentially relevant patents is shared and litigation over patents may proceed in advance of approval. The BPCI Act also provides a period of exclusivity for the first biosimilar to be determined by the FDA to be interchangeable with the reference product. We believe that under the BPCI Act, metreleptin has 12 years of exclusivity in the U.S. from February 24, 2014, the date of the product's approval by the FDA.

The objectives of the BPCI Act are conceptually similar to those of the Hatch-Waxman Act, which established abbreviated pathways for the approval of small molecule drug products. The FDA has not yet issued proposed regulations setting forth its interpretation of the BPCI Act's provisions but has issued guidance documents related to BPCI Act implementation concerning biosimilarity and interchangeability, BLA submission requirements, and exclusivity. We anticipate that contours of the BPCI Act will be further defined through issuance of additional guidance documents by the FDA, proposed regulations, and decisions in the course of considering specific applications.

Like pediatric exclusivity applicable to drug products approved under the FDCA, pediatric exclusivity applicable to biological reference products is subject to an exception. Pediatric exclusivity will not apply to either the 12-year reference product or the seven-year orphan drug exclusivity periods if the FDA determines later than nine months prior to the expiration of such period that the study reports a BLA sponsor submitted in response to a written request for pediatric studies met the terms of that request.

The BPCI Act sets forth a complex mechanism for resolving patent disputes that involves a step-wise exchange of information prior to the initiation of a patent infringement lawsuit against a biosimilar or interchangeable product sponsor. Unlike the Hatch-Waxman Act, the BPCI Act provides no automatic stay on approval of a biosimilar or interchangeable product application based on patents.

#### *U.S. Orphan Drug Designation*

Under the Orphan Drug Act, the FDA may grant orphan drug designation to a drug or biological product intended to treat a rare disease or condition, which is generally a disease or condition that affects fewer than 200,000 individuals in the U.S., or for which there is no reasonable expectation that development and production costs will be recovered from sales of the drug for such disease or condition in the U.S. Orphan drug designation must be requested before submitting an NDA or BLA. After the FDA grants orphan drug designation, the identity of the therapeutic agent and its potential orphan use are disclosed publicly by the FDA. If a product that has orphan drug designation subsequently receives FDA approval and is the first drug approved for the disease for which it has such designation, the product is entitled to orphan product exclusivity, which means that the FDA may not approve for seven years any other applications to market the same drug for the same indication, except in limited circumstances such as a demonstration that the subsequent drug is clinically superior or the inability of the existing manufacturer to supply the market. Orphan drug exclusivity also could block the approval of one of our products for seven years if a competitor obtains approval for an orphan product that the FDA finds to be the "same drug" as our product candidate for the same indication or disease. If a drug or biologic designated as an orphan drug receives marketing approval for an indication broader than the scope of its designation, it may be no longer entitled to orphan drug exclusivity. In addition to creating a 12-year period of reference product exclusivity, the BPCI Act clarifies the interaction of that exclusivity with orphan drug exclusivity, such that the licensure of a biosimilar or interchangeable version of a reference biological product that was designated and approved as an orphan drug may only occur after the later of the expiration of any applicable seven-year orphan drug exclusivity or the 12-year reference product exclusivity (or seven and one-half years and 12.5 years with pediatric exclusivity). The FDA has granted seven years of orphan drug exclusivity for JUXTAPID in the treatment of HoFH which is scheduled to expire on December 21, 2019, and seven years of orphan drug exclusivity for MYALEPT in the treatment of GL which is scheduled to expire on February 24, 2021.

### *Fast Track Designation*

The FDA's Fast Track program is intended to facilitate the development and review of drugs that are intended to treat a serious or life-threatening disease or condition and demonstrate the potential to address unmet medical needs for such a disease or condition. Under the program, the sponsor of a new drug may request that the FDA designate the drug for a specific indication as a Fast Track product concurrent with or after the filing of the IND for the product candidate. A drug that receives Fast Track designation may be eligible for more frequent meetings with the FDA to discuss the drug's development; more frequent written correspondence from the FDA about the design of the proposed clinical trials; and rolling review, meaning the sponsor may submit its NDA in sections rather than wait until the entire NDA is complete. Drugs with Fast Track designation may be more likely to become eligible for a Priority Review, which provides for FDA review of an NDA for a NME within a six-month time frame from the time the complete NDA is accepted for filing (60 days after submission), as opposed to the ten-month time frame for a Standard Review. The FDA grants Priority Review for products that demonstrate the potential to be a significant improvement in the safety or effectiveness of the treatment, prevention, or diagnosis of a serious condition.

### *Rare Pediatric Disease Priority Review Voucher*

Certain drugs may also be candidates for a rare pediatric disease designation by the FDA. In order to qualify for such designation, a sponsor must demonstrate to the FDA's satisfaction that the proposed indication is for the treatment or prevention of a rare disease or condition that affects fewer than 200,000 individuals in the U.S., or that affects more than 200,000 individuals in the U.S. and there is no reasonable expectation that the cost of developing and making available in the U.S. a drug for this type of disease or condition will be recovered from sales in the U.S. for that product, and is a serious or life-threatening disease in which the serious or life-threatening manifestations primarily affect individuals aged from birth to 18 years. Under the FDCA, a sponsor who receives approval of an NDA for a product that is for the prevention or treatment of a rare pediatric disease and meets certain additional criteria, may qualify for a rare pediatric disease priority review voucher ("PRV"). A PRV can be redeemed to receive priority review under an expedited timeframe for a subsequent marketing application for a different product. A PRV may also be sold or transferred from the initial sponsor to another sponsor. The voucher may be further transferred any number of times before it is used. Pursuant to the 21<sup>st</sup> Century Cures Act, the FDA's authority to award rare pediatric disease PRVs has been extended until 2020, and until 2022 for products that receive rare pediatric disease designation by 2020.

### *Post-Approval Requirements in the U.S.*

Once an approval is granted, products are subject to continuing regulation by the FDA. The FDA may withdraw the approval, following notice and an opportunity for a hearing, if, among other things, compliance with certain regulatory standards is not maintained or if safety or efficacy problems occur after the product reaches the market. Later discovery of previously unknown problems with a product may result in restrictions on the product or even complete withdrawal of the product from the market. If new safety issues are identified following approval, the FDA may require the NDA sponsor to take certain measures, such as revising the approved labeling to reflect the new safety information, conducting post-market studies or clinical trials to assess the new safety information, and/or implementing or changing a REMS program to mitigate newly-identified risks. These are often referred to as Phase 4 or post-marketing studies, and may involve additional clinical trials, nonclinical testing and surveillance programs to monitor the safety of approved products which have been commercialized. After approval, most changes to the approved product, such as adding new indications, manufacturing changes and additional labeling claims, are subject to prior FDA review and approval. Manufacturers and other entities involved in the manufacture and distribution of approved drugs or licensed biologics are required to register their establishments with the FDA and certain state agencies, and are subject to periodic unannounced inspections by the FDA and certain state agencies for compliance with cGMP requirements and other laws. In its approval of JUXTAPID, the FDA required three post-marketing requirements; the MYALEPT approval was associated with 15 post-marketing requirements and commitments. See *Marketed Products - Lomitapide - Clinical Development and Post-Marketing Commitments* and *Marketed Products - Metreleptin - Clinical Development and Post-Marketing Commitments* for details of our post-marketing commitments to applicable regulatory authorities.

Companies engaged in manufacturing drug products or their components must comply with applicable cGMP requirements and product-specific regulations enforced by the FDA and other regulatory agencies. Compliance with cGMP includes adhering to requirements relating to organization of personnel, buildings and facilities, equipment, control of components and drug product containers and closures, production and process controls, packaging and labeling controls, holding and distribution, laboratory controls, and records and reports. The FDA regulates and inspects equipment, facilities, and processes used in manufacturing pharmaceutical products prior to approval. If, after receiving approval, a company makes a material change in manufacturing equipment, location, or process (all of which are, to some degree, incorporated in the NDA or BLA), additional regulatory review and approval may be required. Sponsors of approved products also are subject to significant annual program user fees.

### *Packaging and Distribution in the U.S.*

Our products are made available to authorized users of the Federal Supply Schedule ("FSS") of the General Services Administration; as a result, additional laws and requirements apply. For example, our products meet applicable child-resistant packaging requirements in compliance with the U.S. Poison Prevention Packaging Act. Manufacturing, sales, promotion and other activities also are potentially subject to federal and state consumer protection and unfair competition laws.

The distribution of pharmaceutical products is subject to additional requirements and regulations, including extensive record-keeping, licensing, storage and security requirements intended to prevent the unauthorized sale of pharmaceutical products.

The FDA also conducts regular, periodic visits to re-inspect equipment, facilities, and processes following the initial approval of a product. Failure to comply with applicable cGMP requirements and conditions of product approval may lead the FDA to take enforcement actions, which may range from issuing a warning letter to seeking sanctions, including fines, civil penalties, injunctions, suspension of manufacturing operations, operating restrictions, withdrawal of FDA approval, seizure or recall of products, and criminal prosecution. Accordingly, manufacturers must continue to spend time, money and effort to maintain cGMP compliance. We cannot be certain that our present or future third-party manufacturers will consistently comply with cGMP and other applicable FDA regulatory requirements.

In addition, companies manufacturing or distributing drug products pursuant to FDA approvals are subject to record-keeping requirements; requirements on reporting of adverse experiences with the drug, and providing the FDA with updated safety and efficacy or safety, purity, and potency information for drugs and biologics, respectively; compliance within REMS program reporting obligations; drug and biologic sampling and distribution requirements; compliance with certain electronic records and signature requirements, and compliance with FDA promotion and advertising requirements. As discussed more fully below, the FDA strictly regulates labeling, advertising, promotion and other types of information regarding marketed products that are placed on the market. Drugs and biologics may be promoted only for their approved indications and in accordance with the provisions of the approved labeling.

From time to time, legislation is drafted, introduced and passed in Congress that could significantly change the statutory provisions governing the testing, approval, manufacturing and marketing of products regulated by the FDA. In addition to new legislation, FDA regulations and guidance are often revised or interpreted by the agency in ways that may significantly affect our business and our products. Changes in regulations, statutes or the interpretation of existing regulations could impact our business in the future by requiring, for example: (i) changes to our manufacturing arrangements; (ii) additions or modifications to product labeling; (iii) the recall or discontinuation of our products; or (iv) additional record-keeping requirements.

### ***Other Regulatory and Liability Matters***

Manufacturing, sales, promotion and other activities following product approval are also subject to regulation by numerous regulatory authorities in the U.S. in addition to the FDA, including the Centers for Medicare & Medicaid Services ("CMS"), other divisions of the Department of Health and Human Services ("DHHS"), the DOJ, the Drug Enforcement Administration, the Consumer Product Safety Commission, the Federal Trade Commission, the Occupational Safety & Health Administration, the Environmental Protection Agency and state and local governments.

We may be subject to numerous environmental, health and safety laws and regulations, including those governing laboratory procedures and the handling, use, storage, treatment and disposal of hazardous materials and wastes. From time to time and in the future, our operations may involve the use of hazardous and flammable materials, including chemicals and biological materials, and may also produce hazardous waste products. Even if we contract with third parties for the disposal of these materials and waste products, we cannot completely eliminate the risk of contamination or injury resulting from these materials.

We maintain workers' compensation insurance to cover us for costs and expenses we may incur due to injuries to our employees, but this insurance may not provide adequate coverage against potential liabilities. However, we do not maintain insurance for environmental liability or toxic tort claims that may be asserted against us.

### ***Foreign Regulation***

In addition to regulations in the U.S., our business is subject to a variety of foreign regulations governing clinical trials and commercial sales and distribution of our products. We must obtain approval of a product by the regulatory authorities of foreign countries before we can commence clinical trials or marketing of the product in those countries. In addition, the requirements governing the conduct of clinical trials, product licensing, pricing and reimbursement vary greatly from country to country. For example, in the EU, the conduct of clinical trials is currently governed by the EU Clinical Trials Directive 2001/20/EC, which

imposes obligations and procedures that are similar to those provided in applicable U.S. laws. The EU Good Clinical Practice and EU Good Laboratory Practice standards must also be respected during the conduct of the trials. Prior to commencement of a clinical trial in an EU Member State, an application for authorization of a clinical trial must be submitted to the regulatory authority and the relevant Ethics Committee of the relevant EU Member State in which the clinical trial will take place. The regulatory authorities of the EU Member States in which the clinical trial will be conducted must authorize the conduct of the trial, and the independent Ethics Committee must grant a positive opinion in relation to the conduct of the clinical trial in the relevant EU Member State before the commencement of the trial. Any substantial changes to trial protocol or other information submitted with the clinical trial applications must be submitted to or approved by the relevant regulatory authorities and ethics committees. However, under the new EU Clinical Trials Regulation No. 536/2014, which is expected to take effect in late 2018, a more harmonized procedure will apply, with clinical trial authorization and other applications or notifications being submitted through a centralized EU clinical trials portal.

The approval process for clinical trials in other countries outside the U.S. and the EU varies from country to country, and the time may be longer or shorter than that required for FDA approval. In all cases, clinical trials are conducted in accordance with GCP and the applicable regulatory requirements and the ethical principles that have their origin in the Declaration of Helsinki.

#### *EU NCE Exclusivity*

In the EU, new chemical entities, sometimes referred to as new active substances, qualify for eight years of data exclusivity upon marketing authorization and an additional two years of market exclusivity. The data exclusivity, if granted, prevents regulatory authorities in the EU from referencing the innovator's data to assess a generic application for eight years, after which generic marketing authorization can be submitted, and the innovator's data may be referenced, but not approved for two years. The overall ten-year period will be extended to a maximum of 11 years if, during the first eight years of those ten years, the marketing authorization holder obtains an authorization for one or more new therapeutic indications which, during the scientific evaluation prior to their authorization, are determined to bring a significant clinical benefit in comparison with currently approved therapies. Lomitapide has eight years' data exclusivity and ten years' marketing exclusivity in the EU from July 31, 2013, the date of the EC's approval of lomitapide. If approved by the EMA, metreleptin would be entitled to 10 years of market exclusivity by the EU.

#### *EU Drug Review and Approval*

To obtain marketing authorization for a medicinal product in the EU, companies must submit an application for marketing authorization based on the International Conference on Harmonisation of Technical Requirements for Registration of Pharmaceuticals for Human Use ("ICH") Common Technical Document to the regulatory authorities of the EU Member States or to the EMA. Applicants need to demonstrate the quality, safety and efficacy of the medicinal product in the application for marketing authorization. This requires applicants to conduct human clinical trials to generate the necessary clinical data. Moreover, applicants are required to include, as part of the application for marketing authorization, the results of all studies performed and details of all information collected in compliance with an agreed PIP approved by the Pediatric Committee ("PDCO"), or a decision by the EMA granting a product-specific or class waiver for pediatric use or deferral for the conduct of the PIP.

Medicinal products are authorized in the EU through one of several different procedures, either by the regulatory authorities of the EU Member States through the decentralized procedure, mutual recognition procedure, or national procedure, or through the centralized authorization procedure by which the EC takes a decision to grant a marketing authorization following a positive opinion by the EMA.

#### *EU Drug Marketing*

Much like the Anti-Kickback Statute prohibition in the U.S., the provision of benefits or advantages to physicians to induce or encourage the prescription, recommendation, endorsement, purchase, supply, order or use of medicinal products is also prohibited in the EU. The provision of benefits or advantages to physicians is governed by the national anti-bribery laws of EU Member States, such as the UK Bribery Act 2010 (the "UK Bribery Act"). Infringement of these laws could result in substantial fines and imprisonment.

Payments made to physicians in certain EU Member States must be publically disclosed. Moreover, agreements with physicians often must be the subject of prior notification and approval by the physician's employer, his or her professional organization and/or the regulatory authorities of the individual EU Member States. These requirements are provided in the national laws, industry codes or professional codes of conduct, applicable in the EU Member States. Failure to comply with these requirements could result in reputational risk, public reprimands, administrative penalties, fines or imprisonment.

The centralized procedure provides for the grant of a single marketing authorization by the EC that is valid for all EU Member States and three of the four EFTA States (Iceland, Liechtenstein and Norway). The centralized procedure is compulsory for certain medicinal products, including medicinal products derived from certain biotechnological processes, orphan medicinal products, advanced therapy medicinal products and products with a new active substance indicated for the treatment of certain diseases. It is optional for those products that are a significant therapeutic, scientific or technical innovation, or the authorization of which would be in the interest of public. Under the centralized procedure in the EU, the timeframe for the evaluation of a marketing authorization application by the EMA's CHMP is, in principle, 210 days from receipt of a valid application for marketing authorization. This time period excludes any clock stops when additional written or oral information is to be provided by the applicant in response to questions asked by the CHMP and if the applicant requests a re-examination of the CHMP opinion. Accelerated evaluation might be granted, following a substantiated request from the applicant, by the CHMP in exceptional cases, when a medicinal product is of a major public health interest particularly from the point of view of therapeutic innovation. Justification of what constitutes a major public interest is on a case by case basis. The justification should include the major benefits expected and present the arguments to support the claim that the medicinal product introduces new methods of therapy or improves on existing methods, thereby addressing to a significant extent the greater unmet needs for maintaining and improving public health. In this circumstance of an accelerated assessment, the opinion of the CHMP is given, in principle, within 150 days. Regardless of the assessment procedure, the opinion of the CHMP will be provided to the EC who will take the final decision on the application for centralized marketing authorization of a medicinal product. In light of the United Kingdom's ("UK") vote in 2016 to leave the EU, the so-called Brexit vote, there may be changes forthcoming in the scope of the centralized approval procedure as the terms of that exit are negotiated between the UK and the EU.

The decentralized marketing authorization procedure requires a separate application to, and leads to separate approval by, the regulatory authorities of each EU Member State in which the product is to be marketed. One national regulatory authority, the "reference" Member State, selected by the applicant, assesses the application for marketing authorization. As part of this procedure, an applicant submits an application for marketing authorization, or dossier, and related materials, including a draft SmPC, and draft labeling and package leaflet, to the reference EU Member State and the concerned EU Member States. This application is identical to the application that would be submitted to the EMA for authorization through the centralized procedure. The reference EU Member State prepares a draft assessment report and drafts of the related SmPC, labeling and package leaflet within 120 days after receipt of a valid application. The regulatory authorities of the other EU Member States, the "concerned" Member States, are subsequently required to grant marketing authorization for their territory on the basis of this assessment within 90 days of receipt thereof. The only exception to this obligation arises where the regulatory authorities provide evidence of potential serious risk to public health which would require this authorization to be refused. Similarly, the mutual recognition procedure is based on the acceptance by the regulatory authorities of the EU Member States of the marketing authorization of a medicinal product by the regulatory authorities of other EU Member States. The holder of a national marketing authorization may submit an application to the regulatory authority of an EU Member State requesting that this authority recognize the marketing authorization delivered by the regulatory authority of another EU Member State. The reference EU Member State prepares a draft assessment report and drafts of the related SmPC, labeling and package leaflet within 90 days after receipt of a valid application. The resulting assessment report is submitted to the concerned EU Member States, which within 90 days of receipt must each decide whether to approve the assessment report and the related materials. For both the decentralized and mutual recognition procedures, if a concerned EU Member State does not approve the assessment report and related materials on the grounds of potential serious risk to public health, the disputed points may eventually be referred to the Coordination Group for Mutual Recognition and Decentralized Procedures ("CMDh") whose decision is binding on all EU Member States. If the CMDh does not reach an agreement, the disputed points are forwarded to the CHMP. The CHMP then adopts an opinion in the matter, which is forwarded to the EC, which makes the final decision regarding the application for a decentralized or mutual recognition marketing authorization. LOJUXTA was granted a marketing authorization by the EC under the centralized procedure. Because Aegerion was not able to provide comprehensive clinical data on efficacy and safety under normal conditions of use due to the rarity of the disease, and in light of Aegerion's commitments to conduct an appropriate risk-mitigation program, LOJUXTA was approved under exceptional circumstances. This type of marketing authorization requires an annual reassessment of the risk/benefit of LOJUXTA by the CHMP, for which Amryt is now responsible. As part of the post-marketing commitments to the FDA, Aegerion is conducting an observational cohort study to generate more data on the long-term safety profile of lomitapide in the treatment of patients with HoFH, the patterns of use and compliance and the long-term effectiveness of controlling LDL-C levels. The EMA has required that all patients taking lomitapide in the EU be encouraged to participate in the study, and that the study period be open-ended. Amryt will bear the costs of conducting this study in the EEA and other relevant territories. In the study, physicians will follow each patient to track malignancies, tumors, teratogenicity, hepatic effects, and gastrointestinal adverse reactions, events associated with coagulopathy, major adverse cardiovascular events and death. The EMA also required that a vascular imaging study be conducted to determine the impact of lomitapide on vascular endpoints, which is now the responsibility of Amryt.

## *EU Orphan Designation and Exclusivity*

The EMA grants orphan designation to promote the development of products that treat life-threatening or chronically debilitating conditions affecting not more than five in 10,000 people in the EU. In addition, orphan drug designation can be granted only if, for economic reasons, the medicinal product would be unlikely to be developed without incentives and if there is no other satisfactory method approved in the EU of diagnosing, preventing, or treating the condition, or if such a method exists, the proposed medicinal product must potentially be of significant benefit to patients affected by the condition. The application for orphan designation must be granted by the EC before an application for marketing authorization of the medicinal product is submitted. Upon grant of marketing authorization for the medicinal products, orphan designation provides ten years of market exclusivity for the orphan medicinal product in the orphan indication. During this ten-year period, with a limited number of exceptions, the regulatory authorities of the EU Member States and the EMA may not accept applications for marketing authorization, accept an application to extend an existing marketing authorization or grant marketing authorization for other similar medicinal products for the same orphan indication. Under an exception, marketing authorization could be granted to a similar medicinal product with the same orphan indication before the expiry of the ten years if the holder of the marketing authorization for the original orphan medicinal product has given its consent or if the manufacturer of the original orphan medicinal product is unable to supply sufficient quantities. Marketing authorization may also be granted to a similar medicinal product with the same orphan indication if this product is safer, more effective or otherwise clinically superior to the original orphan medicinal product. Moreover, the exclusivity period for the original orphan medicinal product may be reduced to six years if the designation criteria are no longer met, including where it is shown that the product is sufficiently profitable not to justify maintenance of market exclusivity. Despite the prevalence rate, lomitapide does not have orphan medicinal product exclusivity in the EU for the treatment of HoFH because the EMA views the relevant condition, for orphan drug purposes, to include both HoFH and HeFH. In 2012, metreleptin was granted orphan designation by the EC for the treatment of acquired PL, congenital GL, acquired GL and familial PL. If approved by the EMA, metreleptin would be entitled to 10 years of market exclusivity by the EU.

In the EU, certain patents may qualify for a supplemental protection certificate that would extend patent protection for up to five years after patent expiration upon marketing authorization in the EU. Grant of such supplemental protection certificate is, however, subject to strict conditions and it is not automatic. We believe that our EPO method of use patent covering certain dosing regimens for lomitapide which expires in 2025 may be eligible for up to three years of supplemental protection in certain EPO countries, and we are seeking such protection in the EU Member States, on a country-by-country basis.

Similar to the U.S., marketing authorization holders and manufacturers of medicinal products are subject to comprehensive regulatory oversight by the EMA and/or the regulatory authorities of the EU Member States. This oversight applies both before and after grant of manufacturing and marketing authorizations. It includes control of compliance with EU GMP rules, manufacturing authorizations, pharmacovigilance rules and requirements governing advertising, promotion, sale, and distribution, recordkeeping, importing and exporting of medicinal products.

Failure to comply with the EU Member State laws implementing the EU Community Code on medicinal products and other EU Member State laws that apply to the promotion of medicinal products, statutory health insurance, bribery and anti-corruption or with other applicable regulatory requirements can result in enforcement action by the EU Member State authorities.

## *European Data Collection*

The collection and use of personal health data and other personal information in the EU is governed by the provisions of the Data Protection Directive as implemented into national laws by the EU Member States. This Directive imposes a number of strict obligations and restrictions on the ability to collect, analyze and transfer personal data, including health data from clinical trials and adverse event reporting. There is, moreover, a growing trend towards imposition of an obligation of public disclosure of clinical trial data in the EU which adds to the complexity of obligations relating to the processing of health data from clinical trials. Such public disclosure obligations are provided in the new EU Clinical Trials Regulation, EMA disclosure initiatives, and voluntary commitments by industry. The Data Protection Directive also includes requirements relating to the consent of the individuals to whom the personal data relates, the information provided to the individuals prior to processing their personal data or personal health data, notification of data processing obligations to the national data protection authorities and the security and confidentiality of the personal data. The Data Protection Directive also prohibits the transfer of personal data to countries outside of the EU Member States that are not considered by the EC to provide an adequate level of data protection. These countries include the U.S. Failure to comply with the requirements of the Data Protection Directive and the related national data protection laws of the EU Member States may result in fines and other administrative penalties. Data protection authorities from the different EU Member States may interpret the EU Data Protection Directive and national laws differently, which adds to the complexity of processing personal data in the EU. Guidance developed at both EU level and at the national level in individual EU Member States concerning implementation and compliance practices are often updated or otherwise revised. The EU General Data Protection Regulation ("EU No. 2016/679"), which will apply beginning May 25, 2018, will introduce new data protection requirements in

the EU and substantial fines for breaches of the data protection rules. The EU Data Protection Regulation, which will be applicable two years after the date of its publication in the Official Journal for the European Union, will increase our responsibility and liability in relation to personal data that we process and we may be required to put in place additional mechanisms ensuring compliance with the new EU data protection rules. This may be onerous and increase our cost of doing business.

### *Expanded Access*

In certain countries, drug products approved in the U.S. or EU can be accessed by patients before the drug has obtained marketing approval in such country. There are various forms of this access. They include the actual purchase of product by the purchaser, which is often times the government for patients, on a named patient basis, providing the product free of charge on a named patient basis, and providing the product on a compassionate use basis. Each country has its own laws and regulations that apply to these forms of access and the extent and nature of such laws and regulations vary by country. We have made lomitapide available in Brazil and other countries that allow such use, and we plan to continue to consider access to additional countries in compliance with applicable laws and regulations. When Aegerion acquired metreleptin from AstraZeneca in January 2015, there were a number of patients receiving metreleptin therapy free of charge in certain countries outside the U.S. that allow use of a drug, under a compassionate use or other type of expanded access program, before marketing approval has been obtained in such country. Where permitted in accordance with applicable requirements, we have continued to make metreleptin available free of charge under such program, which has resulted in significant costs to us. In 2016, we began generating revenues from named patient sales of metreleptin in certain markets where named patient sales of metreleptin are possible and to the extent permitted by applicable law and local regulatory authorities. In particular, we are in the process of converting GL and PL patients currently in the expanded access program in France to a paid program under the Autorisation Temporaire d'Utilisation (Temporary Authorization for Use). Metreleptin has also been approved for reimbursement by the Turkish SGK, and we are providing metreleptin on a named patient basis for GL patients, including congenital GL patients, and other subsets of lipodystrophy patients, including patients with congenital leptin deficiency, subject to individual assessment in response to unsolicited requests from clinicians.

### *Pharmaceutical Coverage, Pricing and Reimbursement*

Significant uncertainty exists regarding the coverage and reimbursement status of products approved by the FDA and other government authorities. Sales of pharmaceutical products depend in significant part on the availability and adequacy of third-party reimbursement. Third-party payers include government health administrative authorities, including authorities at the U.S. federal and state level, managed care providers, private health insurers and other organizations. Third-party payers are increasingly challenging the prices charged for, examining the medical necessity of, and assessing the cost-effectiveness of medical products and services.

In the U.S., the Medicare program provides health insurance for people who are 65 or older, as well as certain people with disabilities and other conditions irrespective of their age. The Medicare program is funded by the federal government and administered by the CMS. The Medicare Prescription Drug, Improvement, and Modernization Act of 2003 ("MMA"), established the Medicare Part D program to provide a voluntary prescription drug benefit to Medicare beneficiaries. Medicare Part D is a voluntary prescription drug benefit, through which beneficiaries may enroll in prescription drug plans offered by private entities under contract with CMS for coverage of certain outpatient prescription drugs. Medicare Part D generally provides coverage for medically necessary self-administered drugs (i.e., drugs that do not need to be administered by a healthcare practitioner). JUXTAPID and MYALEPT may be covered under Medicare Part D. Coverage and reimbursement for outpatient drugs under Part D is not standardized. Part D prescription drug plan sponsors are not required to pay for all covered Part D drugs, and each drug plan can develop its own drug formulary that identifies which drugs the plan will cover and at what tier or level. However, Part D prescription drug formularies must include drugs within each therapeutic category and class of covered Part D drugs, though not necessarily all the drugs in each category or class. Any formulary used by a Part D prescription drug plan must be developed and reviewed by a pharmacy and therapeutic committee. Government payment for some of the costs of prescription drugs may increase demand for products for which we receive marketing approval.

The availability of coverage under Medicare Part D may affect demand for JUXTAPID and MYALEPT. In order for JUXTAPID and MYALEPT to remain on or be included on the formularies of Part D prescription drug plans, we may have to offer discounts on the price of those products. In addition, manufacturers, including Aegerion, are required to provide a 50% discount on brand name prescription drugs utilized by Medicare Part D beneficiaries when those beneficiaries reach the so-called "donut hole" in their drug benefits in a particular year (i.e., a coverage gap between initial coverage and catastrophic coverage). However, any negotiated prices for our products covered by a Part D prescription drug plan likely will be lower than the prices we might otherwise obtain. Moreover, while the MMA applies only to drug benefits for Medicare beneficiaries, private payers often follow Medicare coverage policy and payment limitations in setting their own payment rates. Any reduction in payment that results from the MMA may result in a similar reduction in payments from non-governmental payers. We believe that investigations

and enforcement actions by certain government agencies have caused a reduction in contributions to these third-party patient organizations, which may prevent these organizations from providing adequate financial assistance, including assistance with co-payment obligations, to individuals who would otherwise be unable to afford our products. As a result, Medicare Part D patients may not be able to afford their out-of-pocket co-payments for our products. In 2017, for example, we believe that a considerable number of JUXTAPID patients who were covered by Medicare Part D ceased treatment with JUXTAPID, due to reductions in contributions to patient assistance programs operated by independent charitable 501(c)(3) organizations, which resulted in prior sources of financial support for these patients being less available.

Medicaid is a health insurance program with mandatory coverage for certain low-income children, families, pregnant women, and people with disabilities. States also have the option of expanding Medicaid coverage to low-income individuals generally and many states have done so. Medicaid is jointly funded by the federal and state governments, but administered by the states. In general, state Medicaid programs are required to cover drugs and biologics of manufacturers that have entered into a Medicaid Drug Rebate Agreement, as discussed further below, although such drugs and biologics may be subject to prior authorization or other utilization controls.

Coverage of drugs and biologics by private health insurance varies. Private payers may use a variety of utilization management techniques designed to control costs, including requiring pre-approval of coverage for drug therapies before reimbursing healthcare providers or patients that use such therapies. In addition, a payer's decision to provide coverage for a drug product does not imply that an adequate reimbursement rate will be provided. Coverage may not be sufficient to maintain price levels high enough to realize an appropriate return on investment in product development.

Government and private third-party payers have a variety of methodologies for paying for drugs and biologics. Payers are increasingly considering new metrics as the basis for reimbursement rates, such as average sales price, average manufacturer price ("AMP") or actual acquisition cost. Recent changes to the Medicaid Drug Rebate Program, effective April 2016, require state Medicaid programs to reimburse certain brand name covered outpatient drugs at actual acquisition cost plus a dispensing fee. The impact of these evolving reimbursement mechanics on the willingness of providers to furnish JUXTAPID or MYALEPT or other products we may market and the prices we can command for these products is difficult to predict.

We participate in various government programs or contracts that require us to calculate and report certain prices for our products to government agencies or provide rebates or discounted pricing on products purchased to certain purchasers or government payers. The requirements for calculating prices and rebates are complex and subject to change. We may also have reimbursement obligations or be subject to penalties if we fail to provide timely and accurate information to the government, pay the correct rebates or offer the correct discounted pricing.

We participate in the Medicaid Drug Rebate Program. The Medicaid Drug Rebate Program requires pharmaceutical manufacturers to enter into and have in effect a national rebate agreement with the Secretary of the DHHS as a condition for states to receive federal matching funds for the manufacturer's outpatient drugs furnished to Medicaid patients. The Healthcare Reform Act made several changes to the Medicaid Drug Rebate Program, including increasing pharmaceutical manufacturers' rebate liability by raising the minimum basic Medicaid rebate on most branded prescription drugs from 15.1% of AMP, to 23.1% of AMP and adding a new rebate calculation for "line extensions" (i.e., new formulations, such as extended release formulations) of solid oral dosage forms of branded products, creating a new methodology by which rebates are calculated for drugs that are inhaled, infused, instilled, implanted or injected, as well as potentially impacting their rebate liability by modifying the statutory definition of AMP. The Healthcare Reform Act also expanded the universe of Medicaid utilization subject to drug rebates by requiring pharmaceutical manufacturers to pay rebates on Medicaid managed care utilization and by enlarging the population potentially eligible for Medicaid drug benefits. Pricing and rebate programs must also comply with the Medicaid rebate requirements of the U.S. Omnibus Budget Reconciliation Act of 1990.

Under the Medicaid Drug Rebate Program, we are required to pay a rebate for each unit of our product reimbursed by a state Medicaid program as a condition of having federal funds made available to the states for our drugs under Medicaid and Medicare Part B. Those rebates are based on pricing data that we report on a monthly and quarterly basis to CMS, the federal agency that administers the Medicaid Drug Rebate Program. We may also participate in state Medicaid supplemental rebate programs which require payment of an incremental rebate to state Medicaid programs for covered utilization of our products. Price reductions as well as price increases that exceed the rate of inflation for our products, such as the price increase for MYALEPT in February 2015, may result in increasing the rebates we are required to pay under the Medicaid Drug Rebate Program or state Medicaid supplemental rebate programs and the discounts we are required to offer under the Public Health Service ("PHS") 340B drug pricing discount program (the "340B Program"), as discussed below. As a result of the substantial price increase for MYALEPT in February 2015, we continue to expect a significant gross-to-net adjustment for Medicaid rebates which will offset the majority of revenues from Medicaid and negatively impact net product sales in future quarters, since Medicaid rebates directly reduce our net product sales. The degree of such impact on our overall financial performance will depend on the percentage of MYALEPT



patients that have Medicaid as their primary insurance coverage and the quantity of units ordered per patient. To date, approximately 34% of patients prescribed MYALEPT have been Medicaid beneficiaries. The number of patients prescribed MYALEPT in the future who are Medicaid beneficiaries could be higher than historical rates.

To maintain coverage of our products under the Medicaid Drug Rebate Program and Medicare Part B, we are required to extend significant discounts to certain “covered entities” (defined by statute to include certain types of hospitals and other healthcare providers that receive federal grants) that purchase products under the 340B Program. The 340B Program requires participating manufacturers to agree to charge such covered entities no more than the 340B “ceiling price” for the manufacturers’ covered outpatient drugs. The 340B ceiling price is calculated using a statutory formula, which is based on the AMP and rebate amount for the covered outpatient drug as calculated under the Medicaid Drug Rebate Program. “Orphan drugs” (those designated under section 526 of the FDCA, such as JUXTAPID and MYALEPT) are exempt from the ceiling price requirements with respect to drugs purchased by certain covered entities (i.e., rural referral centers, sole community hospitals, critical access hospitals, and free-standing cancer hospitals). The Healthcare Reform Act also obligates the Health Resources and Services Administration (“HRSA”), the agency which administers the 340B Program, to promulgate various regulations and implement processes to improve the integrity of the 340B Program. The status of new and pending regulations and guidance is uncertain under the new presidential administration.

Drug products are subject to discounted pricing when purchased by federal agencies via the FSS. FSS participation is required for a drug product to be covered and reimbursed by certain federal agencies and for coverage under Medicaid, Medicare Part B and the 340B Program. FSS pricing is negotiated periodically with the Department of Veterans Affairs. FSS pricing is intended not to exceed the price that a manufacturer charges its most-favored non-federal customer for its product. In addition, prices for drugs purchased by the Veterans Administration, Department of Defense (including drugs purchased by military personnel and dependents through the Tricare Retail Pharmacy program), Coast Guard, and PHS are subject to a cap on pricing (known as the federal ceiling price) and may be subject to an additional discount if pricing increases more than the rate of inflation. Aegerion participates in the FSS, and has had an FSS contract in place for its products since 2016. Aegerion also participates in the Tricare Retail Pharmacy program, under which we pay quarterly rebates on utilization of JUXTAPID and MYALEPT when the products are dispensed through the Tricare Retail Pharmacy network to Tricare beneficiaries.

As noted above, the marketability of any products for which we receive regulatory approval for commercial sale may suffer if the government and third-party payors fail to provide adequate coverage and reimbursement. An increasing emphasis on cost containment measures in the U.S. has increased and we expect will continue to increase the pressure on pharmaceutical pricing. Coverage policies and third-party reimbursement rates may change at any time. Even if favorable coverage and reimbursement status is attained for one or more products for which we receive regulatory approval, less favorable coverage policies and reimbursement rates may be implemented in the future.

In recent years, additional laws have resulted in direct or indirect reimbursement reductions for certain Medicare providers, including:

- The Budget Control Act of 2011, among other things, created measures for spending reductions by Congress. A Joint Select Committee on Deficit Reduction, tasked with recommending a targeted deficit reduction of at least \$1.2 trillion for the years 2013 through 2021, was unable to reach required goals, thereby triggering the legislation’s automatic reduction to several government programs. These changes included aggregate reductions to Medicare payments to providers of up to 2% per fiscal year, which went into effect in April 2013 and will remain in effect through 2025 unless additional Congressional action is taken.
- The American Taxpayer Relief Act of 2012, among other things, reduced Medicare payments to several providers, and increased the statute of limitations period for the government to recover overpayments to providers from three to five years.
- The Middle Class Tax Relief and Job Creation Act of 2012 required that the CMS reduce the Medicare clinical laboratory fee schedule by 2% in 2013, which served as a base for 2014 and subsequent years. In addition, effective January 1, 2014, CMS also began bundling the Medicare payments for certain laboratory tests ordered while a patient received services in a hospital outpatient setting.

These laws, and future state and federal healthcare reform measures may be adopted in the future, any of which may result in additional reductions in Medicare and other healthcare funding and otherwise affect the prices we may obtain for any product candidates for which we may obtain regulatory approval or the frequency with which any such product candidate is prescribed or used.

Further, in the U.S., the cost of pharmaceuticals continues to generate substantial governmental and third-party payer interest. We expect that the pharmaceutical industry will experience pricing pressures due to the increasing influence of managed care (and related implementation of managed care strategies to control utilization), additional federal and state legislative and regulatory proposals to regulate pricing of drugs, limit coverage of drugs or reduce reimbursement for drugs, public scrutiny and the presidential administration's agenda to control the price of pharmaceuticals through government negotiations of drug prices in Medicare Part D and importation of cheaper products from abroad.

In addition, in many foreign countries, including several of the key markets where we do business, the proposed pricing for a drug must be approved before it may be lawfully marketed. The requirements governing drug pricing vary widely from country to country. For example, in the EU, the sole legal instrument at the EU level governing the pricing and reimbursement of medicinal products is Council Directive 89/105/EEC (the "Price Transparency Directive"). The aim of this Directive is to ensure that pricing and reimbursement mechanisms established in the EU Member States are transparent and objective, do not hinder the free movement of and trade in medicinal products in the EU, and do not hinder, prevent or distort competition on the market. The Price Transparency Directive does not provide any guidance concerning the specific criteria on the basis of which pricing and reimbursement decisions are to be made in individual EU Member States, nor does it have any direct consequence for pricing nor reimbursement levels in individual EU Member States. The EU Member States are free to restrict the range of medicinal products for which their national health insurance systems provide reimbursement, and to control the prices and/or reimbursement levels of medicinal products for human use. An EU Member State may approve a specific price or level of reimbursement for the medicinal product, or alternatively adopt a system of direct or indirect controls on the profitability of the company responsible for placing the medicinal product on the market, including volume-based arrangements, caps and reference pricing mechanisms.

Reference pricing used by various EU member states and parallel distribution, or arbitrage between low-priced and high-priced member states, can further reduce prices. A member state may approve a specific price for the medicinal product or it may instead adopt a system of direct or indirect controls on the profitability of the company placing the medicinal product on the market. In some countries, we may be required to conduct a clinical study or other studies that compare the cost-effectiveness of our product candidates, if any, to other available therapies in order to obtain or maintain reimbursement or pricing approval. There can be no assurance that any country that has price controls or reimbursement limitations for pharmaceutical products will allow favorable reimbursement and pricing arrangements for any of our products. Historically, products launched in the EU do not follow price structures of the U.S. and generally published and actual prices tend to be significantly lower. This has largely proven to be the case for lomitapide and we expect to be the case for metreleptin, if approved in the EU. Publication of discounts by third-party payers or authorities may lead to further pressure on the prices or reimbursement levels within the country of publication and other countries.

Health Technology Assessment ("HTA") of medicinal products is becoming an increasingly common part of the pricing and reimbursement procedures in some EU Member States, including the United Kingdom, France, Germany, Ireland, Italy and Sweden. The HTA process in the EU Member States is governed by the national laws of these countries. HTA is the procedure according to which the assessment of the public health impact, therapeutic impact, and the economic and societal impact of use of a given medicinal product in the national healthcare systems of the individual country is conducted. HTA generally focuses on the clinical efficacy and effectiveness, safety, cost, and cost-effectiveness of individual medicinal products as well as their potential implications for the healthcare system. Those elements of medicinal products are compared with other treatment options available on the market. The outcome of HTA regarding specific medicinal products will often influence the pricing and reimbursement status granted to these medicinal products by the regulatory authorities of individual EU Member States. The extent to which pricing and reimbursement decisions are influenced by the HTA of the specific medicinal product vary between EU Member States. A negative HTA of one of our products by a leading and recognized HTA body, such as the National Institute for Health and Care Excellence ("NICE") in the United Kingdom, could not only undermine our ability to obtain reimbursement for such product in the EU Member State in which such negative assessment was issued, but also in other EU Member States. For example, EU Member States that have not yet developed HTA mechanisms could rely to some extent on the HTA performed in countries with a developed HTA framework, such as the United Kingdom, when adopting decisions concerning the pricing and reimbursement of a specific medicinal product.

In 2011, Directive 2011/24/EU was adopted at the EU level. This Directive concerns the application of patients' rights in cross-border healthcare. The Directive is intended to establish rules for facilitating access to safe and high-quality cross-border healthcare in the EU. It also provides for the establishment of a voluntary network of national authorities or bodies responsible for HTA in the individual EU Member States. The purpose of the network is to facilitate and support the exchange of scientific information concerning HTAs. This could lead to harmonization between EU Member States of the criteria taken into account in the conduct of HTA and their impact on pricing and reimbursement decisions.

To obtain reimbursement or pricing approval in some countries, including the EU Member States, we may be required to conduct studies that compare the cost-effectiveness of our product candidates to other therapies that are considered the local

standard of care. In the case of metreleptin, in preparation for seeking reimbursement and pricing approval, if metreleptin is approved by the EMA, we are conducting local and regional studies to ascertain the impact of metreleptin on morbidity, mortality and patients' quality of life, in order to maximize the product's value proposition to payers. There can be no assurance that any country will allow favorable pricing, reimbursement and market access conditions for any of our products, or that it will be feasible to conduct additional cost-effectiveness studies, if required.

In certain of the EU Member States, products that are designated as orphan medicinal products may be exempted or waived from having to provide certain clinical, cost-effectiveness and other economic data in connection with their filings for pricing/reimbursement approval. As noted above, LOJUXTA was not granted orphan designation by the EMA for the treatment of HoFH. As such, it is not eligible for benefits related to orphan designation. As a result, Amryt may not be able to provide all of the data required to obtain pricing/reimbursement approvals in certain EU Member States, which has and could, in the future, result in delays of pricing/reimbursement approvals for LOJUXTA, LOJUXTA not obtaining pricing/reimbursement approval at all, or LOJUXTA obtaining approvals at less than acceptable levels or with significant restrictions on use or reimbursement, all of which thereby potentially negatively impacting sales milestone and royalty payments Aegerion receives under its license agreement with Amryt.

### ***U. S. Healthcare Reform***

Our revenues and operations could be affected by changes in healthcare spending and policy in the U.S. We operate in a highly regulated industry and new laws, regulations or judicial decisions, or new interpretations of existing laws, regulations or decisions, related to health care availability, the method of delivery or payment for health care products and services could negatively impact our business, operations and financial condition. As noted above, the U.S. Congress and state legislatures from time to time propose and adopt initiatives aimed at cost containment, which could impact our ability to sell our products profitably. For example, the Healthcare Reform Act substantially changed the way healthcare is financed by both governmental and private insurers. The law contains a number of provisions that affect coverage and reimbursement of drug products and/or that could potentially reduce the demand for our products such as:

- increasing drug rebates under state Medicaid programs for brand name prescription drugs and extending those rebates to Medicaid managed care; and
- requiring drug manufacturers to provide a 50% discount on Medicare Part D brand name prescription drugs sold to Medicare beneficiaries whose prescription drug costs cause the beneficiaries to be subject to the Medicare Part D coverage cap (i.e., the so-called donut hole).

In 2012, the Supreme Court of the U.S. heard challenges to the constitutionality of the individual mandate and the viability of certain provisions of the Healthcare Reform Act. The Supreme Court's decision upheld most of the Healthcare Reform Act and determined that requiring individuals to maintain "minimum essential" health insurance coverage or pay a penalty to the Internal Revenue Service was within Congress's constitutional taxing authority. As a result of tax reform legislation passed in late December 2017, the individual mandate has been eliminated; the long ranging effects on the viability of the Healthcare Reform Act are unknown at this time.

On January 20, 2017, President Trump signed an Executive Order directing federal agencies with authorities and responsibilities under the Healthcare Reform Act to waive, defer, grant exemptions from, or delay the implementation of any provision of the Healthcare Reform Act that would impose a fiscal or regulatory burden on states, individuals, healthcare providers, health insurers, or manufacturers of pharmaceuticals or medical devices. On October 13, 2017, President Trump signed an Executive Order terminating the cost-sharing subsidies that reimburse insurers under the Healthcare Reform Act. Several state Attorneys General filed suit to stop the administration from terminating the subsidies, but their request for a restraining order was denied by a federal judge in California on October 25, 2017. In addition, CMS has recently proposed regulations that would give states greater flexibility in setting benchmarks for insurers in the individual and small group marketplaces, which may have the effect of relaxing the essential health benefits required under the Healthcare Reform Act for plans sold through such marketplaces. Further, each chamber of Congress has put forth multiple bills this year designed to repeal or repeal and replace portions of the Healthcare Reform Act. In 2018, Congress may consider other legislation to repeal and replace elements of the Healthcare Reform Act. Congress will likely consider other legislation to replace elements of the Healthcare Reform Act; litigation and legislation over the Healthcare Reform Act are likely to continue, with unpredictable and uncertain results.

In addition, other legislative changes have been proposed and adopted since the Healthcare Reform Act was enacted that, directly or indirectly, affect or are likely to affect, the pharmaceutical industry and the commercialization of our products, including the Budget Control Act of 2011, the American Taxpayer Relief Act of 2012 and the Middle Class Tax Relief and Job Creation Act of 2012 as discussed above.

Further, there has been heightened governmental scrutiny over the manner in which manufacturers set prices for their marketed products, which have resulted in several recent Congressional inquiries and proposed bills designed to, among other things, bring more transparency to product pricing, review the relationship between pricing and manufacturer patient programs, and reform government program reimbursement methodologies for products. In addition, the U.S. government, state legislatures, and foreign governments have shown significant interest in implementing cost containment programs, including price-controls, restrictions on reimbursement and requirements for substitution of generic products for branded prescription drugs to limit the growth of government paid healthcare costs. For example, the U.S. government has passed legislation requiring pharmaceutical manufacturers to provide rebates and discounts to certain entities and governmental payers to participate in federal healthcare programs. Individual states in the United States have also become increasingly aggressive in passing legislation and implementing regulations designed to control pharmaceutical and biological product pricing, including price or patient reimbursement constraints, discounts, restrictions on certain product access and marketing cost disclosure and transparency measures, and, in some cases, designed to encourage importation from other countries and bulk purchasing.

#### ***Promotional Activities and Interactions with Healthcare Providers and Patients***

The FDA and other regulatory agencies strictly regulate promotional claims about prescription drug and biological products through, among other things, standards and regulations for direct-to-consumer advertising, communications regarding unapproved uses, industry-sponsored scientific and educational activities, and promotional activities involving the Internet and social media. A product generally cannot be commercially promoted before it is approved. In general, after approval, a drug product may not be promoted for uses that are not approved by the FDA, the EC, and the regulatory authorities of the EU Member States or such other regulatory agencies as reflected in the product's prescribing information. Moreover, the promotion of prescription-only medicinal products to non-healthcare professionals is prohibited in the EU. In the U.S., healthcare professionals are generally permitted to prescribe drugs and biologics for "off-label" uses (i.e., uses not described in the drug's labeling) because the FDA does not regulate the practice of medicine. However, FDA regulations impose stringent restrictions on manufacturers' communications regarding off-label uses. A manufacturer may not promote a drug or biologic for off-label use, but under very specific conditions, it may be permissible for a manufacturer to engage in non-promotional, truthful, non-misleading communication regarding off-label information. If a company is found to have promoted off-label uses, it may become subject to adverse publicity and enforcement action by the FDA, the DOJ, or the Office of the Inspector General of the DHHS, as well as state authorities. This could subject a company to a range of penalties that could have a significant commercial impact, including civil and criminal fines and agreements that materially restrict the manner in which a company promotes or distributes drug or biological products. The federal government has levied large civil and criminal fines against companies for alleged improper promotion, and has also requested that companies enter into consent decrees or permanent injunctions under which specified promotional conduct is changed or curtailed. For example, as described in detail in the " *Legal Proceedings* " section of this Annual Report, to resolve investigations conducted by the DOJ and SEC regarding Aegerion's U.S. commercial activities and disclosures related to JUXTAPID, Aegerion is required, among other things, to pay approximately \$40.1 million in aggregate penalties over three years, which includes \$7.2 million in restitution, a civil penalty of \$4.1 million to be paid to the SEC pursuant to the SEC Judgment, and \$28.8 million to be paid pursuant to the DOJ Civil Settlement Agreement.

#### ***Other Healthcare Laws Affecting Our Business***

Healthcare providers, physicians, and third party payers will play a primary role in the recommendation and prescription of any products for which we obtain marketing approval. Our future arrangements with third party payers, healthcare providers and physicians may expose us to broadly applicable fraud and abuse and other healthcare laws and regulations that may constrain the business or financial arrangements and relationships through which we market, sell and distribute any drugs for which we obtain marketing approval. In the U.S., these laws include, without limitation, state and federal anti-kickback, false claims, physician transparency, and patient data privacy and security laws and regulations, including but not limited to those described below.

- The Anti-Kickback Statute makes it illegal for any person, including a prescription drug manufacturer (or a party acting on its behalf), to knowingly and willfully solicit, receive, offer or pay any remuneration, directly or indirectly, in cash or in kind, that is intended to induce or reward referrals, including the purchase, recommendation, order or prescription of a particular drug, for which payment may be made under a federal healthcare program, such as Medicare or Medicaid. Violations of this law are punishable by up to five years in prison, criminal fines, administrative civil money penalties and exclusion from participation in federal healthcare programs. In addition, a person or entity does not need to have actual knowledge of the statute or specific intent to violate it.
- The federal False Claims Act imposes civil penalties, including through civil whistleblower or qui tam actions, against individuals or entities (including manufacturers) for, among other things, knowingly presenting, or causing to be presented false or fraudulent claims for payment by a federal healthcare program or making a false statement or record material to

payment of a false claim or avoiding, decreasing or concealing an obligation to pay money to the federal government. The government may deem manufacturers to have “caused” the submission of false or fraudulent claims by, for example, providing inaccurate billing or coding information to customers or promoting a product off-label. Claims which include items or services resulting from a violation of the federal Anti-Kickback Statute are false or fraudulent claims for purposes of the False Claims Act. Our future marketing and activities relating to the reporting of wholesaler or estimated retail prices for our products, the reporting of prices used to calculate Medicaid rebate information and other information affecting federal, state and third-party reimbursement for our products, and the sale and marketing of our products and any future product candidates, are subject to scrutiny under this law.

- HIPAA, which imposes criminal and civil liability for knowingly and willfully executing a scheme, or attempting to execute a scheme, to defraud any healthcare benefit program, including private payers, or falsifying, concealing or covering up a material fact or making any materially false statements in connection with the delivery of or payment for healthcare benefits, items or services.
- HIPAA, as amended by the Health Information Technology for Economic and Clinical Health Act of 2009 (“HITECH”), and their respective implementing regulations, which impose, among other things, specified requirements on covered entities and their business associates relating to the privacy and security of individually identifiable health information including mandatory contractual terms and required implementation of technical safeguards of such information. HITECH also created new tiers of civil monetary penalties, amended HIPAA to make civil and criminal penalties directly applicable to business associates, and gave state attorneys general new authority to file civil actions for damages or injunctions in federal courts to enforce the federal HIPAA laws and seek attorneys’ fees and costs associated with pursuing federal civil actions.
- The Physician Payments Sunshine Act, enacted as part of the Healthcare Reform Act, which imposed new annual reporting requirements for certain manufacturers of drugs, devices, biologics, and medical supplies for which payment is available under Medicare, Medicaid, or the Children’s Health Insurance Program, for certain payments and “transfers of value” provided to physicians and teaching hospitals, as well as ownership and investment interests held by physicians and their immediate family members.
- Analogous state and foreign fraud and abuse laws and regulations, such as state anti-kickback and false claims laws, which may be broader in scope and apply regardless of payor. Such laws are enforced by various state agencies and through private actions. Some state laws require pharmaceutical companies to comply with the pharmaceutical industry’s voluntary compliance guidelines and the relevant federal government compliance guidance, require drug manufacturers to report information related to payments and other transfers of value to physicians and other healthcare providers, and restrict marketing practices or require disclosure of marketing expenditures. State and foreign laws also govern the privacy and security of health information in some circumstances. Such data privacy and security laws may differ from each other in significant ways and often are not pre-empted by HIPAA, thus complicating compliance efforts.

The scope and enforcement of each of these laws is uncertain and subject to rapid change in the current environment of healthcare reform, especially in light of the lack of applicable precedent and regulations. If our operations are found to be in violation of any of these laws or any other related governmental regulations that may apply to us, as Aegerion’s operations have been as described below, we may be subject to significant civil, criminal and administrative penalties, damages, fines, imprisonment, disgorgement, exclusion of drugs from government funded healthcare programs, such as Medicare and Medicaid, reputational harm, additional oversight and reporting obligations and the curtailment or restructuring of our operations. As noted above, in September 2017, Aegerion entered into Settlement to resolve investigations conducted by the DOJ and the SEC regarding Aegerion’s U.S. commercial activities and disclosures related to JUXTAPID, and on January 30, 2018, a U.S. District Court judge sentenced Aegerion after the judge accepted Aegerion’s guilty criminal plea. Aegerion is required, among other things, to pay approximately \$40.1 million in aggregate penalties over three years, and has been put on probation for three years. Aegerion is also subject to a five-year CIA, three-year DPA related to HIPPA compliance, and a pending civil consent decree with the FDA and the DOJ relating to the JUXTAPID REMS program. See the “*Legal Proceedings*” section of this Annual Report for further information regarding ongoing investigations, including the Settlement, and other legal proceedings. If any of the physicians or other healthcare providers or entities with whom we expect to do business is found to be not in compliance with applicable laws, they may be subject to similar actions, penalties and sanctions. Ensuring business arrangements comply with applicable healthcare laws, as well as responding to possible investigations by government authorities, can be time- and resource-consuming and can divert a company’s attention from the business. It is possible that governmental authorities will conclude that our business practices may not comply with current or future statutes, regulations or case law involving applicable fraud and abuse or other healthcare laws and regulations.

The number and complexity of both federal and state laws continues to increase, and additional governmental resources are being used to enforce these laws and to prosecute companies and individuals who are believed to be violating them. In particular,

the Healthcare Reform Act includes a number of provisions aimed at strengthening the government's ability to pursue anti-kickback and false claims cases against pharmaceutical manufacturers and other healthcare entities, including substantially increased funding for healthcare fraud enforcement activities, enhanced investigative powers, and amendments to the False Claims Act that make it easier for the government and whistleblowers to pursue cases for alleged kickback and false claim violations. While it is too early to predict what effect these changes will have on our business, we anticipate that government scrutiny of pharmaceutical sales and marketing practices will continue for the foreseeable future and subject us to the risk of government investigations and enforcement actions. For example, federal enforcement agencies recently have shown interest in pharmaceutical companies' product and patient assistance programs, including manufacturer reimbursement support services and relationships with specialty pharmacies. Some of these investigations have resulted in significant civil and criminal settlements.

#### *EU Advertising and Promotion*

In the EU, the advertising and promotion of our products is also subject to EU Member States' laws concerning promotion of medicinal products, interactions with physicians, misleading and comparative advertising and unfair commercial practices. In addition, other legislation of individual EU Member States may apply to the advertising and promotion of medicinal products. These laws require that promotional materials and advertising in relation to medicinal products comply with the product's SmPC as approved by the regulatory authorities. The SmPC is the document that provides information to physicians concerning the safe and effective use of the medicinal product. It forms an intrinsic and integral part of the marketing authorization granted for the medicinal product. Promotion of a medicinal product that does not comply with the SmPC is considered to constitute off-label promotion. The off-label promotion of medicinal products is prohibited in the EU. The applicable laws at the EU level and in the individual EU Member States also prohibit the direct-to-consumer advertising of prescription-only medicinal products. Violations of the rules governing the promotion of medicinal products in the EU could be penalized by administrative measures, fines and imprisonment. These laws may further limit or restrict the advertising and promotion of our products to the general public and may also impose limitations on our promotional activities with healthcare professionals.

Interactions between pharmaceutical companies and physicians are also governed by strict laws, regulations, industry self-regulation codes of conduct and physicians' codes of professional conduct in the individual EU Member States. The provision of benefits or advantages to physicians to induce or encourage the prescription, recommendation, endorsement, purchase, supply, order or use of medicinal products is prohibited in the EU. The provision of benefits or advantages to physicians is also governed by the national anti-bribery laws of the EU Member States. One example is the UK Bribery Act. This Act applies to any company incorporated in or "carrying on business" in the UK, irrespective of where in the world the alleged bribery activity occurs. This Act could have implications for our interactions with physicians both in and outside the UK. Violation of these laws could result in substantial fines and imprisonment.

Payments made to physicians in certain EU Member States must be publicly disclosed. Moreover, agreements with physicians must often be the subject of prior notification and approval by the physician's employer, his/her regulatory professional organization, and/or the authorities of the individual EU Member States. These requirements are provided in the national laws, industry codes, or professional codes of conduct, applicable in the EU Member States. Failure to comply with these requirements could result in reputational risk, public reprimands, administrative penalties, fines or imprisonment.

#### *Additional Laws and Regulations Governing International Operations*

For other countries outside of the EU and the U.S, such as countries in Eastern Europe, Latin America or Asia, the requirements governing the conduct of clinical trials, product licensing, pricing and reimbursement vary from country to country. Additionally, the clinical trials must be conducted in accordance with GCP requirements and the applicable regulatory requirements and the ethical principles that have their origin in the Declaration of Helsinki.

If we fail to comply with applicable foreign regulatory requirements, we may be subject to, among other things, fines, suspension or withdrawal of regulatory approvals, product recalls, seizure of products, operating restrictions and criminal prosecution.

Our international operations are subject to compliance with the Foreign Corrupt Practices Act ("FCPA") which prohibits U.S. corporations and individuals from paying, offering to pay, or authorizing the payment of anything of value, directly or indirectly, to any foreign government official, government staff member, political party, or political candidate for the purpose of obtaining or retaining business or to otherwise influence a person working in an official capacity. We also may be implicated under the FCPA by the activities of our partners, collaborators, contract research organizations, vendors or other agents. The FCPA also requires us, as a public company, to make and keep books and records that accurately and fairly reflect all of our transactions and to devise and maintain an adequate system of internal accounting controls. An aspect of the SEC's investigation into Aegerion's

disclosures and activities relates to alleged FCPA violations in Brazil. These potential violations are excluded from the Settlement of the JUXTAPID Investigations.

Compliance with the FCPA is expensive and difficult, particularly in countries in which corruption is a recognized problem. In addition, the FCPA presents particular challenges in the pharmaceutical industry, because, in many countries, hospitals are operated by the government, and doctors and other hospital employees are considered foreign officials. Certain payments to hospitals in connection with clinical trials and other work have been deemed to be improper payments to government officials and have led to FCPA enforcement actions against other companies.

Various laws, regulations and executive orders also restrict the use and dissemination outside of the U.S., or the sharing with certain non-U.S. nationals, of information classified for national security purposes, as well as certain products and technical data relating to those products. If we are successful in expanding our presence outside of the U.S., such expansion will require us to dedicate additional resources to comply with these laws, and these laws may preclude us from developing, manufacturing, or selling certain products and product candidates outside of the U.S., which could limit our growth potential and increase our development costs.

Our international operations could also be subject to compliance with national laws of the individual EU Member States, such as the UK Bribery Act. The UK Bribery Act applies to any company incorporated in or “carrying on business” in the UK, irrespective of where in the world the offending conduct occurs. The UK Bribery Act prohibits the provision of an “advantage” intended to induce or reward “improper performance” of the recipient’s function. Offenses under the UK Bribery Act include the offer, promise or provision of a bribe to any person including non-UK government officials and private persons, as well as the request, acceptance or agreement to receive a bribe. The failure by a company to prevent third parties from providing a bribe on its behalf could also constitute an offense under the UK Bribery Act. This Act applies to bribery activities both in the public and private sector. Liability in relation to breaches of the UK Bribery Act is strict. This means that it is unnecessary to demonstrate elements of a corrupt state of mind. However, a defense of having in place adequate procedures designed to prevent bribery is available.

We are also subject to compliance with the anti-bribery laws of other countries, including Brazil. Our activities outside the U.S. or those of our employees, licensees, distributors, manufacturers, clinical research organizations, or other third parties who act on our behalf or with whom we do business could subject us to investigation or prosecution under foreign or U.S. laws. For example, federal and São Paulo authorities in Brazil are each conducting an investigation to determine whether there have been any violations of Brazilian laws related to the sales of JUXTAPID in Brazil. See Part I, Item 3 - “ *Legal Proceedings* ” section of this Annual Report for further information regarding these investigations and other legal proceedings.

We are subject to a variety of financial disclosure and securities trading regulations as a public company in Canada and the U.S., including laws relating to the oversight activities of the Canadian securities administrators and the SEC, and the rules and regulations of NASDAQ, on which our shares are traded. In addition, the Financial Accounting Standards Board, the Canadian securities administrators, the SEC, and other bodies that have jurisdiction over the form and content of our Consolidated Financial Statements and other public disclosure are issuing and amending proposed and existing pronouncements designed to ensure that companies disclose relevant and transparent information relating to their respective businesses.

The failure to comply with laws governing international business practices may result in substantial civil and criminal penalties and suspension or debarment from government contracting. The SEC also may suspend or bar issuers from trading securities on U.S. exchanges for violations of the FCPA’s accounting provisions.

Our present and future business has been and will continue to be subject to various other laws and regulations. Various laws, regulations and recommendations relating to safe working conditions, laboratory practices, the experimental use of animals, and the purchase, storage, movement, import and export and use and disposal of hazardous or potentially hazardous substances, including radioactive compounds and infectious disease agents, used or that we may use in the future in connection with our development work are or may be applicable to our activities. Certain agreements entered into by us involving exclusive license rights or acquisitions may be subject to national or supranational antitrust regulatory control, the effect of which cannot be predicted. The extent of government regulation, which might result from future legislation or administrative action, cannot accurately be predicted.

### **Research and Development Costs**

A significant portion of our operating expenses are related to research and development. During the years ended December 31, 2017, 2016, and 2015, our total company-sponsored research and development expenses were \$49.0 million, \$14.8

million , and \$9.8 million , respectively. See the Products in Development section above and in the “ *Management’s Discussion and Analysis of Financial Condition and Results of Operations* ” section of this Annual Report.

### **Significant Customers**

For the year ended December 31, 2017 , two customers (our current specialty pharmacy in the U.S. for both products and former specialty pharmacy in the U.S. for JUXTAPID) accounted for 70% of our net revenues, and of these two pharmacies, one accounted for 63% of our accounts receivable balance.

### **Employees**

As of December 31, 2017 , we had 180 employees, 70 of whom were engaged in research, development, clinical and regulatory affairs, quality control and assurance, 58 of whom were engaged in commercial, and 52 of whom were engaged in finance, business development, information technology, human resources, and legal.

When required, we also engage independent consultants and contractors to perform various professional services including, but not limited to, financial, advisory, clinical, regulatory, supply chain, sales and other commercial services.

### **Corporate Information**

Novelion, formerly known as QLT, was originally formed in 1981 under the laws of the Province of British Columbia. Our principal headquarters are located at c/o Norton Rose Fulbright, 1800-510 West Georgia Street, Vancouver, British Columbia, Canada, and our telephone number is (877)-764-3131.

### **Where You Can Find More Information**

You are advised to read this Annual Report in conjunction with other reports and documents that we file from time to time with the SEC. Copies of our annual reports on Form 10-K will be furnished without charge to any person who submits a written request directed to the attention of our Secretary, at our offices located at c/o Norton Rose Fulbright, 1800-510 West Georgia Street, Vancouver, B.C. V6B 0M3, Canada. You may also obtain copies of these reports from the SEC at the SEC’s Public Reference Room at 100 F Street, N.E. Washington, D.C. 20549. In addition, the SEC maintains information for electronic filers (including Novelion) at its website at [www.sec.gov](http://www.sec.gov). The public may obtain information regarding the operation of the Public Reference Room by calling the SEC at 1-800-SEC-0330. The System for Electronic Document Analysis and Retrieval ("SEDAR") also provides access to most public securities documents and information filed by issuers (including Novelion) with the thirteen provincial and territorial securities regulatory authorities (Canadian Securities Administrators or "CSA") at its website at [www.sedar.com](http://www.sedar.com). We make our periodic and current reports available on our internet website, free of charge, as soon as reasonably practicable after such material is electronically filed with, or furnished to, the SEC. Our internet address is [www.novelion.com](http://www.novelion.com).

### **Item 1A. Risk Factors.**

***Our business depends on the success of our two commercialized products, lomitapide and metreleptin. These products may not be successful and may not generate sales at levels sufficient to sustain our operations.***

Our business depends on the success of our two commercialized products, lomitapide and metreleptin. We have a limited history of generating revenues from the sale of such products, and we anticipate that we will continue to incur significant costs and expend significant operating and management resources associated with developing and commercializing them. We may not be able to undertake such efforts or investments on a timely basis, at desirable levels or on favorable terms, or at all, including as a result of a decline in our total revenues, insufficient cash flows and overall availability of our resources. Even if we are able to undertake any such efforts and investments, these products may not be successful and may not generate sales at levels sufficient to sustain our operations.

Our ability to meet expectations with respect to sales of these products (and revenues from such sales), and to attain profitability and positive cash flow from operations, in the time periods we anticipate, or at all, will depend on the commercial success of these products in the U.S. and other key markets, which will depend on, among other factors: obtaining or maintaining regulatory approvals for such products, and obtaining or maintaining such regulatory approvals without onerous restrictions or limitations in the resulting label; obtaining or maintaining favorable pricing for and reimbursement of these products in the U.S. and in key countries outside the U.S.; and the availability of financial assistance for individuals who otherwise cannot afford our products. Commercial success of these products will also significantly depend on continued and expanded acceptance by the medical community of, and market demand and medical need for, these products, including, in the case of lomitapide, in light of



the availability of PCSK9 inhibitor products, which has had a significant adverse impact on sales of lomitapide in the U.S. We expect that named patient sales of lomitapide in Brazil in the near-term will continue to be a significant source of revenues; however, we expect net product sales from named patient sales in Brazil and other countries to continue to fluctuate quarter-over-quarter significantly more than sales in the U.S. and other markets where our products are approved for commercial use, which could have a negative impact on our stock price. If lomitapide or metreleptin does not achieve, expand or maintain commercial success, our future operating results and financial condition may be materially adversely affected, and we may never achieve profitability.

***We may not be able to maintain or expand market acceptance for lomitapide or metreleptin in the U.S. or to gain market acceptance in markets outside the U.S. where we commercialize such products, and we may continue to see a significant number of patients who choose not to start or stay on therapy.***

The commercial success of lomitapide and metreleptin will depend primarily upon our ability to maintain and expand the acceptance of these products by the medical community, including by physicians and healthcare payers, and by the relevant patients in the U.S., and to gain and maintain such acceptance in countries outside the U.S. where such products are or may be commercialized. The degree of market acceptance of our products will therefore depend on a number of factors, including:

- physicians' views as to the scope of the approved indication and limitations on use and warnings and precautions contained in the approved labeling or prescribing information for our products, including the boxed warnings on the JUXTAPID and MYALEPT labels;
- the extent to which the instructions to patients in the JUXTAPID label to cease therapy upon the occurrence of severe diarrhea and related language about the risk of gastrointestinal issues while on JUXTAPID will negatively affect the ability or willingness of a physician to prescribe JUXTAPID, a patient to be willing to initiate or continue on JUXTAPID therapy, or insurance companies, managed care organizations, other private payers, and government entities that provide reimbursement for medical costs to continue to provide reimbursement for JUXTAPID;
- the willingness of insurance companies, managed care organizations, other private payers, and government entities in the U.S. that provide reimbursement for medical costs to continue to provide reimbursement for JUXTAPID and MYALEPT at the price at which we offer each and without imposing restrictions on the use of the product, such as, for JUXTAPID, requiring adult homozygous familial hypercholesterolemia ("HoFH") patients to undergo treatment with a PCSK9 inhibitor product prior to starting JUXTAPID or switching current JUXTAPID patients to PCSK9 inhibitor products, and for MYALEPT, requiring potential patients to first undergo leptin level tests, in each case, which delay or otherwise impact reimbursement;
- the ability and willingness of adult HoFH and GL patients to pay, or to arrange for payment assistance with respect to, any patient cost-sharing amounts for JUXTAPID or MYALEPT applicable under their insurance coverage, and the availability of co-pay assistance;
- the availability, efficacy, safety, tolerability, and pricing of competitive therapies, including, in the case of lomitapide, PCSK9 inhibitor products, which risk is described in more detail under the risk factor "*We are subject to intense competition. If we are unable to compete effectively, we may not be able to achieve our revenue goals or achieve profitability or cash-flow positive operations. Competitive products could render lomitapide or metreleptin, or any other product or product candidate that we develop or acquire, non-competitive or obsolete, which would have a materially negative impact on our results of operations.*";
- our ability to invest capital, including new capital (which we may not be able to raise in a timely manner, on favorable terms, or at all) into the development and commercialization efforts for our products; and
- the effectiveness of our sales, marketing and distribution strategies and our ability to execute on and achieve these strategies, particularly in light of our recent workforce reduction and cost-containment plan, as well as the continuing challenges to the lomitapide business, including, among other things, the impact of competitive products on JUXTAPID sales, and the use of a contract sales force in Japan.

Further, we may not be able to expand, or even maintain, market share for lomitapide due to the risk of hepatotoxicity, and the enhanced requirements of the recently modified and implemented JUXTAPID Risk Evaluation Management Strategy ("REMS") program (as so modified, the "JUXTAPID REMS program"), which program includes complex and burdensome certification, education, prescribing and documentation requirements. We may lose JUXTAPID patients temporarily or permanently, or add new adult patients with HoFH at a slower than expected pace, for a variety of reasons, including: the inability

to certify new healthcare professionals on a timely basis or at all; the failure of new healthcare professionals and patients to complete the patient counseling requirements and sign and submit the required patient acknowledgment form on a timely basis or at all; the failure of prescriptions for JUXTAPID to meet all of the requirements of the JUXTAPID REMS program, including any resulting payer issues or delays; and that the more complex and involved efforts to educate healthcare professionals of the goals of the JUXTAPID REMS program, and related documentation, may cause healthcare professionals to stop or delay treatment with JUXTAPID, or try alternative therapies for adult HoFH patients before starting or continuing JUXTAPID treatment. Further, it is possible that the enhanced requirements of the JUXTAPID REMS program will cause JUXTAPID to be viewed as more risky than other therapies, and some healthcare providers and patients may determine that the burdens of the JUXTAPID REMS program, and perceived risks of the therapy, outweigh the benefits of JUXTAPID as compared to other available treatments, which would have a negative impact on JUXTAPID revenues. Further, the JUXTAPID REMS program could discourage insurance companies, managed care organizations, other private payers, and government entities that provide reimbursement for medical costs from continuing to provide reimbursement for JUXTAPID, which would also have a negative impact on JUXTAPID revenues. Even without taking into account the implications of the JUXTAPID REMS program, some physicians and HoFH patients may simply determine that the benefits of lomitapide in reducing low-density lipoprotein cholesterol (“LDL-C”) levels do not outweigh the risks associated with the product, including those risks described in the boxed warning for JUXTAPID in the U.S. and in the prescribing information for lomitapide in the other countries in which it is approved, which warn that lomitapide can cause hepatotoxicity.

Similarly, MYALEPT is available only through a REMS program (the “MYALEPT REMS program”), because of the potential for development of anti-metereptin antibodies and the associated risks of serious adverse sequelae (such as severe infections, excessive weight gain, glucose intolerance, diabetes mellitus) and risk of lymphoma. As part of this program, we must certify all healthcare providers who prescribe MYALEPT, certify the pharmacies that dispense the medicine, and obtain prescriber attestation that each patient has a diagnosis consistent with GL. Even without taking into account the implications of the MYALEPT REMS program, some physicians and congenital or acquired GL patients may determine that the benefits of metreleptin in treating complications of leptin deficiency in GL do not outweigh the risks of using metreleptin, including those risks set forth in the boxed warning for MYALEPT in the U.S., which label warns of the risk of anti-metereptin antibodies with neutralizing activity and the risk of lymphoma.

In addition, we have adopted risk management plans in other countries where we have obtained approval of lomitapide to help educate physicians on the safety information for lomitapide and appropriate precautions to be followed by healthcare professionals and patients. Other countries that may approve lomitapide or metreleptin may require risk management plans that may be similar to or more onerous than those we have adopted to date. The prescribing information for each product also describes a number of additional contraindications, warnings, and precautions, including those related to pregnancy and potential adverse interactions with other drugs, and other potential adverse reactions, that could limit the market acceptance of lomitapide and metreleptin. These contraindications, warnings, and precautions make it more difficult for some patients to decide to begin therapy or to stay on therapy. GI adverse reactions, which are common with lomitapide and the risk of which can be reduced only by adherence to a low-fat diet, and elevated alanine aminotransferase (“ALT”) and aspartate aminotransferase (“AST”) also lead to treatment discontinuation in a significant percentage of lomitapide patients. With respect to metreleptin, concerns related to the route of administration of metreleptin, as a daily injection, may deter some patients from beginning therapy or staying on therapy. As a result, even if a physician prescribes one of our products, the prescription may not result in a patient beginning therapy or staying on therapy.

If we are not able to achieve a high degree of market acceptance of lomitapide in the treatment of adult patients with HoFH and metreleptin in the treatment of GL, we may not be able to achieve our revenue goals or other financial goals or to achieve profitability or cash-flow positive operations in the time periods we expect, or at all, and our results of operations and stock price could be negatively impacted.

***We may not have sufficient cash to support further development and growth of our business and we will likely need to raise substantial additional capital in the near term. Raising additional capital may restrict our operations, require us to relinquish rights, including to our products and intellectual property, or cause dilution to our existing shareholders. If additional capital is not available at all or on acceptable terms when we need it, we will have to delay, reduce or cease operations.***

Aegerion’s level of indebtedness, the significant negative impact of PCSK9 inhibitor products on U.S. JUXTAPID sales, the payments by Aegerion to the government under the Settlement, and the costs and expenses Aegerion has incurred, and expects to continue to incur, in connection with the JUXTAPID Investigations and other ongoing government investigations and related matters have significantly diminished and are expected to further significantly diminish the capital we have to fund anticipated and unanticipated expenses and any strategic needs or opportunities that may arise. These significant expenses coupled with our current and expected revenue levels could leave us with insufficient cash to sustain our operations. Aegerion’s current level of indebtedness primarily consists of the Convertible Notes (\$325 million principal, due in August 2019), the Senior Loan (with a

current principal amount outstanding of \$38.1 million, due in July 2019), and the New Loan (\$20 million in principal outstanding as of March 16, 2018, due at the earliest of (i) August 1, 2019, (ii) 30 days prior to the maturity date of the Convertible Notes, (iii) the date that any restructuring or recapitalization of all or substantially all of the Convertible Notes, including any exchange offer or similar transaction, is substantially consummated, and (iv) upon acceleration of the obligations under the New Loan Agreement), as described, and as such terms are defined in the Part I, Item 1 - " *Business - Recent Corporate and Securities Transactions* " section of this Annual Report.

In January 2018, as a result of the challenges we have been facing and in an effort to manage our cash resources, we announced significant operating cost and workforce reduction plans and our intention to review our capital structure. We may need to implement additional cost containment measures based on our expected revenues and expenses, as well as the upcoming maturity of Aegerion's Convertible Notes, the Senior Loan and the New Loan Agreement. There can be no assurance, however, that any cost containment measures, including the recent cost and workforce reductions, will result in the cost savings we anticipate or that additional cost containment measures will be capable of being obtained or implemented. Accordingly, we will likely need to seek additional capital through debt and/or equity financing to service our indebtedness, strengthen our cash position and fund our operations. We may not be able to obtain additional capital when we need it, or such capital may not be available on terms that are favorable to us, particularly while the Convertible Notes, the Senior Loan and the New Loan Agreement are outstanding. We may also pursue opportunities to obtain additional external financing in the future through collaborations, strategic licensing arrangements, lease arrangements related to facilities, in order to, among other things, fund operations or finance potential product acquisitions and maintain sufficient resources for unanticipated events. Any such additional financing may not be available when we need it or may not be available on terms that are favorable to us. Our need to raise additional capital in the near term, the size of any such financings and the availability and terms of any such financings, will depend on many factors, including:

- the success of our commercialization efforts and the level of revenues generated from sales of lomitapide and metreleptin in the U.S., and of lomitapide in other key countries where it is approved and being commercialized, including Japan;
- the status of ongoing or recently concluded government investigations and lawsuits, such as the Settlement and the JUXTAPID Investigations, including relevant obligations, the disclosure of possible or actual outcomes, and the negative publicity surrounding such matters, and the costs associated with the resolution of these investigations and lawsuits, including the civil penalties, restitution and settlement amounts discussed in the " *Legal Proceedings* " section of this Annual Report and the cost of implementing and complying with the CIA, the DPA, and the FDA Consent Decree and criminal probation terms;
- the timing and costs of satisfying our debt obligations, including interest payments and any amounts due upon the maturity of such debt, including under the indebtedness described above;
- the level of revenues we receive from named patient sales of our products in Brazil and other key countries where a mechanism exists to sell the product on a pre-approval basis in such country based on U.S. approval of such products or EU approval of lomitapide, particularly in light of the regulatory approval of Amgen's PCSK9 inhibitor product in Brazil in April 2016, the potential availability of that and other PCSK9 inhibitor products on a named patient sales basis in Brazil, the additional requirements that have been recently imposed on named patient sales of pharmaceutical products in Brazil, including our products, and potential future additional requirements or limitations, and the ongoing court proceedings in Brazil reviewing the regulatory framework for named patient sales;
- the level of physician, patient and payer acceptance of lomitapide and metreleptin, and the extent of the negative impact of and other risks associated with the availability of PCSK9 inhibitor products on sales of JUXTAPID in the U.S.;
- our ability to continue to manage our costs and expenses to better align with our revenues and strengthen our capital structure, while supporting approved products in a compliant manner;
- our ability to provide security to collateralize any financings, which may be required by lenders as a condition to providing us with any funding, particularly given the fact that substantially all of Aegerion's assets have been pledged as collateral under the Senior Loan and the New Loan Agreement, including the intellectual property of metreleptin and lomitapide;
- gaining regulatory and pricing and reimbursement approvals to market our products in countries in which the products are not currently approved and/or reimbursed, where it makes business sense to seek such approval, without significant restrictions, discounts, caps or other cost containment measures, including regulatory and pricing and reimbursement approval of metreleptin in the EU for both GL and the PL subgroup, in connection with which we filed an MAA in the EMA in December 2016, and the timing and costs of seeking such approvals;

- the timing and cost of lifecycle management and clinical development activities, particularly our anticipated trial assessing metreleptin in patients with hypoleptinemic metabolic disorder (“HMD”);
- the willingness of insurance companies, managed care organizations, other private payers, and government entities that provide reimbursement for medical costs in the U.S. to continue to provide reimbursement for our products at the prices at which we offer our products without imposing any additional major hurdles to access or other significant restrictions or limitations, and the ability and willingness of HoFH and GL patients to pay, or to arrange for payment assistance with respect to, any patient cost-sharing amounts for our products applicable under their insurance coverage, particularly in light of recent reductions in contributions to 501(c)(3) patient organizations by pharmaceutical companies;
- the cost of maintaining the sales and marketing capabilities necessary for the commercialization of our products for their targeted indications in the market(s) in which each has received regulatory approval and we elect to commercialize such products, to the extent reimbursement and pricing approvals are obtained, and certain other key international markets, if approved;
- the timing and costs of future business development opportunities;
- the cost of filing, prosecuting and enforcing patent claims, including the cost of defending any challenges to the patents or our claims of exclusivity;
- the costs of our manufacturing-related activities and the other costs of commercializing our products;
- the levels, timing and collection of revenues received from sales of our products in the future;
- whether we are successful in our efforts to defend ourselves in, or to settle on acceptable terms, any significant litigation, including litigation that may result, directly or indirectly from the Settlement; and
- the cost of our current and future observational cohort studies and other post-marketing commitments, including to the FDA, the EMA and in any other countries where our products are ultimately approved.

To the extent that we raise additional capital through the sale of equity or convertible debt securities, the ownership interest of our existing shareholders will be diluted, and the terms may include liquidation or other preferences that adversely affect the rights of our shareholders. For example, our existing shareholders may be diluted if the Convertible Notes or the warrants issued in connection with the New Loan Agreement are converted into Novelson common shares. Additional debt financing similar to or more burdensome than our outstanding debt, if available, could result in increased fixed payment obligations and may involve agreements that include granting of collateral, subordinating the seniority of our security interests to Aegerion’s assets under the Senior Loan to Aegerion, granting instruments convertible into our equity, and impose additional covenants limiting or restricting our ability to take specific actions such as incurring debt, making capital expenditures, undertaking business development activities or declaring dividends. If we raise additional funds through collaboration, strategic alliance and licensing arrangements with third parties, we may have to relinquish valuable rights to our technologies, future revenue streams or product candidates, or grant licenses on terms that are not favorable to us.

We may be unable to obtain additional financing on favorable terms, at required levels, in a timely manner, or at all. If we are unable to obtain additional financing, including for purposes of settling conversions of the Convertible Notes, or repaying the Senior Loan or amounts under the New Loan Agreement, we may be required to delay, reduce or cease operations, including our planned development, sales and marketing and business development efforts. Any of these outcomes would harm our business, financial condition and operating results. The source, timing, terms and availability of any future financing will depend principally upon equity and debt market conditions, interest rates and, more specifically, on our commercial success, the status of Aegerion’s ongoing government investigations, including the Investigation, and the results of our future development efforts.

***Servicing Aegerion’s debt requires a significant amount of cash. Aegerion may not have sufficient cash from its business to make payments on its debt, and it may not have the ability to raise the funds necessary to settle conversions of, or to repurchase, the Convertible Notes upon a fundamental change, which could adversely affect our business, financial condition, results of operations on a consolidated basis, and our ability to respond to changes in our business.***

In August 2014, Aegerion incurred indebtedness in the amount of \$325.0 million in aggregate principal with additional accrued interest under the Convertible Notes, for which interest is payable semi-annually in arrears on February 15 and August 15 of each year.

On June 14, 2016, Aegerion entered into the Senior Loan Agreement pursuant to which Aegerion could initially borrow up to \$15.0 million used to fund Aegerion's working capital. Since the consummation of the business combination, Aegerion has continued to borrow pursuant to the terms of the Senior Loan Agreement, which has been amended from time to time, including on March 15, 2018 pursuant to the Amended and Restated Senior Loan Agreement. As of March 15, 2018, there was approximately \$38.1 million outstanding under the Amended and Restated Senior Loan Agreement, including all accrued interest paid in kind. The Senior Loan accrues interest at the rate of 8.0% per annum (which increases by 3.0% in connection with an event of default), which accrues and compounds quarterly in arrears until July 1, 2019, the maturity date of the Senior Loan. Also on March 15, 2018, Aegerion entered into the New Loan Agreement with the Lenders, pursuant to which the Lenders provided a single-draw term loan to Aegerion in an aggregate amount of \$20.0 million, and secured by substantially all of Aegerion's assets, subordinated to the Senior Loan. Interest on the New Loan accrues at 9.0% per annum, and the New Loan matures on the earliest of (i) August 1, 2019, (ii) 30 days prior to the maturity date of the Convertible Notes, (iii) the date that any restructuring or recapitalization of all or substantially all of the Convertible Notes, including any exchange offer or similar transaction, is substantially consummated, and (iv) upon acceleration of the obligations under the New Loan Agreement.

Aegerion's business may not generate sufficient cash from operations to service its debt. If Aegerion is unable to generate such cash, it may be required to adopt one or more alternatives, such as selling or licensing one or both of its products, or portions of its rights thereto, such as rights to certain geographies or key markets, further reducing the size of its workforce and curtailing operations and planned development activities, restructuring debt or obtaining financing on terms that may be onerous, if such financing is available at all. Aegerion's ability to refinance this indebtedness will depend on the debt and capital markets, which are unpredictable, and our financial condition on a consolidated basis at such time. Aegerion may not be able to engage in any of these activities or engage in these activities on desirable terms, which could cause a default on these debt obligations, resulting in a materially adverse effect on our consolidated business.

Aegerion's significant indebtedness, and our financial obligations and contractual commitments, including the payments Aegerion is required to make under the Settlement, could materially and adversely affect our ability to finance our operations or capital needs or to engage in other business activities. For example, they could:

- limit our ability to borrow additional amounts, or raise additional capital through equity or other types of financings, for supporting our operations, including for working capital, capital expenditures, acquisitions, payment of expenses, general corporate purposes or other purposes;
- place us at a disadvantage compared to our competitors who have less debt;
- limit our flexibility in planning for, or reacting to, changes in our business and our industry; and
- make us more vulnerable to adverse changes in general U.S. and worldwide economic, industry and competitive conditions and adverse changes in government regulation.

In addition, we cannot be sure that additional financing will be available when required or, if available at all, will be on terms satisfactory to us. Further, even if we are able to obtain additional financing, the terms may impose burdensome constraints, including conditions on how we can operate our business, and we may be required to use proceeds to repay a portion of Aegerion's debt and subordinate our security interest in Aegerion's assets which secures the Senior Loan to Aegerion. Holders of the Convertible Notes have the right to require the repurchase of their notes for cash upon the occurrence of a fundamental change at a repurchase price equal to 100% of the respective principal amount, plus accrued and unpaid interest, if any. Subject to certain exceptions as provided in the indenture governing the Convertible Notes, a fundamental change includes (a) delisting of Novelion's common shares, (b) liquidation of Aegerion, (c) the acquisition of 50% or more of the voting interests in Aegerion, (d) an event in which Aegerion merges or consolidates with another entity and (e) an event in which Aegerion conveys, sells, transfers or leases all or substantially all of its assets to another entity. Among the exceptions provided in the indenture are for transactions described in (c), (d) and (e) in which (i) Aegerion's common stock holders immediately prior to the transaction have the right to exercise, directly or indirectly, 50% or more of the total voting power of the capital stock of the continuing or surviving entity or transferee or parent thereof following the transaction or (ii) 90% of the consideration paid for Aegerion's common stock in a transaction consists of stock that is or will be quoted on the New York Stock Exchange or NASDAQ.

Further, unless Aegerion elects to deliver our common shares to settle any conversions of Convertible Notes, Aegerion would be required to settle a portion or all of the conversion obligation through the payment of cash, which could adversely affect its liquidity. Aegerion may not have enough available cash or be able to obtain financing at the time it is required to make repurchases of Convertible Notes surrendered therefor or Convertible Notes being converted. The failure to repurchase Convertible Notes at a time when the repurchase is required by the indenture or to pay any cash payable on future conversions of the Convertible Notes as required by the indenture would constitute a default under the indenture. A default under the indenture or a fundamental change

itself could also lead to a default under agreements governing our or Aegerion's current and future indebtedness, including the Senior Loan and the New Loan. If the repayment of such indebtedness were to be accelerated after any applicable notice or grace periods, Aegerion may not have sufficient funds to repay such indebtedness and repurchase the Convertible Notes or make cash payments upon conversions thereof or other required payments on its other indebtedness. In addition, even if holders of the Convertible Notes do not elect to convert their Convertible Notes, Aegerion could be required under applicable accounting rules to reclassify all or a portion of the outstanding principal of the Convertible Notes as a current rather than non-current liability, which would result in a material reduction of Aegerion's net working capital, possibly creating a working capital deficit.

Further, Aegerion's obligations under the Settlement, or other obligations that may be imposed in connection with other investigations with governmental agencies, could have a material adverse effect on our ability to raise capital or borrow funds that might be necessary to fund our operations.

Any of these factors could materially and adversely affect our business, financial condition and results of operations on a consolidated basis. In addition, if we or Aegerion incur additional indebtedness, the risks related to our business and our ability to service or repay our indebtedness would increase.

***Aegerion's resolution of investigations with governmental agencies, including the DOJ and the SEC, in connection with its U.S. commercial activities and disclosures related to JUXTAPID could have a material adverse effect on our business, including our ability to raise capital, results of operations, and financial condition. The Investigation and Settlement could result in additional third party claims or litigation, which could be costly to resolve.***

On September 22, 2017, Aegerion entered into a series of agreements in an effort to resolve investigations being conducted by the DOJ and the SEC regarding Aegerion's U.S. commercial activities and disclosures related to JUXTAPID (the "JUXTAPID Investigations"). Under these agreements, Aegerion is or will become obligated under these various agreements and judgments (collectively referred to as the "Settlement"), including a plea agreement with the DOJ (the "Plea Agreement"), a civil settlement agreement with the DOJ (the "Civil Settlement Agreement"), separate civil settlement agreements with multiple U.S. states, a final judgment entered in connection with a complaint filed by the SEC, a three-year deferred prosecution agreement with the DOJ (the "DPA"), the five-year corporate integrity agreement with the Office of Inspector General of the Department of Health and Human Services (the "CIA"), and a pending civil consent decree with the FDA and the DOJ relating to the JUXTAPID REMS (the "FDA Consent Decree"). Each of these Settlement agreements is described in the "Legal Proceedings" section of this Annual Report. On January 30, 2018, a U.S. District Court (the "Court") judge sentenced Aegerion after the judge accepted Aegerion's guilty criminal plea. The terms of the sentence are identical, in terms of monetary impact to Aegerion, to the terms recommended by the DOJ and Aegerion in the Plea Agreement. The Court did not impose a criminal fine and instead established a restitution fund in the amount of \$7.2 million, which includes administration costs, to be paid in installments over three years, plus interest on any unpaid balance at a rate of 1.75% per annum. As contemplated by the Plea Agreement, the Court put Aegerion on probation for three years and required Aegerion to not waste, or without permission of Aegerion's probation officer, sell, assign or transfer its assets.

Under the terms of the agreements and the sentence, Aegerion is required to pay approximately \$40.1 million penalties, plus interest, over three years, which includes the restitution described above, a civil penalty of \$4.1 million to be paid to the SEC pursuant to an SEC Judgment, and \$28.8 million, including (\$2.7 million designated for certain states), to be paid pursuant to the Civil Settlement Agreement, which is a significant financial burden given Aegerion's financial condition. Aegerion made an initial payment to the DOJ on February 12, 2018, and an initial payment to certain states on February 15, 2018. On February 20, 2018, the DOJ filed a stipulation of dismissal in the civil qui tam matter. The FDA Consent Decree remains subject to approval by a U.S. District Court Judge.

Pursuant to the Settlement, Aegerion is also required to implement various remedial and compliance measures, which could negatively impact our results of operations, as such efforts will require Aegerion to expend significant costs and resources and will divert those resources from investing in the commercialization of our products and other potential development and growth initiatives. Further, Aegerion has no experience in complying with a settlement of this type and magnitude and may be unsuccessful in implementing all of the elements of the Settlement in a timely or satisfactory manner, or at all. Failure to comply with any provisions of the Settlement could result in the imposition of additional fines, penalties and obligations by the applicable government agency, and could subject Aegerion to prosecution.

For example, the CIA, which has taken effect, requires Aegerion, among other things, to: maintain a compliance program that includes comprehensive written policies and procedures and appropriate conduct related to sales, marketing, reimbursement, incentive compensation and other matters; conduct training and education regarding the compliance program and requirements of the CIA; conduct an independent review and analysis of Aegerion's systems, transactions, risk assessment and mitigation process and other compliance activities; maintain a disclosure program that allows individuals to report issues or questions associated with

Aegerion's policies, conduct, practices or procedures; have a field force monitoring program to evaluate and monitor sales personnel's interactions with healthcare professionals; monitor non-promotional activities, including consultants, donations to independent third party patient assistance programs and other types of grants; have certain requirements for the variable compensation programs for its U.S. sales personnel; and have an executive financial recoupment program that puts at risk of forfeiture and recoupment performance pay for certain of Aegerion's and the Company's executives. Aegerion also has reporting obligations under the CIA, including with respect to any ongoing investigation or legal proceeding involving an allegation that Aegerion has engaged in any fraudulent activities or committed a crime, any communications with the FDA regarding improper promotion or marketing of Aegerion's products and any probable violations of criminal, civil or administrative laws applicable to federal healthcare programs. In the event Aegerion breaches the CIA, the government could seek to impose remedies provided for in the CIA, including seeking to impose stipulated penalties against Aegerion and/or seeking to exclude Aegerion from participation in federal healthcare programs.

Similarly, the DPA, which has also taken effect, provides that Aegerion must continue to cooperate fully with the DOJ concerning its investigation into other individuals or entities. The DPA also provides that Aegerion must maintain a robust Compliance and Ethics Program (as defined in the DPA) that consists of, among other things, a designated Compliance Officer and Compliance Committee; written compliance policies and procedures; a training program focused on Aegerion's compliance policies and procedures; a disclosure program to allow individuals to report potential legal and/or compliance violations, including violations of HIPAA; a non-retaliation policy; and a monitoring and auditing program. Under the DPA, Aegerion, as well as the Board of Directors of the Company (or a designated committee thereof), must also conduct regular reviews of its Compliance and Ethics Program, provide certifications to the DOJ that the program is believed to be effective and notify the DOJ of any probable violations of HIPAA. In the event Aegerion breaches the DPA, there is a risk the government would seek to impose remedies provided for in the DPA, including instituting criminal prosecution against Aegerion and/or seeking to impose stipulated penalties against Aegerion.

The FDA Consent Decree, which is still pending, requires Aegerion, among other things, to comply with the JUXTAPID REMS program; retain a qualified independent auditor to conduct annual audits of its compliance with the JUXTAPID REMS program; and remediate any noncompliance identified by the auditor within specified timeframes. In the event Aegerion fails to comply with the JUXTAPID REMS program or any other provisions of the FDA Consent Decree, Aegerion could be subject to additional administrative remedies, civil or criminal penalties and/or stipulated damages. Aegerion is required to notify the FDA in advance of certain changes in control, or changes in its business that may affect its operations, assets, rights or liabilities in the U.S.

Also, in the event Aegerion fails to comply with the terms of the criminal probation, it could be subject to criminal penalties and/or damages. Further, the JUXTAPID Investigations and the Settlement have resulted in third party demands and may in the future give rise to third party demands, claims or litigation, including demands or claims by, or litigation with, third party payers, healthcare providers, or patients or investors, for matters related to the subject matter of or disclosure in connection with such Investigations or the Settlement. For example, Aegerion has received two demand letters, one from an insurance company, seeking reimbursement for the JUXTAPID claims it paid based on alleged false misrepresentations made by Aegerion, and the second from an investor in Aegerion's Convertible Notes alleging that it purchased the notes based on misrepresentations and omissions. The JUXTAPID Investigations and the Settlements could also lead to potential investigations, claims or litigation by consumer protection agencies or groups (including State Attorneys General consumer protection units), or provide a basis for product liability claims or litigation. Such claims or investigations, regardless of the merits, can be costly to resolve, and may lead to lengthy litigation and settlement negotiations, the results of which could require us to pay significant amounts of damages. Such third party claims often unfold in a public manner, and any public claims can encourage further claims from additional third parties.

The results of the JUXTAPID Investigations (including the Settlement) could also have adverse effects on Aegerion's commercial operations, research and development activities, contracts or business in general because:

- the JUXTAPID Investigations have diverted, and compliance with the terms of the Settlement will divert resources away from developing and commercializing lomitapide and metreleptin, and our ability to meet expectations with respect to sales of these products may be negatively impacted;
- despite remedial efforts, the reputational harm from the JUXTAPID Investigations and the Settlement (including in connection with the Court's initial rejection of the Plea Agreement) could subject us to increased governmental, industry and public scrutiny or criticism, which could negatively impact physicians' inclination to prescribe (or patients' willingness to use) our products, or dissuade vendors, distributors, partners or other third parties from working or collaborating with us or Aegerion;

- the JUXTAPID Investigations have diverted, and compliance with the Settlement may continue to divert, the attention of management from operating our business, and may be disruptive to our employees, result in employee attrition, and make it more difficult to attract qualified candidates for employment;
- our business development efforts may be limited as we will have fewer resources available to pursue our commercialization efforts and potential strategic acquisitions or licensing arrangements, and because certain payments under the Settlement could be accelerated in connection with certain transfers of Aegerion's rights in our products or other business combinations; and
- our stock price may suffer, and our ability to raise capital in the future on favorable terms or at all may be adversely affected, depending on the perception of the terms of the Settlement (and in connection with underlying Investigations) and any adverse consequences that may result from the Settlement, including if we are unable to comply with the Settlement, or if the Settlement results in additional litigation or investigations.

The Settlement could encourage investigations by foreign governments, or we may face additional investigations by U.S. federal, state or local agencies for activities that were not covered by the releases provided in the Settlement or our other operations (including international operations), especially in light of the government's recitation of its assessment of the background of the investigation in the criminal filings made by the government and in the Civil Settlement Agreement as well as the Court's commentary in connection with its rejection of the Plea Agreement in September 2017. For example, the Settlement did not resolve the DOJ and SEC's investigations into Aegerion's operations in Brazil or the DOJ's inquiry into donations Aegerion made in 2015 and 2016 to 501(c)(3) organizations that provide financial assistance to patients. Aegerion continues to cooperate with the DOJ and the SEC with respect to such matters.

Any additional claims or investigations could distract management, and may not be covered by our D&O insurance and accordingly could cause our legal costs to increase. The outcome of any such inquiries could have a material and negative effect on our business.

Further, the Settlement did not resolve the DOJ and SEC investigations into the conduct of individuals. Aegerion continues to cooperate with the DOJ and the SEC with respect to such investigations. Additionally, the Settlements do not resolve civil claims brought by relators against certain former employees, officers, directors and other third parties. In March 2014, an amended qui tam complaint was filed under seal in the District of Massachusetts against Aegerion, two former executive officers and a former employee, and on September 27, 2017, the qui tam relators filed a second amended complaint naming additional parties, including a former board member, former executives, and former employees of Aegerion, as well as other third parties. On February 20, 2018, the DOJ filed a stipulation of dismissal with respect to Aegerion in the civil qui tam matter. Aegerion is required under its bylaws to advance the reasonable legal costs and expenses of certain former executives and has and may elect to continue to advance such costs and expenses to certain other former and current employees, and other third parties with respect to their involvement in the JUXTAPID Investigations and in connection with their involvement in the civil suit brought by relators. These costs and expenses will continue to have a significant impact on Aegerion's costs in 2018 and perhaps beyond, although we expect that Aegerion's legal expenses for the JUXTAPID Investigations will continue to decrease year over year in the aggregate.

***A number of adverse effects have been reported in our clinical studies for lomitapide and metreleptin, and the prescribing information for each of lomitapide and metreleptin contains significant limitations on use and other important warnings and precautions, including boxed warnings in the U.S. labeling, any of which could negatively affect the market acceptance, dropout rates and marketing approval for our products. Post-marketing commitments could identify additional adverse events and safety or efficacy risks, which could further harm the market for lomitapide and metreleptin.***

The prescribing information for lomitapide in the U.S. and the EU and in the other countries in which lomitapide is approved contains significant limitations on use and other important warnings and precautions, including a boxed warning in the JUXTAPID labeling, and warnings in the prescribing information for LOJUXTA (lomitapide) hard capsules ("LOJUXTA") citing concerns over liver toxicity associated with use of lomitapide. The MYALEPT label in the U.S. has a boxed warning, citing the risk of anti-metreleptin antibodies with neutralizing activity and risk of lymphoma.

As discussed under the heading "*Phase 3 Clinical Study (HoFH)*," patients reported various adverse reactions in our Phase 3 study of lomitapide, including reports of gastrointestinal events by 93% of patients, and in our HoFH clinical trial, including diarrhea, nausea, vomiting, dyspepsia, abdominal pain, weight loss, abdominal discomfort, abdominal distension, constipation, flatulence, increased ALT, chest pain, influenza, nasopharyngitis, and fatigue. Elevations in liver enzymes and hepatic (liver) fat were also observed.



As discussed under the heading “*Phase 3 Clinical Study (GL)*,” GL patients in our Phase 3 study of metreleptin reported various adverse drug reactions, including weight loss, hypoglycemia, decreased appetite, fatigue, neutralizing antibodies and alopecia. Additionally, although none were assessed as drug related, there have been four reported treatment-emergent deaths over the course of the 14-year study duration, which reports were consistent with the underlying morbidity of lipodystrophy and included renal failure, cardiac arrest (with pancreatitis and septic shock), progressive end-stage liver disease (chronic hepatic failure), and hypoxic-ischemic encephalopathy. In the open-label, long-term, investigator-sponsored study of metreleptin for the treatment of metabolic disorders associated with LD syndromes initiated in 2000 and conducted at the National Institutes of Health (“NIH”), there have been two cases of peripheral T-cell lymphoma and one case of a localized anaplastic lymphoma kinase-positive anaplastic large cell lymphoma (a type of T-cell lymphoma) reported in patients with acquired GL (there was evidence of pre-existing lymphoma and/or bone marrow/hematologic abnormalities in the two patients with peripheral T-cell lymphoma before metreleptin therapy, and the third case of anaplastic large cell lymphoma occurred in the context of a specific chromosomal translocation).

These adverse events, coupled with the boxed warnings and other label restrictions, could cause healthcare providers, regulators and patients or potential patients to view the risks associated with our products as outweighing the benefits such products provide, which could cause patients to discontinue use and limit the number of new patients, thereby negatively affecting our revenues.

As part of our post-marketing commitment to the FDA for lomitapide, we have initiated an observational cohort study to generate more data on the long-term safety profile of lomitapide, the patterns of use and compliance and the long-term effectiveness of controlling LDL-C levels. As part of the post-marketing commitments to the FDA for metreleptin, we have initiated a long-term, prospective, observational study (product exposure registry) in patients to evaluate serious risks related to the use of the product. We have completed two of three sequential programs to expand the understanding of the immunogenicity of metreleptin and certain studies related to the manufacturing of metreleptin. The final program regarding the immunogenicity of metreleptin is expected to be initiated in 2018, and the two final post-marketing studies related to the manufacturing of metreleptin are expected to be completed by the deadlines set by the FDA. A failure to meet post-marketing commitments to the FDA, EMA or other regulatory authorities could impact our ability to continue to market lomitapide or metreleptin, respectively, in countries where we are unable to meet such commitments.

In addition, as part of our observational cohort studies or in the conduct of additional clinical studies or in post-marketing surveillance of our products, or if any completed clinical study data is re-evaluated, we or others may identify additional safety information on known or unknown side effects or new undesirable side effects caused by our products, or the data may raise other issues with respect to our products, and, in that event, a number of potentially significant negative consequences could result, including:

- we may experience a negative impact on market acceptance and dropout rates;
- regulatory authorities may suspend, withdraw or alter their approval of the relevant product;
- regulatory authorities may require the addition of labeling statements, such as warnings or contraindications or distribution and use restrictions, such as, for example, the modifications to the JUXTAPID label to include language instructing patients to cease therapy upon the occurrence of severe diarrhea;
- regulatory authorities may require us to issue specific communications to healthcare professionals, such as “Dear Doctor” letters;
- negative publicity, including safety communications;
- we may be required to change the way the relevant product is administered, conduct additional preclinical studies or clinical trials or restrict the distribution or use of the relevant product;
- patients could suffer harm, and we could be sued and held liable for harm caused to patients;
- the regulatory authorities may require us to amend the relevant REMS program or risk management plan; and
- our reputation may suffer.

Any known safety concerns for our products, or any unknown safety issues that may develop or surface, including drug interaction problems or an increase in the severity or frequency of known adverse events or the discovery of previously unknown adverse events, or the evaluation or reevaluation of study data could prevent us from achieving or maintaining market acceptance

of the respective product, affect our ability to obtain or retain marketing approval of the respective product in one or more countries, result in onerous restrictions on such approval or the implementation or modification of REMS program or any other enforcement actions, result in claims, lawsuits and increased regulatory scrutiny and affect our ability to achieve our financial goals.

***If we fail to obtain or maintain orphan drug or data and marketing exclusivity for our products in any country where exclusivity is available (including the U.S.) or our patent position does not adequately protect our products in any country, we will be unable to prevent competitors from selling generic versions of our products and others could compete against us more directly, which would harm our business, possibly materially.***

We rely on a combination of regulatory exclusivities and patents in the conduct of our business. We have obtained orphan drug exclusivity for JUXTAPID in the U.S. for the treatment of HoFH (which expires in December 2019). JUXTAPID's new chemical entity ("NCE") exclusivity expired on December 21, 2017, and, as such, an Abbreviated New Drug Application (an "ANDA") or 505(b)(2) NDA was submittable for JUXTAPID as of December 21, 2016, which lowers the barriers to entry for generic competitors. If one or more ANDA filers were to receive approval to sell a generic or follow-on version of JUXTAPID, those competitor products could potentially be marketed, and we would become subject to increased competition in the U.S., as early as December 21, 2019, the date on which JUXTAPID's orphan drug exclusivity ends, although we expect that any such launch would be delayed until at least February 21, 2020, the date on which JUXTAPID's composition of matter patent expires. The recent expiration of JUXTAPID'S NCE exclusivity and upcoming expiration of its orphan drug exclusivity make our patent protection even more critical.

Our lomitapide patent portfolio, which is described in detail under the heading "*Patent Rights and Regulatory Exclusivity*" in this Annual Report, includes a method-of-use patent that provides protection in the U.S. until 2027. There is currently no intellectual property protection or any orphan drug, data, or marketing exclusivity for lomitapide in the U.S. beyond 2027.

We have obtained orphan drug exclusivity for MYALEPT in the U.S. for the treatment of GL (which expires in February 2021). In the U.S., we also believe that metreleptin qualifies for a 12-year period of exclusivity, which will expire in 2026, under the Biologics Price Competition and Innovation Act (the "BPCI Act").

Our metreleptin patent portfolio, which is described in detail under the heading "*Patent Rights and Regulatory Exclusivity*" in this Annual Report, includes a method-of-use patent that provides protection in the U.S. until 2027. There is currently no intellectual property protection or any orphan drug, data, or marketing exclusivity for metreleptin in the U.S. beyond 2027. New indications for metreleptin are being investigated, including HMD, but it is not certain whether additional intellectual property designed to cover such indications or other forms of intellectual property can be obtained or will adequately protect us from competition.

We have also pursued or may pursue orphan drug designation and equivalent exclusivity opportunities for our products outside of the U.S. Despite the prevalence rate, lomitapide does not have orphan medicinal product exclusivity in the EU for the treatment of HoFH because the EMA views the relevant condition, for orphan drug purposes, to include both HoFH and heterozygous familial hypercholesterolemia ("HeFH"). Our non-U.S. patents for lomitapide, including the European Patent Office ("EPO") methods of use patent, expire in 2025. The EPO method of use patents are eligible for up to about three years of supplemental protection in certain EPO countries, and we have applied for such protection in the countries in which LOJUXTA is approved, on a country-by-country basis; in some countries supplemental protection has been granted to extend patent protection to July or August of 2028, while in other countries the application is still pending. An opposition was filed with respect to the EPO method of use patent, but has since been revoked. In Japan, where we launched JUXTAPID in December 2016, we have received orphan drug designation for JUXTAPID in the treatment of HoFH from Japan's regulatory authority, the Ministry of Health, Labor and Welfare ("MHLW") (which expires in September 2026). The patents issued in Japan directed to lomitapide methods-of-use are scheduled to expire in 2025 and may be eligible for supplemental protection until 2026.

In 2012, metreleptin was granted orphan designation by the EC for the treatment of Barraquer-Simons syndrome (acquired partial lipodystrophy ("PL")), Berardinelli-Seip syndrome (congenital GL), Lawrence syndrome (acquired GL) and familial PL, which is expected to expire 10 years from approval of any such indication by the EMA. If approved by the EMA, metreleptin would also be entitled to 10 years of market exclusivity in the EU. The patent issued in the EU directed to metreleptin methods-of-use expires in 2022.

We also have other forms of regulatory exclusivity for our products in certain other markets. However, there are many other countries, including some key markets for our products, like Brazil, in which we do not have intellectual property coverage for our products, and where neither orphan drug exclusivity nor data and marketing exclusivity is available. As with the U.S., without orphan drug or other forms of exclusivity in countries where we currently or plan to commercialize our products (if

approved), we could face generic competition in the near term, particularly if no other intellectual property coverage, including other types of exclusivity, is available.

Our commercial success with respect to our products will depend significantly on our ability to obtain and maintain regulatory exclusivity for our products and to protect our existing patent position with respect to our products, as well as our ability to obtain and maintain adequate protection of other intellectual property for any future products or product candidates we may acquire in the U.S. and other countries. If we do not adequately protect our intellectual property, competitors, including companies that sell generics, may be able to erode or negate any competitive advantage we may have, which could harm our business and ability to achieve our expected financial results. Our ability to use the patents and patent applications licensed to us to protect our business will also depend on our ability to comply with the terms of the applicable licenses and other agreements and to obtain requisite licenses. The laws of some foreign countries do not protect our proprietary rights to the same extent as the laws of the U.S., and we may encounter significant problems in protecting our proprietary rights in these countries.

The patent positions of biotechnology and pharmaceutical companies, including our patent position described in detail under the heading “*Patent Rights*” in this Annual Report, involve complex legal and factual questions, and, therefore, validity and enforceability cannot be predicted with certainty. Patents may be challenged, deemed unenforceable, invalidated or circumvented. U.S. patents and patent applications may also be subject to interference proceedings, ex parte reexamination, *inter partes* review (“IPR”) and post-grant review proceedings and supplemental examination and may be challenged in district court. Patents granted in certain other countries may be subjected to opposition or comparable proceedings lodged in various national and regional patent offices. These proceedings could result in either loss of the patent or denial of the patent application or loss or reduction in the scope of one or more of the claims of the patent or patent application. In addition, such interference, re-examination, opposition, post-grant review, IPR, supplemental examination or revocation proceedings may be costly. We will be able to protect our proprietary rights from unauthorized use by third parties only to the extent that our proprietary technologies and any future products or product candidates are covered by valid and enforceable patents or are effectively maintained as trade secrets.

For example, on August 28, 2015, the Coalition for Affordable Drugs VIII L.L.C. (the “CFAD”) filed two separate IPR petitions with the Patent Trial and Appeal Board (the “PTAB”) of the U.S. Patent and Trademark Office, challenging the validity of U.S. Patent Nos. 7,932,268, and 8,618,135, which are directed to methods-of-use for lomitapide. Although on March 6, 2017, the PTAB determined that the CFAD failed to show that the claims of these patents were unpatentable, we cannot predict whether an appeal or a request for a rehearing on this determination will be filed, or if additional IPR challenges will be filed by another entity, or the outcome of any future IPR, and responding to such claims is costly and diverts our limited resources from other business activities.

In the U.S., the Hatch-Waxman Act established a patent restoration term of up to five years as compensation for patent term lost during product development and the FDA regulatory review process. However, we may not be granted an extension in a particular country if we, for example, fail to apply within applicable deadlines, fail to apply prior to expiration of relevant patents or otherwise fail to satisfy applicable requirements. Moreover, the applicable time period of the extension or the scope of patent protection afforded could be less than we request. If we are unable to obtain patent term extension or restoration of the term of any such extension is shorter than we request, our competitors, including manufacturers of generic alternatives, may obtain approval of competing products following expiration of our patents, and regulatory exclusivity, if any, and our revenues could be reduced, possibly materially.

Even if we obtain orphan drug exclusivity for a product, that exclusivity may not effectively protect the product from competition because different drugs can be approved for the same condition. For example, in the U.S., even after an orphan drug is approved, the FDA can subsequently approve the same drug for the same condition if the FDA concludes that the later drug is safer, more effective or makes a major contribution to patient care, which could materially adversely affect our financial condition and results of operations.

With the enactment of the BPCI Act, an abbreviated pathway for the approval of biosimilar and interchangeable biological products was created. Under the BPCI Act, an application for a biosimilar product cannot be approved by the FDA until 12 years after the original reference product was approved under a BLA. The law is complex and is still being interpreted and implemented by the FDA. As a result, its ultimate impact, implementation, and meaning are subject to uncertainty. While it is uncertain when such processes intended to implement the BPCI Act may be fully adopted by the FDA, any such processes could have a material adverse effect on the future commercial prospects for metreleptin. In particular, the approval of a biological product biosimilar to one of our products could have a material negative impact on our business because it may be significantly less costly to bring to market and may be priced significantly lower than our products. While we believe that metreleptin, which is approved under a BLA, qualifies for the 12-year period of exclusivity, there is a risk that this exclusivity could be shortened due to congressional action or otherwise, or that the FDA will not consider metreleptin to be a reference product for competing products, potentially creating the opportunity for competition sooner than anticipated. Moreover, the extent to which a biosimilar, once approved, will

be substituted for metreleptin in a way that is similar to traditional generic substitution for non-biological products is not yet clear, and will depend on a number of marketplace and regulatory factors that are still developing.

Innovative medicinal products authorized in the EU on the basis of a full marketing authorization application (as opposed to an application for marketing authorization that relies on data in the marketing authorization dossier for another, previously approved medicinal product) are entitled to eight years' data exclusivity. During this period, applicants for approval of generics of these innovative products cannot rely on data contained in the marketing authorization dossier submitted for the innovative medicinal product. Innovative medicinal products are also entitled to ten years' market exclusivity. During this ten-year period no generic medicinal product can be placed on the EU market. The ten-year period of market exclusivity can be extended to a maximum of 11 years if, during the first eight years of those ten years, the Marketing Authorization Holder for the innovative product obtains an authorization for one or more new therapeutic indications which, during the scientific evaluation prior to their authorization, are held to bring a significant clinical benefit in comparison with existing therapies. If we are not able to gain or exploit the period of data exclusivity, we may face significant competitive threats to our commercialization of these compounds from other manufacturers, including the manufacturers of generic alternatives. Further, despite exclusivity protections, another company nevertheless could market another version of our product if such company submits a full NDA or a full application for marketing authorization in the EU with a complete human clinical trial program and obtains marketing approval of its product.

The degree of future protection for our products and proprietary rights is uncertain, and we cannot ensure that:

- we will be able to successfully develop or commercialize our product before some or all of our relevant patents or regulatory exclusivity expire, or in countries where we do not have patent protection or exclusivity;
- we or our licensors were the first to make the inventions covered by each of our pending patent applications and patents;
- we or our licensors were the first to file patent applications for these inventions;
- others will not independently develop similar or alternative technologies or duplicate any of our technologies;
- any of our pending patent applications or those we have licensed will result in issued patents;
- any of our patents or those we have licensed will be valid or enforceable;
- we are able to license patents or pending patents that are necessary or desirable to enforce or protect our patent rights on commercially reasonable terms or at all;
- any patents issued to us or our licensors and collaborators will provide a basis for any additional commercially viable products, will provide us with any competitive advantages or will not be challenged by third parties;
- we will develop additional proprietary technologies or product candidates that are patentable;
- orphan drug exclusivity marketing rights for our products in the U.S. will not be lost, if, for example, the FDA determines in the future that the request for orphan drug designation was materially defective or if the manufacturer is unable to assure sufficient quantity of the drug to meet the needs of patients with the rare disease or condition; or
- the patents of others will not have an adverse effect on our business.

If we do not obtain or maintain regulatory exclusivity and/or patent protection for our products, our business may be materially harmed.

***The number of patients affected by the diseases for which our products are approved, or for which we may seek approval in the near term, is small, and has not been established with precision. Our assumptions and estimates regarding prevalence may be wrong. If the actual number of patients is smaller than we estimate, or if any approval is based on a narrower definition of these patient populations, our revenues and our ability to achieve profitability and cash-flow positive operations will be adversely affected, possibly materially.***

The patient population for the diseases that our products treat is small, and networking, data gathering and support channels are not as established as those for more prevalent and researched disease indications, and as such, there is no patient registry or other method of establishing with precision the actual number of HoFH or GL and PL subset patients with the diseases our products treat in any geography. Estimating the prevalence of a rare disease is difficult and we therefore need to rely on assumptions, beliefs

and an amalgam of information from multiple sources, resulting in potential under- or over-reporting. There is no guarantee that our assumptions and beliefs are correct, or that the methodologies used and data collected have generated or will continue to generate accurate estimates. There is therefore significant uncertainty around the estimated total potential addressable patient population for treatment with lomitapide and metreleptin worldwide.

Medical literature has historically reported the prevalence rate of HoFH as one person per million, based on an estimated prevalence rate for HeFH of one person in 500. However, analyses of HoFH prevalence have been evolving in recent years and published medical literature suggests that the actual prevalence of both HeFH and HoFH may be significantly higher than the historical estimates. For example, in 2014, the European Atherosclerosis Society Consensus Panel on Familial Hypercholesterolemia published an article citing research that would result in an estimated prevalence of HoFH in the range of between one person in 300,000 and one person in 160,000 or 3.33 persons per million to 6.25 persons per million, which is consistent with estimates that can be derived from other publications over the last few years. The FDA cited this estimate in its review of PCSK9 inhibitor products in June 2015. Despite this research and citation thereof, there is no guarantee that the prevalence of HoFH is actually higher than historical literature had reported and the number of patients with HoFH could actually be significantly lower than historically reported. Given that JUXTAPID is a last-line treatment for adult HoFH patients, the market for JUXTAPID may be significantly smaller than the prevalence of HoFH suggested by current and historical medical literature.

We believe that the prevalence rate of HoFH in countries outside the U.S. is likely consistent with the prevalence rate in the U.S.; however, we expect that our net product sales in countries outside the U.S. are likely to be lower than in the U.S. given significant economic pressures to reduce healthcare costs in certain ex-U.S. countries, resulting in pricing controls, reimbursement restrictions and caps on patients treated and/or drug expenditures, the more widespread availability of apheresis, in certain countries, like Japan, and the possibility that genotyping may be required in some countries, reducing the number of patients diagnosed with HoFH.

Currently available data suggests that the approximate prevalence of GL in the U.S. is slightly under one per million persons, and for PL overall is three per million persons. Although the data are even more limited, the prevalence in the U.S. of a subset of more severe PL, which is comparable to the PL subset indication we are currently pursuing in the EU, is estimated to be between 0.5 and one per million persons. We believe that the prevalence rate of GL and PL, and correspondingly the PL subset, in countries outside the U.S. is likely consistent with the prevalence rate in the U.S. There is no guarantee, however, that our estimates are correct. The actual prevalence of GL, PL and the PL subset may be significantly lower than we expect. Ultimately, the likely size of the total addressable market in the U.S. and other key markets where metreleptin is or may be sold, if approved, will be better understood only after we have substantial commercial history selling metreleptin.

If the total addressable market for our products in the U.S. and other key markets is smaller than we expect, then it may be more difficult for us to achieve our revenue goals and estimates and to attain profitability and meet our expectations with respect to results of operations and cash flows.

***If we are unable to establish and maintain effective sales, marketing and distribution capabilities, or to enter into agreements with third parties to do so, we will be unable to successfully commercialize our products.***

We are selling JUXTAPID and MYALEPT directly in the U.S., using our own marketing and sales resources, after converting our former contract sales force to employees in January 2018. We are selling JUXTAPID in Japan using a contract sales force. We are marketing and selling, or plan to market and sell, our products directly, using our own marketing and sales resources, in certain key countries outside the U.S. in which our products are, or may be, approved and where it makes business sense to do so. Following our workforce reduction in January 2018, which impacted our U.S. sales, market access and marketing teams, we will be undertaking these efforts with considerably less manpower, and this may impact our ability to effectively market and sell our products in the U.S. We use, and plan to continue to use, third parties to provide warehousing, shipping, third-party logistics, invoicing, collections and other distribution services on our behalf in the U.S. and in other countries throughout the world. For example, we currently have a contract with a single specialty pharmacy distributor in the U.S. for the distribution of lomitapide and metreleptin, a single distributor in Brazil for both products, and single distributors, importers and/or specialty pharmacies in certain other countries. We have entered into, or may selectively seek to establish, distribution and similar forms of arrangements to reach patients in certain geographies that we do not believe we can cost-effectively address with our own sales and marketing capabilities. If we are unable to establish and maintain the capabilities to sell, market and distribute our products, either through our own capabilities or through arrangements with third parties, and to effectively manage those third parties when we choose to use them, or if we are unable to enter into distribution agreements in those countries that we do not believe we can cost-effectively address with our own sales and marketing capabilities, we may not be able to successfully sell our products. We cannot guarantee that we will be able to establish and maintain our own capabilities or to enter into and maintain favorable distribution agreements with third-parties on acceptable terms, if at all.

To the extent that we enter into arrangements with third parties to perform sales, marketing or distribution services, our product revenues or the profitability of these product revenues to us are likely to be lower than if we were to commercialize our products ourselves. We will have limited control over such third parties, and any of them may fail to devote the necessary resources and attention to sell and market our products effectively, and may also, despite our compliance diligence reviews, audits and training, engage in non-compliant activities that, directly or indirectly, impact the use or sales of our products or damage our relationships with relevant stakeholders. Any performance failure, inability or refusal to perform on the part of our specialty pharmacy distributor in the U.S., our third-party sales force in Japan, our distributor in Brazil, or our third-party service providers in certain other countries, or any failure to renew existing agreements or enter into new agreements when these relationships expire on favorable terms, or at all, could cause serious disruption and impair our commercial or named patient sales of our products, which would have a negative impact on our revenues. Furthermore, our expenses associated with maintaining our sales force and distribution capabilities may continue to be substantial compared to the revenues we generate. If we are unable to establish and effectively maintain adequate sales, marketing and distribution capabilities, whether independently or with third parties, particularly as we continue to assess our cost structure in light of anticipated revenues, we may not be able to generate product revenues consistent with our expectations and may not become profitable or achieve cash-flow positive operations.

***Our success is dependent upon expanding regulatory approval for our products. The regulatory approval process is costly and lengthy, and we may not receive such regulatory approvals.***

We are currently permitted to market lomitapide in the U.S. and in a limited number of other countries on a commercial basis, and to market metreleptin in the U.S. Shionogi holds a marketing authorization for metreleptin in Japan under a distribution agreement assigned to us as part of the acquisition of the metreleptin assets. There is no assurance that we will be able to obtain marketing authorizations for either product in additional regions or countries or for the indications we may pursue, including MYALEPTA in the EU for which we are seeking approval of GL and a PL subgroup. To obtain marketing approvals, we must establish, and comply with, numerous and varying regulatory requirements regarding safety and efficacy and governing, among other things, clinical trials, pricing, promotion and distribution of the respective product, and we may be required to conduct post-marketing studies as a condition to such approvals, as we have been by the FDA for both JUXTAPID and MYALEPT and by the EMA for LOJUXTA. Approval procedures vary among countries, and can involve additional product testing and additional administrative review periods. Marketing approval in one country does not ensure such approval in another.

Regulatory authorities in countries where we seek approval for our products may not be satisfied with the design, size, endpoints or efficacy and safety results of the pivotal trial of the product, or the risk/benefit profile of the product, and may reject our applications for approval. For example, we filed to register JUXTAPID as a marketed product in Brazil, and, in May 2014, appealed a rejection of the registration by Brazil's National Health Surveillance Agency ("ANVISA"). We subsequently withdrew our appeal, and intend to resubmit our marketing application with additional data and information in 2018. In December 2017, a regulation was approved in Brazil providing for special procedures concerning the registration of new drugs for the treatment of rare diseases, which could expedite approval of our products in Brazil. The impact and applicability of this regulation is still unclear, and we may not be successful in receiving regulatory approval to market JUXTAPID in Brazil. It is also possible that regulatory authorities in countries where we are seeking, or may in the future seek, approval may disagree with our assessment that certain changes made to lomitapide's physical parameters and specifications as compared to the material used in the pivotal trial are not clinically meaningful. If regulatory authorities require additional studies or trials for either of our products or changes to specifications, we would incur increased costs and delays in the marketing approval process and may not be able to obtain approval.

In addition, regulatory authorities in countries outside the U.S. and EU are increasingly requiring risk management plans and post-marketing commitments, which may be more onerous than those required in the U.S. and EU. In certain countries, if the post-marketing commitment is a post-marketing study that would qualify as an interventional or similar form of study, we may be required to provide free product to participants in the study in such country even if our products are reimbursed there. The time required to obtain approval in other countries may differ from that required to obtain FDA approval or marketing authorization in the EU.

***The availability and extent of reimbursement from third-party payers is critical for the successful commercialization of lomitapide and metreleptin. We may face resistance from certain private, government and other third-party payers and from healthcare professionals and patients given the prices we charge for our products. If reimbursement for our products is limited or delayed, we may not be able to achieve our revenue goals or achieve profitability or achieve cash-flow positive operations from the lomitapide or metreleptin businesses in the time periods we expect, or at all.***

Market acceptance and sales of lomitapide and metreleptin in the U.S. will continue to depend, to a significant degree, on insurance coverage and reimbursement policies, and may be affected by healthcare reform measures. Government authorities and third-party payers, such as private health insurers and health maintenance organizations, decide for which medications they

will pay and establish reimbursement levels. A primary trend in the U.S. healthcare industry and elsewhere is cost containment and predictability. Government authorities and these third-party payers have attempted to control costs by limiting coverage and limiting the amount of reimbursement for particular medications. If we fail to successfully secure and maintain reimbursement coverage for our products at levels that are acceptable to us or are significantly delayed in doing so or if onerous conditions are imposed or announced by private payers, government authorities or other third-party payers on such reimbursement, we will have difficulty achieving or maintaining market acceptance of our products and our business and ability to achieve our financial expectations will be harmed.

Given that HoFH and GL are rare diseases with extremely small patient populations, we have established prices for JUXTAPID and MYALEPT in the U.S. that are significantly higher per dose on an annual basis than those of most commercially available pharmaceutical products. We expect to increase the price of lomitapide and metreleptin from time to time in the future. We believe pricing for our products in the U.S. is consistent with the level of pricing for other orphan drugs that treat diseases with comparable prevalence rates. Although a majority of payers in the U.S. are providing coverage for our products and, with respect to JUXTAPID, most payers in the U.S. have not required genotyping to determine a diagnosis of HoFH for reimbursement purposes, many of such payers have imposed requirements, conditions or limitations as conditions to coverage and reimbursement for JUXTAPID as a result of commercial availability of PCSK9 inhibitor products, which often include a requirement that HoFH patients have not achieved an adequate LDL-C response on PCSK9 inhibitor products before access to lomitapide is approved. For patients currently taking JUXTAPID, several U.S. pharmacy benefit managers (“PBMs”) are using a prior authorization requiring current JUXTAPID patients to “step through” the less expensive PCSK9 inhibitor product, and additional PBMs and payers may follow this practice. We have been engaging with PBMs to discuss and negotiate potential agreements to limit these so-called “step edits”, which, if successfully negotiated, may require us to provide discounts and other price protections and would impact our net revenues from JUXTAPID.

During the payer review process for MYALEPT in the U.S., some U.S. payers are requiring additional patient information, including details regarding a potential patient’s leptin level, which requires administration of a leptin level test, which may delay or otherwise impact reimbursement. The cost of JUXTAPID and MYALEPT in the U.S. may result in cost-sharing amounts for some patients that are prohibitive, and prevent these patients from being able to commence or continue therapy on JUXTAPID or MYALEPT, respectively. We provide support to eligible commercial patients for certain drug co-pays and co-insurance obligations for JUXTAPID and MYALEPT treatment. We currently do not plan to provide financial support to patient assistance programs operated by independent charitable 501(c)(3) organizations in the U.S. that are permitted, subject to compliance with strict legal and regulatory requirements, to assist eligible HoFH and GL patients, as determined solely by the organization, with certain co-payments or co-insurance requirements for their drug therapies, which may include lomitapide or metreleptin. If we do elect to provide such financial support, we would not have control or input into the decisions of these organizations. Our own co-pay assistance programs and any future contributions, if any, we make to any 501(c)(3) organization could result in significant costs to us.

The impact of evolving reimbursement policies and processes on the willingness of providers to furnish JUXTAPID or MYALEPT and the prices we can command for these products is difficult to predict and could have a negative impact on our revenues. Legislative changes to the Public Health Service Section 340B drug pricing program (the “340B Program”), the Medicaid Drug Rebate Program, and the Medicare Part D prescription drug benefit under the Medicare Prescription Drug, Improvement, and Modernization Act of 2003 (the “MMA”) could also impact our revenues. If reimbursement is not available, or available only to limited levels, or if the mix of patients for our products is more heavily weighted to patients reimbursed under government programs, we may not be able to generate sufficient revenues to meet our operating costs and continue our operations, and we may never be profitable or achieve positive cash flow in the timeframe that we expect, or at all. Price reductions and other discounts we offer or may offer for our products, and significant price increases typically result in increasing the rebates we are required to pay under the Medicaid Drug Rebate Program or state Medicaid supplemental rebate programs and the discounts we are required to offer under the 340B Program. For example, we currently pay a significant rebate for MYALEPT that could offset the majority of revenues from Medicaid patients, and this rebate will have a continuing significant impact in future periods. The degree of such impact on our overall financial performance will depend on the percentage of MYALEPT patients who have Medicaid as their primary insurance coverage and the quantity of units ordered per patient. In addition, a considerable number of JUXTAPID patients in the U.S. are Medicare Part D patients. A significant percentage of such patients may not be able to afford their out-of-pocket co-payments, which could result in such patients seeking an alternative free or lower-cost drug, or ceasing treatment with our product, given that the only source of financial support for such patients may be through independent 501(c)(3) patient organizations, which may not provide adequate financial assistance, including due to reductions in contributions to such patient organizations. Any negative developments with regard to reimbursement of our products, for any reason, could have a material adverse effect on our revenues or financial condition.

Further, any negotiated prices for our products covered by a Part D prescription drug plan will likely be lower than the prices we might otherwise obtain and, while the MMA applies only to drug benefits for Medicare beneficiaries, private payers

often follow Medicare coverage policy and payment limitations in setting their own payment rates. Any reduction in payment that results from the MMA may result in a similar reduction in payments from non-governmental payers. In addition, in the U.S., the cost of pharmaceuticals continues to generate substantial governmental and third-party payer scrutiny. We expect that the pharmaceutical industry will continue to experience pricing pressures due to the increasing influence of managed care (and related implementation of managed care strategies to control utilization) organizations, additional federal and state legislative and regulatory proposals to regulate pricing of drugs, limit coverage of drugs or reduce reimbursement for drugs, public scrutiny, and the current presidential administration's agenda to control the price of pharmaceuticals through government negotiations of drug prices in Medicare Part D and importation of less expensive products from abroad. While we cannot predict what executive, legislative and regulatory proposals will be adopted or other actions will occur, or what the impact on our products might be, such events and impact could have a material adverse effect on our business, financial condition and results of operations.

In countries outside the U.S. where lomitapide is or may be approved, or where metreleptin may be approved, including countries in the EU, we are also seeking or expect to seek a price that is significantly higher than that of most commercially available pharmaceutical products, and which reflects the rare nature of the diseases our products treat. There is no assurance that government agencies in such countries that are responsible for reimbursement of healthcare costs, or other third-party payers in such countries, will agree to provide sufficient or any coverage for our products at the prices we expect to propose. In many countries outside the U.S., including key markets in the EU, if approved by the relevant regulatory authority, a product must also receive pricing and reimbursement approval before it can be commercialized broadly or at all. This approval requirement, and the lengthy pricing and/or reimbursement negotiations with governmental authorities that may occur due to such requirement, can result in substantial delays in commercializing products in such countries, and the price that is ultimately approved may be lower than the price for which we expect to offer, or would be willing to offer, our products in such countries, and may impact pricing in other countries. Pricing and reimbursement approval in one country does not ensure such approvals in another. Failure to obtain the approvals necessary to commercialize our products in other countries after receipt of regulatory approval at reimbursement levels that are acceptable to us, or any delay or setback in obtaining such approvals, would impair our ability to develop foreign markets for our products. For example, Aegerion experienced significant delays and challenges obtaining pricing and reimbursement approval for LOJUXTA in several countries of the EU, including in France, where LOJUXTA twice received a "minor improvement" rating, which significantly limited the potential reimbursement level for lomitapide in France; in Germany, where LOJUXTA twice received a "no additional benefit" assessment from the German reimbursement authority; and in Spain, where the price of LOJUXTA was rejected by the Spanish Ministry of Health. We may experience similar delays and challenges in connection with seeking pricing and reimbursement approval for metreleptin in the EU, assuming approval by the EMA, even though metreleptin has been granted orphan designation by the EC for the indications that we are pursuing, which designation lomitapide was not granted. In part because Aegerion was unable to obtain commercially acceptable pricing and reimbursement approvals for LOJUXTA in several of the key markets of the EU, Aegerion elected to cease commercialization of LOJUXTA in the EU and, in December 2016, entered into a license agreement with Amryt Pharma plc ("Amryt") under which Amryt was granted an exclusive license to develop and commercialize LOJUXTA in the European Economic Area ("EEA"), Switzerland, Turkey, and certain Middle Eastern and North African countries, including Israel. The requirements governing drug pricing vary widely from country to country. We expect that some countries will seek, and in certain cases, have sought and received, price and patient number and other price restrictions for our products, or, in some cases, if sold on a named patient basis. To obtain reimbursement or pricing approval in some countries, we may be required to conduct a clinical trial that compares the cost-effectiveness of our product, or any product candidate, to other available therapies, which may not be possible for us to do.

Outside the U.S., the macroeconomic climate, or local regulations or practices, may adversely affect our ability to set and charge a price sufficient to generate adequate revenues in those markets. The price of lomitapide, or metreleptin if approved, in one country may adversely affect the price in other countries. This is particularly true among countries in the EU. We may elect not to launch our products in any country where it does not make commercial sense to do so given the approved price or other conditions. In addition, while we do not expect to obtain approval of our products outside of rare disease indications, in the future if we were to obtain such approval for new indications with a higher prevalence rate than our existing indications, it may be more difficult for us to obtain or maintain our current price levels and targets for lomitapide and metreleptin. For example, due to the broader indication for MYALEPT in Japan, MYALEPT is sold by Shionogi in Japan at a price significantly lower than the U.S. price. Even if we are successful in obtaining pricing and reimbursement approval for our products in a particular country, such country may impose onerous conditions on reimbursement, which may, for example, include genotyping or the use of other therapies, such as apheresis, prior to the prescription and reimbursement of lomitapide, and include leptin testing or other requirements prior to the prescription and reimbursement of metreleptin.

We may not be able to provide all of the data required to obtain pricing/reimbursement approvals in certain countries outside the U.S. where we seek to commercialize our products, if approved, or we may not satisfactorily meet the technical or substantive requirements of such submissions or receive ratings from pricing or other regulatory authorities commensurate with our expectations or that would support the price levels we want for our products, which could result in delays of pricing/reimbursement approvals for our products, our products not obtaining pricing/reimbursement approval at all, or our products



obtaining approvals at less than acceptable levels or with significant restrictions on use or reimbursement. For example, Health Technology Assessments ("HTA") have become an increasingly common part of the pricing and reimbursement procedures in some EU Member States, including the United Kingdom, France, Germany, Ireland, Italy and Sweden. HTA is the procedure according to which the assessment of the public health impact, therapeutic impact, and the economic and societal impact of use of a given medicinal product in the national healthcare systems of the individual country is conducted. HTA generally focuses on the clinical efficacy and effectiveness, safety, cost, and cost-effectiveness of individual medicinal products as well as their potential implications for the healthcare system. Those elements of medicinal products are compared with other treatment options available on the market. The data available to us from the phase 3 study of metreleptin conducted at the NIH may not be ideal to support HTAs and the market access process in the EU, due to, among other things, the study having been single-arm, non-comparative and not having produced outcome data. Due to our current budgetary constraints, we may not be able to fund additional analyses of the NIH data or supplemental studies that could support reimbursement applications in the EU. This may result in metreleptin not obtaining pricing/reimbursement approval at all, or at less than acceptable levels or with significant restrictions on use or reimbursement. Further, the outcome of HTA regarding specific medicinal products will often influence the pricing and reimbursement status granted to these medicinal products by the regulatory authorities of individual EU Member States. The extent to which pricing and reimbursement decisions are influenced by the HTA of the specific medicinal product vary between EU Member States. A negative HTA of metreleptin by a leading and recognized HTA body, such as the National Institute for Health and Care Excellence ("NICE") in the United Kingdom, could not only undermine our ability to obtain reimbursement for such product in the EU Member State in which such negative assessment was issued, but also in other EU Member States. For example, EU Member States that have not yet developed HTA mechanisms could rely to some extent on the HTA performed in countries with a developed HTA framework, such as the United Kingdom, when adopting decisions concerning the pricing and reimbursement of a specific medicinal product.

We may also face pricing and reimbursement pressure in the U.S. and other countries as a result of prices charged for competitive products or therapies. In addition, in certain countries, such as Brazil, the price we are able to charge for named patient sales prior to approval may be higher than the price that is approved by governmental authorities after approval.

***We rely on named patient sales of our products in certain territories, but there is no assurance that named patient sales of our products will continue at current levels, or at all.***

In Brazil, Turkey, and in a limited number of other countries where permitted based on U.S. or EU approval, lomitapide and metreleptin are available on a named patient sales or similar basis. There is no assurance that named patient sales will continue to be authorized in any particular country. Even if they are authorized, we are not permitted to promote, market or otherwise engage in proactive selling activities for products sold on a named patient basis, which makes named patient sales much less predictable, and susceptible to unexpected decreases. If violations of any laws or governmental regulations are found to have occurred in connection with our products in connection with named patient sales, significant criminal or civil lawsuits may be filed, or investigations may be commenced. For example, in Brazil, under certain circumstances, we could be barred from further named patient sales of our products to federal and/or state governments in Brazil due to penalties imposed by Brazilian regulatory authorities or through civil actions initiated by federal or state public prosecutors, or we could face administrative penalties imposed by Brazilian regulatory authorities and additional damages and fines. We believe the investigations in Brazil have contributed to a slower turn-around between price quotation and orders, including re-orders, from the federal government, and, in some cases, delays in orders and re-orders from the government of the State of São Paulo after a patient has obtained access to JUXTAPID through the judicial process. These delays may continue, and we may experience other delays or suspensions of the ordering process. Similarly, there has been, and may continue to be, some reluctance by physicians to prescribe JUXTAPID, and some patients to take or stay on JUXTAPID, while the investigations are ongoing, particularly given that some of the investigators in Brazil made formal inquiries of certain prescribers of JUXTAPID, and there has been significant local media coverage of such inquiries and our activities in Brazil, including in 2017. For example, in the second quarter of 2015 and in the second quarter of 2016, we observed a significant increase in patients discontinuing therapy in Brazil, and we believe that the increases in discontinuations during those times were due in part to the investigations. In addition, the Brazilian Supreme Federal Court is currently discussing, in two ongoing lawsuits, whether the government has an obligation to continue to provide, on a named patient sales basis, drugs that have not received regulatory and/or pricing and reimbursement approval in Brazil, such as lomitapide and metreleptin. Further, in October 2017, a new set of regulatory requirements governing use of product candidates was published in Brazil which has added complexity to the process for the purchase, on a named patient basis, of drugs which have not received regulatory and/or pricing and reimbursement approval in Brazil, such as lomitapide and metreleptin, which has, along with the ongoing court proceeding, resulted in delays in the receipt of orders from Brazil for existing lomitapide and metreleptin patients and we believe has led certain patients to discontinue therapy with lomitapide and metreleptin. Although we intend to file for marketing approval in Brazil for both JUXTAPID and MYALEPT in 2018, the approval process can be lengthy, and there is no guarantee that we will be able to obtain such approval; as a result, we may have to rely on our ability to generate named patient sales for a considerable amount of time, or indefinitely. The result of the lawsuits, the recent requirements regarding named patient

sales in Brazil, and other issues could significantly negatively affect product revenues from named patient sales of our products in Brazil.

We do not yet know the full extent of the impact that the approval of PCSK9 inhibitor products in the U.S., or the approval of a PCSK9 inhibitor product in Brazil in 2016, will have on the named patient sales of lomitapide in Brazil or other countries. We also do not know whether we will be permitted to sell lomitapide or metreleptin on a named patient basis in any additional countries. In certain countries, we may decide not to pursue named patient sales even if permitted. Even if named patient sales (or equivalent sales) are permitted in a certain country, and we elect to make lomitapide or metreleptin available on such basis, there is no guarantee that physicians in such country will prescribe the product, which they can only do if they proactively reach out to us or our distributors and also undertake the effort, time and cost of following the stringent local requirements to get their patient on therapy on a named patient basis, and that patients will be willing to start and adhere to therapy, or that the country will pay for the product at all, or at a level that is acceptable to us, without delay or imposing other hurdles on payment. These risks may be heightened in Brazil, in light of the 2016 approval of a PCSK9 inhibitor product in Brazil, ongoing state and federal government investigations, a 2016 decision by the Brazilian pharmaceutical industry association that we violated its Code of Conduct, and negative coverage by Brazilian media. There is no guarantee that we will generate sales or substantial revenues from such sales.

If named patient sales do not meet our expectations in key named patient sales markets, particularly Brazil, we may not be able to meet our expectations with respect to sales of our products or revenues from such sales, achieve cash-flow positive or profitable operations, or meet our expectations with respect to profitability in the time periods we anticipate, or at all. Further, there are countries where we choose to make our products available under an expanded access program at no cost prior to approval in such countries. There is no assurance that we will be able to obtain marketing approval or reimbursement at all or at acceptable levels or to maintain reimbursement for our products in any country where we have expanded access programs or that patients on such programs will convert to commercial product even if we do obtain requisite approvals. In certain countries where we seek reimbursement for the product during the pre-approval phase, we are able to establish the price for the product, while in other countries we need to negotiate the price. Such negotiations may not result in a price acceptable to us, in which case we may elect not to distribute our products in such country prior to approval or we may curtail distribution. Our expanded access program may result in significant expenses and may not result in expected future sales at desired levels or at all, and could negatively impact our financial results.

***We are subject to intense competition. If we are unable to compete effectively, we may not be able to achieve our revenue goals or achieve profitability or cash-flow positive operations. Competitive products could render lomitapide or metreleptin, or any other product or product candidate that we develop or acquire, non-competitive or obsolete, which would have a materially negative impact on our results of operations.***

Our industry is highly competitive and subject to rapid and significant technological change. Our potential competitors include large pharmaceutical and biotechnology companies, specialty pharmaceutical and generic drug companies, academic institutions, government agencies and research institutions. All of these competitors currently engage in, have engaged in or may in the future engage in the development, manufacturing, marketing and commercialization of new pharmaceuticals, some of which may compete with lomitapide or metreleptin, or other products or any product candidates we may acquire, license or develop. Smaller or early stage companies may also be significant competitors, particularly through collaborative arrangements with large, established companies. Key competitive factors affecting the commercial success of lomitapide, metreleptin and any other products that we develop or acquire are likely to be safety, efficacy, tolerability profile, reliability, convenience of dosing, price and reimbursement.

The market for cholesterol-lowering therapeutics is large and competitive with many drug classes. Lomitapide is approved in the U.S., Japan, the EU and in certain other countries as an adjunct to a low-fat diet and other lipid-lowering treatments to reduce LDL-C in adult HoFH patients. As a treatment for HoFH, JUXTAPID competes in the U.S. and certain other countries with Kynamro. Developed by Ionis Pharmaceuticals, Inc. (“Ionis”) and now marketed by Kastle Therapeutics, Kynamro is an antisense apolipoprotein B-100 inhibitor which is taken as a weekly subcutaneous injection. JUXTAPID also faces significant competition in the treatment of adult HoFH patients with a class of drugs known as PCSK9 inhibitors. In July 2015, the FDA approved the BLA for Regeneron Pharmaceuticals, Inc. and Sanofi’s PCSK9 inhibitor candidate, alirocumab, for use in addition to diet and maximally tolerated statin therapy in adult HeFH patients and in patients with clinical atherosclerotic cardiovascular disease who require additional lowering of LDL-C. In September 2015, the EC approved alirocumab for the treatment of adult patients with HeFH or mixed dyslipidemia as an adjunct to diet, either in combination with a statin, or statin with other lipid-lowering therapies in patients unable to reach their LDL-C goals with the maximally-tolerated statin, or alone or in combination with other lipid-lowering therapies for patients who are statin intolerant, or for whom a statin is contraindicated. The FDA approved Amgen Inc.’s BLA for its anti-PCSK9 antibody, evolocumab, in August 2015, as an adjunct to diet and maximally tolerated statin therapy for the treatment of adults with HeFH or clinical atherosclerotic cardiovascular disease, who require additional lowering of LDL-C;

and as an adjunct to diet and other LDL-lowering therapies for the treatment of patients with HoFH, who require additional lowering of LDL-C. In July 2015, the EC approved the marketing authorization of evolocumab for the same indication as alirocumab, and for the treatment for certain patients with high cholesterol, including patients aged 12 years and over with HoFH in combination with other lipid-lowering therapies. In January 2016, the MHLW in Japan approved evolocumab for the treatment of patients with FH or hypercholesterolemia who have high risk of cardiovascular events and do not adequately respond to statins, and in July 2016 the MHLW approved alirocumab for the same indication. The regulatory agencies responsible for reviewing marketing authorization applications in Brazil, Canada, Colombia, and Argentina have also approved evolocumab for the treatment of patients with HoFH. Other companies, including Eli Lilly & Co. and Amlylam Pharmaceuticals, Inc., in collaboration with The Medicines Company, are also developing PCSK9 inhibitor products.

The introduction of PCSK9 inhibitors in the U.S. and in other key markets has already significantly and negatively impacted sales of JXTAPID and we expect continued pressure on sales of JXTAPID. This impact on JXTAPID sales results from several factors, including: healthcare professionals switching some existing JXTAPID patients to a PCSK9 inhibitor product, and healthcare professionals trying most new adult HoFH patients on a PCSK9 inhibitor product before trying JXTAPID because such products, in comparison to JXTAPID, have fewer side effects, are significantly less expensive, and do not require patients to follow a special low-fat diet; requirements imposed by insurance companies, managed care organizations and other private payers in the U.S. that HoFH patients demonstrate an inability to achieve an adequate LDL-C response on PCSK9 inhibitor products before access to JXTAPID is approved; and the likelihood that prior authorization (which we believe is required by all U.S. payers) will encourage a switch of current JXTAPID patients to a less expensive PCSK9 inhibitor product. Competitors for JXTAPID may enjoy other competitive advantages if insurance companies, managed care organizations or other private payers in the U.S. impose other hurdles to access or other significant restrictions or limitations on reimbursement, or require switching of JXTAPID patients to PCSK9 inhibitor products. We believe that many of the PCSK9 inhibitor switches from JXTAPID patients have been at the direction of the prescribing physician, and physicians may ultimately not decide to switch the adult HoFH patient back to JXTAPID even if the patient does not reach a goal of LDL-C response while being treated with a PCSK9 inhibitor product, or otherwise prescribe JXTAPID to patients. It is unknown how many adult HoFH patients, if any, may be switched back to or started on JXTAPID, or the period of time in which this might take place.

The availability of PCSK9 inhibitor products in commercial markets outside of the U.S. is having similar negative effects on sales, including named patient sales, of lomitapide outside the U.S., particularly in Brazil, Japan, Canada and Colombia, where PCSK9 inhibitor products have been approved by the regulatory authorities and launched commercially. If the continued negative impact of PCSK9 inhibitors is greater than we expect, it may make it more difficult for us to generate revenues and achieve profitability. Also, although there are no other microsomal triglyceride transfer protein inhibitor (“MTP-I”) compounds currently approved by the FDA for the treatment of hyperlipidemia in humans, there may be other MTP-I compounds in development.

Potential future competition for JXTAPID may include drugs and biologics currently in clinical development for the treatment of patients with HoFH. Development programs currently include other oral drug therapeutics, such as Gemphire Therapeutics’ gemcabene which targets apolipoprotein C-III and is currently preparing to enter Phase 3 development for the treatment of FH. Other potential therapies include gene therapies, such as REGENXBIO’s LDL-receptor gene therapy which is currently in Phase 1/2 development for HoFH. Also, although there are no other MTP-I compounds currently approved by the FDA for the treatment of hyperlipidemia in humans, there may be other MTP-I compounds in development. If approved, novel drug or biologic therapies for the treatment of HoFH or FH could negatively impact the sales of JXTAPID in the future.

Although MYALEPT is the first and only product approved in the U.S. for the treatment of complications of leptin deficiency in patients with GL, there are a number of therapies approved to treat these complications independently that are not specific to GL. Certain clinical complications of GL, including diabetes and hypertriglyceridemia, may be treated with insulin and/or oral medications, such as metformin, insulin secretagogues, fibrates, or statins, but patients with GL often have an inadequate response to these therapies. Akcea Therapeutics, is developing volanesorsen, an antisense therapy targeting apolipoprotein C-III, which is currently in Phase 3 development for patients with familial PL with diagnosed type 2 diabetes mellitus or hypertriglyceridemia, which, if approved and depending on the labeled indication, could potentially compete with metreleptin, if metreleptin is approved for the treatment of a PL subset.

We may also face future competition from companies selling generic alternatives of our products in countries where we do not have patent coverage, orphan drug status or another form of data or marketing exclusivity or where patent coverage or data or marketing exclusivity has expired, is not enforced, or may, in the future, be challenged.

Many of our current and potential competitors have substantially greater financial, technical and human resources than we do, which is exacerbated by several factors related to our business, including the negative impact of PCSK9 inhibitor products on the JXTAPID business, the financial penalties and compliance terms in the settlement agreements entered into in connection with the DOJ and SEC investigations, Aegerion’s outstanding debt obligations, and the Company’s consolidated financial condition

and cash position. Many of these companies also have significantly greater experience in the discovery and development of drug candidates, obtaining FDA and other regulatory approvals of products and the commercialization of those products. Accordingly, our competitors may be more successful than us in obtaining marketing approvals for drugs and achieving and maintaining widespread market acceptance.

Our competitors' drugs may be more effective, or more effectively marketed and sold, than any drug we may commercialize, and may render lomitapide or metreleptin, or any other product or any product candidate that we acquire, license or develop, obsolete or non-competitive before we can recover the expenses of developing and commercializing the product. We anticipate that we will face intense and increasing competition as new drugs enter the market and advanced technologies become available. Finally, the development of new treatment methods for the diseases we are targeting could render lomitapide or metreleptin, or any other product or any product candidate that we acquire, license or develop, non-competitive or obsolete, which would have a materially negative impact on our results of operations.

***We depend entirely on third-party manufacturers to produce our drug substance and drug product for each of our products, and diluent for metreleptin. If they are unable to comply with applicable regulatory requirements, unable to source sufficient raw materials, experience manufacturing or distribution difficulties, or are otherwise unable to manufacture and distribute sufficient quantities to meet demand, our commercialization efforts may be materially harmed. Having a single source manufacturer may increase the risk that we will not have sufficient quantities of our products or diluent for metreleptin, or will not be able to obtain such quantities at an acceptable cost, or at all, which could negatively impact commercialization of our products or delay, prevent or impair our clinical development programs.***

We have no internal manufacturing facilities and employ limited personnel with manufacturing experience. We are completely dependent upon contract manufacturers to produce drug substance and drug product for commercial supplies and for our clinical trials.

We have long-term supply agreements with our lomitapide drug product and drug substance manufacturers, as well as supply agreements with our metreleptin drug substance and drug product manufacturers. We do not have agreements in place for redundant supply or a second source for drug substance or drug product for either of our products. Any termination or non-renewal of our agreements with our contract manufacturers, or any failure of our suppliers to perform under such contracts, could impact availability of commercial supply of lomitapide or metreleptin, which would have an adverse impact on sales, or supply for use in clinic trials, which may delay further clinical development or marketing approval of such product in additional countries. If for any reason our contract manufacturers cannot or will not perform as agreed, or if they terminate or fail to renew our arrangement, we may be required to replace such manufacturer, which would take a considerable amount of time and effort, and we may not be able to replace such manufacturer on as favorable terms, or at all. For example, in December 2017, the contract manufacturer for metreleptin drug product informed Aegerion that it will terminate its supply contract in December 2019. Although Aegerion has a supply of safety stock and has a mitigation plan in place with another contract manufacturer for metreleptin drug product (where technology transfer has already been completed), there is no assurance that the new metreleptin manufacturer will be fully validated and that all necessary approvals will be received for such manufacturer prior to exhausting our existing supply and reserves manufactured in the interim of metreleptin drug product. Any interruption in supply of metreleptin drug product could have a material and adverse impact on our sales, depending upon the length of interruption. In part to prepare for launch of metreleptin in the EU, assuming approval of our MAA, we are in the process of developing new presentations in nominal 2.5 mg and 5 mg vials. In order to provide metreleptin in these new presentations in the EU, assuming we obtain approval of our MAA, we must, after approval, file variations with the EMA establishing that these new presentations are comparable to the current 10 mg vial presentation. If our data fail to establish comparability, the EMA may not approve the new presentations, or approve them with a limited shelf life, or we may not meet the deadline to file our pricing and reimbursement dossiers in certain key markets, or file them on the basis of the current presentation only, which could, given that metreleptin requires daily dosing and only the 10 mg vial presentation would be available, impact our ability to secure an approved price on a timely basis or at all for metreleptin in those key markets, and ultimately impact our revenue goals.

In addition, our metreleptin drug product manufacturer also supplies bacteriostatic water for injection ("BWFI"), one of the approved diluents for reconstitution of metreleptin which allows for use of a reconstituted vial of metreleptin for up to three days when stored appropriately. BWFI is only available for sale and approved for use in the U.S., and we or our contract manufacturers purchase it for supply with ex-US named patient sales and other expanded access distribution to reduce the number of metreleptin vials provided. Although BWFI is currently in shortage and on back order from our contract manufacturer, we have secured sufficient inventory to maintain a supply through late 2018 based on current demand. If in the future we exhaust our inventory of BWFI, ex-U.S. patients who obtain metreleptin via named patient sales or an expanded access program will need to use water for injection, a diluent which requires immediate use of product after reconstitution, and the discarding of any product remaining in the vial, which will likely result in more metreleptin vials being shipped and could decrease our revenues from named patient sales, increase our expenses, and impact our inventory of metreleptin and our agreements with our distributors.

If we are unable to maintain arrangements for third-party manufacturing, or are unable to do so on commercially reasonable terms, or are unable to obtain timely regulatory approvals in connection with our contract manufacturers, we may not be able to successfully commercialize our products or complete development of our products. We may incur significant added costs and substantial delays in identifying and qualifying any replacement manufacturers, and in obtaining regulatory approval to use such replacement manufacturer in the manufacture of our products. Any such delays could result in significant delay in the supply of drug product for an ongoing trial due to the need to replace a third-party manufacturer and could delay completion of the trial. If for any reason we are unable to obtain adequate supplies of lomitapide or metreleptin, or any other product or any product candidate that we develop or acquire, or the drug substances used to manufacture them, it will be more difficult or impossible for us to compete effectively, generate revenues, meet our expectations for financial performance and further develop our products. In addition, if we are unable to assure a sufficient quantity of the drug for patients with rare diseases or conditions, we may lose any orphan drug exclusivity to which the product otherwise would be entitled.

We rely on our contract manufacturers to utilize processes that consistently produce drug substance and drug product to their required specifications, including those imposed by the FDA, the EMA and other regulatory authorities, as applicable. There can be no assurance that our contractors will consistently be able to produce commercial supplies of drug substance or drug product meeting the approved specifications. A number of factors could cause production interruptions at the facilities of our contract manufacturers, including equipment malfunctions, facility contamination, labor problems, raw material shortages or contamination, natural disasters, disruption in utility services, terrorist activities, regulatory actions resulting from failure to comply with current Good Manufacturing Practice ("cGMP"), human error or disruptions in the operations of our suppliers. We have experienced failures by our third-party manufacturers to produce product that meets our specifications in the past, and any future failure by our third-party manufacturers to produce product that meets specifications could lead to a shortage of lomitapide or metreleptin.

The manufacture of biologic pharmaceuticals, such as metreleptin, is more difficult and riskier than the manufacture of small molecule pharmaceuticals, such as lomitapide. The process of manufacturing biologics is highly susceptible to product loss due to contamination, oxidation, equipment failure or improper installation or operation of equipment, or vendor or operator error. Even minor deviations from normal manufacturing processes could result in reduced production yields, product defects and other supply disruptions. If microbial, viral or other contaminations are discovered in metreleptin or the facilities of our contract manufacturer, we may need to cease the manufacture of metreleptin for an extended period of time to investigate and remediate the contaminant. A contamination, recall, raw material shortage, or other supply disruption could adversely impact or disrupt commercial manufacturing of metreleptin or could result in a withdrawal of metreleptin from the market. Any such disruption, in turn, could adversely affect our ability to satisfy demand for metreleptin, which could materially and adversely affect our operating results and expectations for financial performance.

The FDA, the EMA and other regulatory authorities require that drug products be manufactured according to cGMP. Any failure by our third-party manufacturers to comply with cGMP could lead to a shortage of our products. In addition, such failure could be the basis for action by the FDA, the EMA or regulatory authorities in other territories or countries to withdraw approvals previously granted to us and for other regulatory action, including seizure, injunction or other civil or criminal penalties. For example, in February 2017, the original contract manufacturer for metreleptin drug product received a Warning Letter from the FDA citing significant violations of cGMP regulations at the manufacturing facility where metreleptin drug product is manufactured. In response, the manufacturer may make modifications to the line on which metreleptin drug product is filled. We believe that we have sufficient inventory of drug substance for each of our products to maintain a supply for more than one year. The failure of any of our third-party manufacturers to address any concerns raised by the FDA or foreign regulators could lead to plant shutdown or the delay or withholding of product approval by the FDA in additional indications, or by foreign regulators in any indication, including, with respect to metreleptin, the MAA in the EMA for GL and the PL subset. Certain countries may impose additional requirements on the manufacturing of drug products or drug substance, and on our third-party manufacturers, as part of the regulatory approval process for our products in such countries. The failure by us or our third-party manufacturers to satisfy such requirements could impact our ability to obtain or maintain approval of our products in such countries.

***The FDA, the EU Member States and other regulatory agencies outside the U.S. and the EU enforce laws and regulations prohibiting the promotion of off-label uses. Violations of these laws and regulations, and the resulting enforcement actions by these agencies, can result in significant liability.***

The FDA, the regulatory authorities of the EU Member States and other regulatory agencies outside the U.S. and the EU strictly regulate the promotional claims that may be made about prescription drug products. In particular, a drug product may not be promoted in a jurisdiction prior to approval or for uses that are not approved by the FDA, the EC, the regulatory authorities of the EU Member States or such other regulatory agencies, as applicable, as reflected in the product's approved prescribing information or summary of product characteristics. In the U.S., promotion of products for unapproved (or off-label) uses may result in enforcement letters, inquiries and investigations, and civil and criminal sanctions by the FDA. Promotion of products for

off-label uses in the U.S. can also result in false claims litigation under federal and state statutes, which can lead to consent decrees, civil monetary penalties, restitution, criminal fines and imprisonment, and exclusion from participation in Medicare, Medicaid and other federal and state healthcare programs. Aegerion has been the subject of such investigations and enforcement in connection with the JUXTAPID Investigations and the related Settlement. Cooperation with the JUXTAPID Investigations and negotiation of and compliance with the Settlement has been and will continue to be costly and burdensome on our management and other resources, and our business has suffered reputational harm.

In addition, Novelon or Aegerion may see new governmental investigations of or actions against it citing additional theories of recovery, or may encounter difficulties and expend considerable further resources in complying with the Settlement. The JUXTAPID Investigations and the Settlement have resulted in third party demands and may in the future give rise to third party demands, claims or litigation, including demands or claims by, or litigation with, third party payers, healthcare providers, patients or investors, for matters related to the subject matter of or disclosure in connection with such Investigations or the Settlement. For example, Aegerion has received two demand letters, one from an insurance company, seeking reimbursement for the JUXTAPID claims it paid based on alleged false misrepresentations made by Aegerion, and the second from an investor in Aegerion's Convertible Notes alleging that it purchased the notes based on misrepresentations and omissions. The JUXTAPID Investigations and the Settlements could also lead to potential investigations, claims or litigation by consumer protection agencies or groups (including State Attorneys General consumer protection units), or provide a basis for product liability claims or litigation. Such claims or investigations, regardless of the merits, can be costly to resolve, and may lead to lengthy litigation and settlement negotiations, the results of which could require us to pay significant amounts of damages. Such third party claims often unfold in a public manner, and any public claims can encourage further claims from additional third parties. See the "Legal Proceedings" section of this Annual Report for further information regarding the JUXTAPID Investigations, including the Settlement, and other ongoing investigations and legal proceedings.

The ongoing investigations and litigation involving Aegerion, including the JUXTAPID Investigations and related third party claims, and future investigations or litigation in which we are involved could have a material adverse effect on our business, financial condition, results of operations, and share price, and divert the attention of our management from operating our business and may be disruptive to our employees, possibly resulting in further employee attrition. In addition, the existence of the investigations, including the JUXTAPID Investigations, and related activities, have impacted, and may continue to impact, the willingness of some physicians to prescribe JUXTAPID and/or MYALEPT.

***Our relationships with customers and payers in the U.S. are subject to applicable anti-kickback, fraud and abuse and other healthcare laws and regulations, violations of which could expose us to criminal sanctions, civil penalties, contractual damages, and reputational harm and could diminish future earnings and prevent us from achieving our expected financial results.***

Our arrangements with third-party payers and customers in the U.S. expose us to broadly applicable fraud and abuse and other healthcare laws and regulations, including the federal healthcare Anti-Kickback Statute, the False Claims Act, the Health Insurance Portability and Accountability Act of 1996 ("HIPAA") and the Physician Payment Sunshine Act, and analogous state and foreign laws and regulations that may regulate the business or financial arrangements and relationships through which we market, sell and distribute our products, as discussed above under the heading "Promotional Activities and Interactions with Healthcare Providers and Patients."

We are also subject to laws and regulations covering data privacy and the protection of health-related and other personal information. The legislative and regulatory landscape for privacy and data protection continues to evolve, and there has been an increasing focus on privacy and data protection issues which may affect our business, including recently enacted laws in many jurisdictions where we operate.

Failure to comply with these regulatory frameworks, including healthcare laws and laws and regulations covering data privacy and the protection of health-related and other personal information, could result in government enforcement actions, which could include civil or criminal penalties, private litigation and/or adverse publicity and could negatively affect our operating results and business. In addition, we obtain patient health information from most healthcare providers who prescribe our products and research institutions with which we collaborate, and they are subject to privacy and security requirements under HIPAA. Although we are not directly subject to HIPAA other than with respect to providing certain employee benefits, we could potentially be subject to criminal penalties if we knowingly obtain or disclose individually identifiable health information maintained by a HIPAA-covered entity in a manner that is not authorized or permitted by HIPAA.

In addition to the JUXTAPID Investigations, we or our subsidiaries, including Aegerion, could become subject to other government investigations and related subpoenas. Subpoenas are often associated with previously filed qui tam actions, or lawsuits filed under seal under the federal civil False Claims Act. Qui tam actions are brought by private plaintiffs suing on behalf of the federal government for alleged federal civil False Claims Act violations. The time and expense associated with responding to

subpoenas, and any related qui tam or other actions, may be extensive, and we cannot predict the results of our review of the responsive documents and underlying facts or the results of such actions. Any investigation, including the investigations described in detail in the “ *Legal Proceedings* ” section of this Annual Report, could result in civil and/or criminal sanctions being levied against us or Aegerion, including significant fines, sanctions, and other negative consequences that will have a material adverse effect on our business, financial condition, results of operations and/or cash flows. Aegerion’s Settlement has resulted in material fines, sanctions and other remedies against Aegerion. Responding to subpoenas and investigations in general is costly and time-consuming. Moreover, responding to any additional government investigations, defending any claims raised, and any resulting fines, restitution, damages and penalties, settlement payments and administrative actions, as well as any related actions brought by shareholders or other third parties, could have further material adverse impacts beyond those attributable to the JUXTAPID Investigations and the Settlement, including on our reputation, our business, financial condition, results of operations, and share price. These investigations have diverted, and may continue to divert, the attention of our management from operating our business, and have been disruptive, and may continue to be disruptive, to our employees, possibly resulting in employee attrition. In addition, the existence of the investigations described in detail in the “ *Legal Proceedings* ” section of this Annual Report, and related activities, have impacted, and may continue to impact, the willingness of some physicians to prescribe JUXTAPID and/or MYALEPT.

The number and complexity of both federal and state laws continue to increase, and additional governmental resources are being used to enforce these laws and to prosecute companies and individuals who are believed to be violating them. In particular, the Healthcare Reform Act includes a number of provisions aimed at strengthening the government’s ability to pursue anti-kickback and false claims cases against pharmaceutical manufacturers and other healthcare entities, including substantially increased funding for healthcare fraud enforcement activities, enhanced investigative powers, and amendments to the False Claims Act that make it easier for the government and whistleblowers to pursue alleged violations of the Anti-Kickback Statute, the Food, Drug and Cosmetics Act (the “FDCA”), the False Claims Act and other relevant laws. While the evolving nature of this regulatory framework makes it difficult to predict what effect the framework and any recent or future changes will have on our business, we anticipate that government scrutiny of pharmaceutical sales and marketing practices will continue for the foreseeable future and subject us to the risk of government investigations and enforcement actions. For example, federal enforcement agencies recently have shown interest in, and engaged in enforcement actions against, pharmaceutical companies’ product and patient assistance programs, including manufacturer reimbursement support services and relationships with specialty pharmacies, as well as contributions by companies to third-party 501(c)(3) charitable organizations that assist patients in accessing treatment for certain diseases and conditions. This was also a part of the JUXTAPID Investigations, including certain contributions that were not resolved in the Settlement. Some of these investigations have resulted in significant civil and criminal settlements. Responding to a government investigation or enforcement action would be expensive and time-consuming, and could have a material adverse effect on our business and financial condition and growth prospects.

***Enacted and future legislation and related regulations may increase the difficulty and cost for us to commercialize lomitapide or metreleptin, or any other product or any product candidate for which we obtain marketing approval, and may affect the prices we are able to obtain for them, if and where approved.***

In the U.S., there have been a number of legislative and regulatory changes and proposed changes regarding the healthcare system that restrict or regulate post-approval activities, and may affect our ability to profitably sell JUXTAPID or MYALEPT, or any other product or any product candidate for which we obtain marketing approval. Legislative and regulatory proposals have been made to expand post-approval requirements and restrict sales and promotional activities for pharmaceutical products. We cannot predict whether additional legislative changes will be enacted, or whether the FDA regulations, guidance or interpretations will be changed, or what the impact of such changes for JUXTAPID or MYALEPT may be. In addition, increased scrutiny by Congress of the FDA’s approval process may subject us to more stringent product labeling and post-marketing testing and other requirements.

In the U.S., most outpatient prescription drugs, including JUXTAPID and MYALEPT, may be covered under Medicare Part D. Medicare Part D prescription drug plans are authorized to use formularies where they can limit the number of drugs that will be covered in any therapeutic class and/or impose differential cost sharing or other utilization management techniques. The possibility that our products will be subject to such formularies places pressure on us to contain and reduce costs, which could negatively impact our commercialization success. Changes to Medicare Part D that give plans more freedom to limit coverage or manage utilization, and/or other cost reduction initiatives in the program could decrease the coverage and price that we receive for any approved products, and could be detrimental to our business.

The Healthcare Reform Act substantially changed the way healthcare is financed by both governmental and private insurers. The law contains a number of provisions that affect coverage and reimbursement of drug products and/or that could potentially reduce the demand for our products such as:

- increasing drug rebates under state Medicaid programs for brand name prescription drugs and extending those rebates to Medicaid managed care; and
- requiring drug manufacturers to provide a 50% discount on Medicare Part D brand name prescription drugs sold to Medicare beneficiaries whose prescription drug costs cause the beneficiaries to be subject to the Medicare Part D coverage cap (i.e., the so-called donut hole).

Modifications to or repeal of all or certain provisions of the Healthcare Reform Act has been a considerable focus of President Trump's term, and certain changes have already been approved and others changes may be pursued. We cannot predict the ultimate content, timing or effect of any changes to the Healthcare Reform Act or other federal and state reform efforts. There is no assurance that federal or state health care reform will not adversely affect our business and financial results, and we cannot predict how future federal or state legislative, judicial or administrative changes relating to healthcare reform will affect our business.

Any significant spending reductions affecting Medicare, Medicaid or other publicly funded or subsidized health programs that may be implemented and/or any significant taxes or fees that may be imposed on us, as part of any broader deficit reduction effort or legislative replacement to current laws or regulations, could have an adverse impact on our results of operations. Further, countries outside the U.S. may make changes to their healthcare systems, which may in the future affect the revenues we generate from sales of lomitapide and, if approved outside of the U.S., metreleptin, and other product or product candidates for which we obtain approval.

***We face extensive regulatory requirements, and may continue to face future development and regulatory difficulties.***

Even after marketing approval, a regulatory authority may still impose significant restrictions on a product's indications, conditions for use, distribution or marketing or impose ongoing requirements for post-marketing surveillance, post-approval studies or clinical trials. JUXTAPID is available in the U.S. only through the JUXTAPID REMS program. We must certify all healthcare providers who prescribe JUXTAPID and the pharmacies that dispense the medicine, and under the JUXTAPID REMS program, patients must now formally acknowledge that they understand the goals of the JUXTAPID REMS program and have undergone counseling by their prescriber to this effect. The FDA has also required that we periodically assess the effectiveness of the JUXTAPID REMS program. The FDA assesses on a periodic basis whether a REMS program is meeting its goals and whether the goals or elements of the plan should be modified. The modifications to the JUXTAPID REMS program approved in January 2017 and implemented later in 2017, including the labeling modifications, may negatively affect the ability or willingness of a healthcare professional to prescribe JUXTAPID, a patient to be willing to initiate or continue on therapy, or insurance companies, managed care organizations, other private payers, and government entities that provide reimbursement for medical costs to continue to provide reimbursement for JUXTAPID, in which case we will have difficulty achieving or maintaining market acceptance of JUXTAPID, and our business and ability to achieve our financial expectations will be negatively impacted. For additional information regarding changes to the JUXTAPID REMS program, see the risk factor captioned "*We may not be able to maintain or expand market acceptance for lomitapide or metreleptin in the U.S. or to gain market acceptance in markets outside the U.S. where we commercialize such products, and we may continue to see a significant number of patients who choose not to start or stay on therapy.*"

Similarly, MYALEPT is available only through the MYALEPT REMS program because of the potential for development of anti-metreleptin antibodies and the associated risks of serious adverse sequelae (such as severe infections, excessive weight gain, glucose intolerance, diabetes mellitus) and risk of lymphoma. As a part of this program, we must certify all healthcare providers who prescribe MYALEPT, certify the pharmacies that dispense the medicine, and obtain prescriber attestation that each patient has a diagnosis consistent with GL. We are responsible for maintaining, monitoring and evaluating the implementation of the MYALEPT REMS program.

Regulatory authorities have significant post-marketing authority, including, the authority to require labeling changes based on new safety information, and to require post-marketing studies or clinical trials to evaluate serious safety risks related to the use of a drug or biologic. For example, in July 2015, the FDA notified Aegerion that they considered post-marketing reports of anaphylaxis to be new safety information, and requested that we add it to the prescribing information for MYALEPT. Although Aegerion complied with that request, the FDA could request that additional safety information be included.

We are subject to certain post-marketing commitments to the FDA and the EMA with respect to lomitapide and to the FDA with respect to metreleptin, and we expect the EMA to subject metreleptin to post-marketing commitments as part of any approval of metreleptin. We expect that the regulatory authorities in certain other countries outside the U.S. and EU where our products are, or may be, approved may impose post-approval obligations, including patient registries, and requirements that may in some countries be more onerous than those imposed by the FDA and EMA. Depending on the nature of these post-marketing



studies, we may be required to provide our products free of charge to participants in the studies in certain countries even if we have pricing and reimbursement approval in such countries, which would negatively impact our level of revenues.

Where our products are approved outside the U.S. or are in the future approved, we are and will also be subject to other ongoing regulatory requirements governing the labeling, packaging, storage, advertising, distribution, promotion, recordkeeping and submission of safety, REMS, risk management program and other post-marketing information, including adverse reactions, and any changes to the approved product, product labeling, or manufacturing process. As a company with limited internal resources and expertise in these areas, we rely on third parties to facilitate our compliance with many of these extensive regulatory requirements, which often include detailed record keeping and reporting requirements. We and the third parties we work with may not be able to fully comply with these requirements or the reports we file with regulatory authorities may result in changes to our post-marketing compliance requirements. In addition, manufacturers of drug products and their facilities are subject to continual review and periodic inspections by the FDA, the EMA, the regulatory authorities of the EU Member States and other regulatory authorities for compliance with cGMP, and other regulations.

If we, or third-party service providers acting on our behalf, or our drug substance or drug product or the manufacturing facilities for our drug substance or drug product, fail to comply with applicable regulatory requirements, including global pharmacovigilance requirements and meeting the requirements of the JXTAPID and MYALEPT REMS programs, a regulatory agency may:

- issue warning letters or untitled letters;
- seek an injunction or impose civil or criminal penalties or monetary fines;
- suspend, withdraw or alter the conditions of our marketing approval;
- require us to provide corrective information to healthcare practitioners;
- require us to modify our product labels;
- suspend any ongoing clinical trials;
- require entrance into a consent decree, which, for example, is a component of Aegerion's Settlement, which can include imposition of various fines, reimbursements for inspection costs, required due dates for specific actions and penalties for noncompliance;
- refuse to approve pending applications or supplements to applications submitted by us;
- suspend or impose restrictions on operations, including costly new manufacturing requirements;
- seize or detain products, refuse to permit the import or export of products or request that we initiate a product recall;
- impose further refinements and enhanced obligations under existing risk management and other forms of post-marketing requirements and programs; or
- refuse to allow us to enter into supply contracts, including government contracts.

The occurrence of any event or penalty described above may inhibit our ability to commercialize our products and to generate revenues, which would have a material and adverse effect on our results of operations.

***We rely on third parties to conduct our clinical trials and registry studies and to perform related services, and those third parties may not perform satisfactorily, including by failing to meet established deadlines for the completion of such clinical trials and compliance with post-marketing requirements. We may become involved in commercial disputes with these parties.***

We do not have the ability to independently conduct clinical trials or registry studies, or perform pharmacovigilance and REMS program monitoring and reporting, and we rely on third parties, such as contract research organizations, medical institutions, academic institutions, clinical investigators, specialty pharmacies and other third-party service providers, to perform these functions. Our reliance on these third parties for clinical development, pharmacovigilance and REMS program activities reduces our control over these activities. However, if we sponsor clinical trials, we are responsible for ensuring that each of the clinical trials that we sponsor is conducted in accordance with the general investigational plan and protocols for the trial. We are responsible

for REMS program activities in connection with marketing lomitapide and metreleptin in the U.S. and pharmacovigilance monitoring and reporting for all of our products on a global basis, except that Shionogi is responsible for these activities for metreleptin in Japan, Korea and Taiwan, and Amryt is responsible for these activities for lomitapide in the EEA, Switzerland, Turkey and certain Middle Eastern and North African territories, including Israel. Moreover, the FDA and the regulatory authorities in the EU and Japan require us to comply with standards, commonly referred to as Good Clinical Practices, for conducting, recording, and reporting the results of clinical trials to assure that data and reported results are credible and accurate and that the rights, integrity and confidentiality of trial participants are protected. Our reliance on third parties does not relieve us of these responsibilities and requirements. Furthermore, these third parties may have relationships with other entities, some of which may be our competitors. If these third parties do not successfully carry out their contractual duties or meet expected deadlines, if they need to be replaced or if the quality or accuracy of the data they provide is compromised or delayed due to the failure to adhere to regulatory requirements or our clinical trial protocols, or for other reasons, our current marketing authorizations may be revoked, suspended, or revised to be more stringent, our development programs may be extended, delayed or terminated, additional marketing approvals for lomitapide or metreleptin, or any other product or any product candidate we might acquire, may be delayed or denied in the targeted indication or jurisdiction, and we may be delayed or precluded in our efforts to successfully commercialize lomitapide, metreleptin, or any other product for targeted indications or in the targeted jurisdiction or it may impact existing approvals, which could have a materially negative impact on our commercialization efforts.

In addition, we may, from time to time, become involved in commercial disputes with these third parties, for example regarding the quality of the services provided by these third parties or our ultimate liability to pay for services they purported to provide, or the value of such services. In some cases, we may be required to pay for work that was not performed to our specifications or not utilized by us, and these obligations may be material and may therefore have a material adverse effect on our results of operations.

***If we fail to comply with our reporting and payment obligations under the Medicaid Drug Rebate Program or other governmental pricing programs in the U.S., we could be subject to additional reimbursement requirements, penalties, sanctions and fines which could have a material adverse effect on our business, financial condition, results of operations and growth prospects.***

We participate in various government programs and contracts that require us to calculate and report certain prices for our products to government agencies or provide rebates or discounted pricing on products purchased to certain purchasers or government payers. The requirements for calculating prices and rebates are complex and subject to change. Changes to such requirements may affect our business and operations. We may also have reimbursement obligations or be subject to penalties if we fail to provide timely and accurate information to the government, pay the correct rebates or offer the correct discounted pricing.

For example, because of our participation in the Medicaid Drug Rebate Program, we are required to pay a rebate for each unit of our lomitapide or metreleptin reimbursed by a state Medicaid program as a condition of having federal funds made available to the states for our drugs under Medicaid and Medicare Part D. Those rebates are based on pricing data that we report on a monthly and quarterly basis to CMS, the federal agency that administers the Medicaid Drug Rebate Program. We may also participate in state Medicaid supplemental rebate programs which require payment of an incremental rebate to state Medicaid programs for covered utilization of our products. Price reductions as well as price increases that exceed the rate of inflation for our products, such as the price increase for MYALEPT in February of 2015, may result in increasing the rebates we are required to pay under the Medicaid Drug Rebate Program or state Medicaid supplemental rebate programs and the discounts we are required to offer under the Public Health Service 340B drug pricing discount program, as discussed below.

To maintain coverage of our products under the Medicaid Drug Rebate Program and Medicare Part D, we are required to extend significant discounts to certain “covered entities” (defined by statute to include certain types of hospitals and other healthcare providers that receive federal grants) that purchase products under the 340B Program. The 340B Program requires participating manufacturers to agree to charge such covered entities no more than the 340B “ceiling price” for the manufacturers’ covered outpatient drugs. The 340B ceiling price is calculated using a statutory formula, which is based on the average manufacturer price (“AMP”) and rebate amount for the covered outpatient drug as calculated under the Medicaid Drug Rebate Program. “Orphan drugs” - those designated under section 526 of the FDCA, such as JUXTAPID and MYALEPT - are exempt from the ceiling price requirements with respect to drugs purchased by certain covered entities (i.e., rural referral centers, sole community hospitals, critical access hospitals, and free-standing cancer hospitals).

Pricing and rebate calculations vary among products and programs. The calculations are complex and are often subject to interpretation by governmental or regulatory agencies and the courts. For example, the Medicaid rebate amount is computed each quarter based on our submission to the CMS of our AMP and best price for the quarter. If we become aware that our reporting for prior quarters was incorrect, or has changed as a result of recalculation of the pricing data, we will be obligated to resubmit the corrected data for a period not to exceed twelve quarters from the quarter in which the data originally were due. Such restatements and recalculations would serve to increase our costs for complying with the laws and regulations governing the Medicaid Drug

Rebate Program. Any corrections to our rebate calculations could result in an overage or underage in our rebate liability for past quarters, depending on the nature of the correction. Price recalculations also may affect the price that we will be required to charge certain safety net providers under the Public Health Service 340B drug discount program. In February 2015, we significantly increased the U.S. wholesale acquisition cost per 11.3 mg vial of MYALEPT. As a result of this substantial price increase, we continue to expect a significant gross-to-net adjustment for Medicaid rebates which will offset the majority of revenue from Medicaid and negatively impact net product sales in future quarters, since Medicaid rebates directly reduce our net product sales. The degree of such impact on our overall financial performance will depend on the percentage of MYALEPT patients that have Medicaid as their primary insurance coverage and the quantity of units ordered per patient. To date, approximately 34% of patients prescribed MYALEPT have been Medicaid beneficiaries. The number of patients prescribed MYALEPT in the future who are Medicaid beneficiaries could be higher than historical rates.

We are liable for errors associated with our submission of pricing data and for overcharging government payers. For example, in addition to retroactive rebates and the potential for 340B Program refunds, if we are found to have knowingly submitted false AMP or best price information to the government, we may be liable for civil monetary penalties in the amount of \$100,000 per item of false information. Our failure to submit monthly/quarterly AMP and best price data on a timely basis could result in a civil monetary penalty of \$10,000 per day for each day the submission is late beyond the due date. In the event that CMS were to terminate our rebate agreement, no federal payments would be available under Medicaid or Medicare Part D for our products. In addition, if we overcharge the government in connection with our Federal Supply Schedule (“FSS”) contract or under any other government program, we will be required to refund the difference to the government. Failure to make necessary disclosures and/or to identify contract overcharges could result in allegations against us under the federal civil False Claims Act and other laws and regulations.

To maintain coverage of our products under the Medicaid Drug Rebate Program and Medicare Part D and when purchased by four federal agencies, we are required to participate in the FSS pricing program. Under this program, we are obligated to make JUXTAPID and MYALEPT available for procurement on an FSS contract at a negotiated price and also charge a price to four federal agencies-VA, Department of Defense, Public Health Service, and Coast Guard-that is no higher than the statutory Federal Ceiling Price (“FCP”). The FCP is based on the non-federal average manufacturer price (“Non-FAMP”), which we calculate and report to the VA on a quarterly and annual basis. If a company misstates Non-FAMPs or FCPs it must restate these figures. Pursuant to the Veterans Health Care Act of 1992, knowing provision of false information in connection with a Non-FAMP filing can subject a manufacturer to penalties of \$100,000 for each item of false information.

FSS contracts are federal procurement contracts that include standard government terms and conditions, separate pricing for each product, and extensive disclosure and certification requirements. In addition to the four agencies described above, all other federal agencies and some non-federal entities are authorized to access FSS contracts. FSS contractors are permitted to charge FSS purchasers other than the four federal agencies “negotiated pricing” for covered drugs that is not capped by the FCP; instead, such pricing is negotiated based on a mandatory disclosure of the contractor’s commercial “most favored customer” pricing. Moreover, all items on FSS contracts are subject to a standard FSS contract clause that requires FSS contract price reductions under certain circumstances where pricing to an agreed “tracking” customer is reduced. In July 2016, we concluded negotiations with the Department of Veterans Affairs (the “VA”), and effective August 15, 2016, we have an FSS contract for both JUXTAPID and MYALEPT.

We also participate in the Tricare Retail Pharmacy program, under which we pay quarterly rebates on utilization of JUXTAPID and MYALEPT when the products are dispensed through the Tricare Retail Pharmacy network to Tricare beneficiaries. The rebates are calculated as the difference between annual Non-FAMP and FCP.

If we overcharge the government in connection with the VA FSS pricing program or the Tricare Retail Pharmacy program, whether due to a misstated FCP or otherwise, we are required to refund the difference to the government. Failure to make necessary disclosures and/or to identify contract overcharges could result in allegations against us under the False Claims Act and other laws and regulations.

Unexpected refunds to the U.S. government, and responding to a government investigation or enforcement action, would be expensive and time-consuming, and could have a material adverse effect on our business, financial condition, results of operations and growth prospects.

***Product development is a long, expensive and uncertain process, and we may terminate one or more of our development programs. If we do not achieve our projected development goals in the timeframes we expect and announce, or otherwise terminate one or more of our development programs, marketing approval and commercialization of our products may be delayed or otherwise cease. As a result, our credibility may suffer, our share price may decline and we may incur significant expenses that could adversely affect our prospects, our financial condition, and results of operations.***

For strategic and operational planning purposes, we may estimate the timing of the accomplishment of various scientific, clinical, regulatory and other product development goals. These milestones may include the commencement or completion of scientific studies and clinical trials and the submission and approval of regulatory filings. From time to time, we may publicly announce the expected timing of some of these milestones. All of these milestones and timelines are based on a variety of assumptions. The actual timing of these milestones, if even achieved, can vary dramatically compared to our estimates, in many cases for reasons beyond our control.

We may determine to discontinue or narrow certain programs because we determine they do not have potential or because we do not have sufficient resources to dedicate to such efforts, or we may elect to suspend, terminate or modify one or more of our programs, which could include changing our clinical or business model for further development, including by attempting to extract or monetize value from the program by either selling, out-licensing or potentially partnering part or all of the program. For example, through Aegerion, although we planned to submit a supplemental biologics licensing application (an “sBLA”) to the FDA in 2017 to expand MYALEPT’s indication in the U.S. to the PL subset, we received feedback in May 2017 from the FDA that a prospective placebo-controlled study will be required to support a marketing application in the U.S. for the use of metreleptin to treat a subset of the PL indication. We are evaluating next steps. If we terminate and seek to monetize part or all of a program in which we have invested significant resources, or we continue to expend further resources on a program and subsequently fail to achieve our intended goals, our prospects may suffer, as we will have expended resources on a program that may not provide a suitable return, if any, on our investment and we may have missed the opportunity to allocate those resources to potentially more productive uses. In addition, in the event of a termination of any product program, we may incur significant expenses and costs associated with the termination of the program or could be criticized or lose credibility in the industry, which could adversely affect our financial condition or results of operations.

***Failures or delays in the completion or commencement of any planned clinical trials of our products could result in increased costs to us and delay, prevent or limit our ability to generate revenues with respect to the relevant product in a new territory or indication. In addition, positive results in preclinical studies and earlier clinical trials of our products may not be replicated in later clinical trials, or changes in regulatory requirements or unanticipated events during our clinical trials may occur, which could result in development delay or a failure to obtain marketing approval or affect market acceptance.***

The commencement and completion of clinical trials may be delayed or prevented for a number of reasons, including:

- difficulties obtaining regulatory clearance to commence a clinical trial or complying with conditions imposed by a regulatory authority regarding the scope or term of a clinical trial;
- delays in reaching or failing to reach agreement on acceptable terms with prospective clinical research organizations (“CROs”) and trial sites, and problems with the performance of CROs;
- insufficient or inadequate supply or quality of a product or other materials necessary to conduct our clinical trials, or other manufacturing issues;
- difficulties obtaining institutional review board (“IRB”) approval or Ethics Committee’s positive opinion to conduct a clinical trial at a prospective site;
- challenges recruiting and enrolling patients to participate in clinical trials for a variety of reasons, including the size and nature of a patient population, the proximity of patients to clinical sites, the eligibility criteria for the trial, the nature of trial protocol, the availability of approved treatments for the relevant disease and the competition from other clinical trial programs for similar indications;
- severe or unexpected drug-related side effects experienced by patients in a clinical trial;
- if we do not have the funding or resources necessary to conduct, maintain or conclude such trials; and
- difficulties retaining patients who have enrolled in a clinical trial but may be prone to withdraw due to the rigors of the trials, lack of efficacy, side effects or personal issues, or who are lost to further follow-up.

For example, in May 2016, in connection with our MAA filing for metreleptin for the treatment of GL patients and a subset of PL patients in the EU, the EMA's PDCO issued a summary report regarding the metreleptin pediatric investigation plan ("PIP"). The PDCO expressed concern that the number of young patients with lipodystrophy included in the clinical trials proposed to be included in the PIP is very limited, and that no information on metreleptin as used by European patients was provided. In July 2016, the PDCO approved our proposal that we conduct a study in GL patients below the age of 6, as a deferred commitment. Given the prevalence of GL and other factors, conducting a clinical trial in GL patients in the EU below a defined age will likely make such a trial lengthy in nature and potentially difficult to complete. Even if we conduct a study in pediatric GL patients, we may not be able to show, to the satisfaction of the EMA, that metreleptin is safe and effective in pediatric patients under the age of 6, and we may never receive approval for this indication in the EMA. The lack of approval to market metreleptin for the pediatric GL population outside of the U.S. would limit expansion of our product revenue potential. Further, we plan to initiate, by late 2018, a phase 2 trial assessing metreleptin in HMD, subject to approval of our protocol and statistical plan by applicable regulatory authorities. Such authorities could impose conditions regarding our development plan that could delay commencement of the trial or we may face other hurdles to starting the trial. Even if our development plan is approved by applicable regulatory authorities, we could encounter difficulties enrolling patients and/or completing the trial.

Clinical trials may also be delayed or terminated as a result of ambiguous or negative interim results, or the results or reevaluation, of other clinical, preclinical or nonclinical studies. In addition, a clinical trial may be suspended or terminated by us, the FDA, the regulatory authorities of the EU Member States and other countries, the IRBs or the Ethics Committees at the sites, or a data safety monitoring board overseeing the clinical trial at issue, or other regulatory authorities due to a number of factors, including:

- failure to conduct the clinical trial in accordance with regulatory requirements or our clinical protocols;
- failure to respect applicable data privacy obligations;
- inspection of the clinical trial operations or trial sites by the FDA or other regulatory authorities;
- unforeseen safety issues or lack of effectiveness; and
- lack of adequate funding to continue the clinical trial.

If we do not meet timelines or achieve milestones in a timely manner or at all, the market approval and commercialization of the relevant product may be delayed or denied, and our credibility may be adversely affected and, as a result, our share price may decline.

Positive results in preclinical or clinical studies of lomitapide or metreleptin, or any other product or any product candidate that we may acquire, license or develop, may not be predictive of similar results in humans during further clinical trials. Accordingly, there is, for example, a possibility that any potential future clinical development of metreleptin for patients with HMD, in pediatric patients, or for any new indications may generate results that are not consistent with the results of the Phase 3 clinical study for the product, or the results of the Phase 2 clinical studies that showed, for instance, that metreleptin reduced the weight of overweight and obese subjects with low leptin levels, or the results of other relevant studies. The results of such clinical trials may not be sufficient to gain approval of metreleptin in any new population or indication, or may generate data that may cause reevaluation of and/or negatively impact the existing data and labels for approved indications. Our preclinical studies or clinical trials may produce negative or inconclusive results, and we may decide, or regulators may require us, to conduct additional preclinical studies or clinical trials. Moreover, preclinical and clinical data can be susceptible to varying interpretations and analysis, and many companies that believed their product candidates performed satisfactorily in preclinical studies and clinical trials have nonetheless failed to obtain FDA or other regulatory approval for their products.

Changes in regulatory requirements and guidance or unanticipated events during our clinical trials may occur, and as a result we may need to amend clinical trial protocols. Amendments may require us to resubmit our clinical trial protocols to the FDA, IRBs, Ethics Committees or the regulatory authorities of the applicable jurisdictions for review and approval, which may impact the cost, timing or successful completion of a clinical trial. If we experience delays in completion of, or if we terminate, any of our clinical trials or generate results that differ from earlier clinical trial results, the commercial prospects for the applicable product may be harmed.

***Recent federal legislation and actions by state and local governments may permit re-importation of drugs from foreign countries into the U.S., including foreign countries where the drugs are sold at lower prices than in the U.S. Similarly, purchasers in the EU are permitted to purchase products in one EU Member State and import them into another EU Member State where the price may be higher. These practices could materially adversely affect our operating results and our overall financial condition.***

The Medicare Prescription Drug, Improvement and Modernization Act contains provisions that may change importation laws and expand pharmacists' and wholesalers' abilities to import lower priced versions of an approved drug and competing products from Canada, where there are government price controls. These changes to U.S. importation laws, which will not take effect unless and until the Secretary of Health and Human Services certifies that the changes will pose no additional risk to the public's health and safety, may result in a significant reduction in the cost of products to consumers. While the Secretary of Health and Human Services has not yet announced any plans to make this required certification, we may ultimately face the risk that a distributor or other purchaser of JUXTAPID or MYALEPT in the U.S. will be permitted to import lower priced product from a country outside the U.S. that places price controls on pharmaceutical products. This risk may be particularly applicable to JUXTAPID and MYALEPT as drugs that currently command premium prices, and especially to JUXTAPID, as a drug that is formulated for oral delivery. In addition, some state governments have implemented importation schemes for their citizens and, in the absence of federal action to curtail such activities, other state governments may launch importation efforts.

In the EU, a purchaser cannot be restricted from purchasing a medicinal procedure in one EU Member State and importing the product into another EU Member State in which it is also subject to marketing authorization, provided the product concerned is the same or very similar to a product already authorized for sale in the Member State into which it is to be imported. This activity is called parallel importing, which is permitted in the EU based on the principle of free movement of goods. As a result, a purchaser in one EU Member State where lomitapide or, if approved, metreleptin, is sold at a specific price may seek to import the product from another EU country where the product is sold at a lower price.

The re-importation of lomitapide or metreleptin into the U.S. market from a foreign market and the parallel importation of lomitapide, and, if approved, metreleptin, among countries of the EU or other regions could negatively impact our revenues and anticipated financial results, possibly materially.

***We face potential product liability exposure, and, if claims are brought against us, we may incur substantial liability.***

The use of any product or product candidate in clinical trials and the sale and use of any product for which we have or obtain marketing approval expose us to the risk of product liability claims. Product liability claims might be brought against us by consumers, healthcare providers or others selling or otherwise coming into contact with our products, or any product candidates. If we cannot successfully defend ourselves against product liability claims, we could incur substantial liabilities. In addition, regardless of merit or eventual outcome, product liability claims may result in:

- decreased demand or coverage for our products and any product candidate for which we obtain marketing approval;
- impairment of our business reputation and exposure to adverse publicity;
- increased warnings on product labels;
- withdrawal of clinical trial participants;
- costs as a result of related litigation;
- distraction of management's attention from our primary business;
- substantial monetary awards to patients or other claimants;
- loss of revenues; and
- the inability to successfully commercialize our products or any product candidate for which we obtain marketing approval.

Although we have obtained product liability insurance coverage for both our clinical trials and our commercial exposures, our insurance coverage may not be sufficient to reimburse us for any expenses or losses we may suffer. Moreover, insurance coverage is becoming increasingly expensive, and, in the future, we may not be able to maintain insurance coverage at a reasonable cost or in sufficient amounts to protect us against losses due to liability. On occasion, large judgments have been awarded in class

action lawsuits relating to drugs that had unanticipated side effects or warnings found to be inadequate. The cost of any product liability litigation or other proceedings, even if resolved in our favor, could be substantial. A product liability claim or series of claims brought against us could harm our reputation and cause our share price to decline and, if the claim is successful and judgments exceed our insurance coverage, could have a material adverse impact on our business, financial condition, results of operations and prospects.

***Our operations outside of the U.S. subject us to a variety of risks, which could materially adversely affect our business.***

In each country outside the U.S. in which lomitapide is approved, or where we are making lomitapide or metreleptin available on a named patient or compassionate use basis before it has obtained marketing approval, we are subject to additional risks related to international business operations, directly and as a result of the activities of third parties with whom we do business, including:

- differing regulatory requirements for drug approvals in foreign countries;
- pricing, pricing deals and reimbursement approvals that have a negative impact on our global pricing strategy;
- potentially reduced protection for intellectual property rights;
- the potential for parallel importing;
- unexpected changes in tariffs, trade barriers and regulatory requirements;
- economic weakness, including inflation, or political instability in particular foreign economies and markets, including, for example, the current political instability in Brazil, our largest source of revenues on a country-by-country basis outside the U.S.;
- compliance with foreign and U.S. laws, rules, regulations or industry codes, including data privacy requirements, labor relations laws, anti-competition regulations, import, export and trade restrictions, and required reporting of payments to healthcare professionals and others;
- negative consequences from changes in applicable tax laws;
- foreign currency fluctuations, which could result in increased operating expenses and reduced revenues, and other obligations incident to doing business in another country;
- workforce uncertainty in countries where labor unrest is more common than in the U.S.;
- production shortages resulting from any events affecting raw material supply or manufacturing capabilities abroad;
- dependence upon third parties to perform distribution, pharmacovigilance, quality control testing, collections and other aspects of the distribution, supply chain and commercialization of our products that are required to be performed in order to conduct such activities in international markets, and our ability to effectively manage such third parties; and
- business interruptions resulting from geopolitical and economic events or actions, including social unrest, economic crises, war, terrorism, or natural disasters.

In addition to the foregoing, we are subject to anti-corruption laws, including the Foreign Corrupt Practices Act (the “FCPA”) and various other anti-corruption laws. The FCPA generally prohibits companies and their intermediaries from making improper payments to foreign officials for the purpose of obtaining or keeping business and/or other benefits. An aspect of the SEC’s ongoing investigation into Aegerion’s disclosures and activities relates to alleged FCPA violations in Brazil. These potential violations are excluded from the Settlement and may therefore subject us to additional fines, penalties or remedial actions.

Our activities outside the U.S. and those of our employees, licensees, distributors, manufacturers, clinical research organizations and other third parties who act on our behalf or with whom we do business subject us to the risk of investigation and prosecution under foreign and U.S. laws. For example, as described in detail in the “*Legal Proceedings*” section of this Annual Report, federal and state authorities in Brazil are conducting an investigation to determine whether there have been violations of Brazilian laws related to sales of JUXTAPID in Brazil. These issues could negatively affect our ability to generate product revenues

for JUXTAPID consistent with our expectations, and may impact our ability to achieve profitability or cash-flow positive operations. Prescriptions for and named patient sales of MYALEPT in Brazil may also be negatively affected.

Despite our ongoing efforts to ensure compliance with foreign and domestic laws, our employees, agents, and companies with which we do business may nevertheless take actions in violation of our policies, for which we may be ultimately held responsible. If so, we may be subject to criminal or civil penalties or other punitive measures, including restrictions on our ability to continue selling in certain markets. Any such outcome, or any allegation or investigation regarding such actions involving us, could harm our reputation and have a material adverse impact on our business, financial condition, results of operations and prospects.

***We may infringe the intellectual property rights of others, which may prevent or delay our product development efforts and stop us from commercializing or may increase the costs of commercializing our products.***

Our success will depend in part on our ability to operate without infringing the proprietary rights of third parties. There could be issued patents of which we are not aware that our products infringe. There also could be patents that we believe we do not infringe, but that we may ultimately be found to infringe. Moreover, patent applications are in some cases maintained in secrecy until patents are issued. The publication of discoveries in the scientific or patent literature frequently occurs substantially later than the date on which the underlying discoveries were made and patent applications were filed. Because patents can take many years to issue, there may be currently pending applications of which we are unaware that may later result in issued patents that our products infringe. For example, pending applications may exist that provide support or can be amended to provide support for a claim that results in an issued patent that our product infringes.

The pharmaceutical industry is characterized by extensive litigation regarding patents and other intellectual property rights, such as the IPR challenges filed with the PTAB concerning certain patents directed to methods-of-use of lomitapide, which were dismissed by the PTAB in March 2017. Other parties may obtain patents in the future and allege that our products or the use of our technologies infringes these patent claims or that we are employing their proprietary technology without authorization. Likewise, third parties may challenge or infringe upon our existing or future patents.

Proceedings involving our patents or patent applications or those of others could:

- put one or more of our patents at risk of being invalidated or interpreted narrowly;
- adversely impact the patentability of our inventions relating to our products;
- result in monetary damages, injunctive relief or otherwise harm our competitive position, including by limiting our marketing and selling activities, increasing the risk for generic competition, limiting our development and commercialization activities or requiring us to obtain licenses to use the relevant technology (which licenses may not be available on commercially reasonable terms, if at all); and
- otherwise negatively impact the enforceability, validity or scope of protection offered by our patents relating to our products.

Even if we are successful in these proceedings, we may incur substantial costs and divert management's time and attention in pursuing these proceedings, which could have a material adverse effect on us. If we are unable to avoid infringing the patent rights of others, we may be required to seek a license, defend an infringement action or challenge the validity of the patents in court. Patent litigation is costly and time consuming. We may not have sufficient resources to bring these actions to a successful conclusion.

In addition, if we do not obtain a license, develop or obtain non-infringing technology, fail to defend an infringement action successfully or have infringed patents declared invalid, we may:

- incur substantial monetary damages;
- encounter significant delays in expanding the market of our products, or bringing any future products to market; and
- be precluded from manufacturing or selling any products, or any future product candidates.

In such event, our business could be adversely affected, possibly materially.



***If we are not able to adequately prevent disclosure of trade secrets and other proprietary information, the value of our technology and product portfolio could be significantly diminished.***

We rely on trade secrets to protect our proprietary technologies, especially where we do not believe patent protection is adequate or obtainable. However, trade secrets are difficult to protect. We rely in part on confidentiality agreements with our employees, consultants, outside scientific collaborators, sponsored researchers and other advisors to protect our trade secrets and other proprietary information. These agreements may not effectively prevent disclosure of confidential information, and may not provide an adequate remedy in the event of unauthorized disclosure of confidential information. In addition, others may independently discover our trade secrets and proprietary information. For example, the FDA is currently considering whether to make additional information publicly available on a routine basis, and the EMA is planning to amplify its disclosure rules. These changes could mean that information that we may consider to be trade secrets or other proprietary information may be disclosed, and it is not clear at the present time how the FDA's and EMA's disclosure policies may change in the future, if at all. Costly and time-consuming litigation could be necessary to enforce and determine the scope of our proprietary rights, and failure to obtain or maintain trade secret protection could adversely affect our competitive business position.

***If we fail to comply with our obligations in our license agreements for our products, we could lose license rights that are important to our business or have to make additional payments to our licensors.***

Our existing license agreements with respect to our products impose, and we expect any future license agreements that we enter into will impose, various diligence, milestone payment, royalty, insurance, reporting, audit and other obligations on us. If we fail to comply with such obligations or encounter disagreements with our license partners, we could lose license rights that are important to our business. For further information regarding our obligations and restrictions under our license agreements for our products, see the " *Licensing* " section of this Annual Report.

If we fail to comply with the obligations and restrictions under our license agreements, the applicable licensor may have the right to seek financial payments or damages and terminate the license, in which case we might not be able to market any product that is covered by the license. Further, the terms of such license agreements can be intensely negotiated, complex and subject to differing interpretations. Any breach or alleged breach (whether intentional or unintentional), or termination, of the license agreements applicable to our products, or any disagreements as to the application of the terms of our license agreements with our licensing partners, could expose us to considerable costs and expenses as such allegations, terminations or disputes can be costly to remedy or resolve (regardless of the merits), and may not be remedied or resolved in a manner favorable to us, or at all. For example, under our co-development agreement for zuretinol and the underlying license agreement between our co-development partner and the licensor of zuretinol, we and our co-development partner are required to use commercially reasonable and diligent efforts to develop and commercialize zuretinol. We and our co-development partner are also responsible for committing certain annual funding to support research and development of zuretinol, and the agreement between our co-development partner and the licensor requires the achievement of specific development milestones within certain timeframes, one of which was required to be achieved by December 31, 2016. However, the agreement contains provisions for extensions of those dates in certain circumstances. Based on the terms of these agreements, we believe that we may be entitled to certain additional extensions of that milestone date until December 31, 2019, along with a potential additional extension of up to 12 months should enrollment in a planned trial be delayed, provided that we continue to comply with the relevant provisions of the license agreements and expend certain minimum amounts on the development of zuretinol. Any breach or alleged breach of the quantitative or qualitative diligence provisions of these agreements could result in termination of our co-development agreement and our loss of the program and also expose us to considerable costs and expenses and any disputes may not be remedied or resolved in a manner favorable to us, or at all.

Any such difficulties with our license agreements could have a significant adverse effect on our business because of our reliance on the commercial success of our products.

***The occurrence of cyber incidents, or a deficiency in cybersecurity, could negatively impact our business by causing a disruption to our operations, a compromise or corruption of confidential information, exposure to legal and regulatory action, or damage to our patient, partner or employee relationships, any of which could subject us to loss and harm our reputation.***

A cyber incident is considered to be any event that threatens the confidentiality, integrity or availability of information resources. More specifically, a cyber incident is an intentional attack or an unintentional event that can include gaining unauthorized access to systems to disrupt operations, corrupt data or steal confidential information about patients, suppliers, partners or employees. A number of companies have recently experienced serious cyber incidents and breaches of their information technology systems. Cyber incidents pose risks both to our internal systems and to those we have outsourced, including the risk of operational interruption, damage to our reputation and relationships with patients, partners and employees, and private data exposure. We have implemented processes, procedures and controls to help mitigate these risks. However, these measures, as well as our increased

awareness of the risk of a cyber incident, do not guarantee that our reputation, operations and financial results would not be adversely affected by such an incident.

In the ordinary course of our business, we collect and store sensitive data, including intellectual property, proprietary business information and patient data. This includes, where required or permitted by applicable laws, personally identifiable information. Certain third parties with whom we contract also collect and store such data related to clinical trial subjects and patients. The secure maintenance of this information is critical to our operations and business strategy. Despite our security measures, our information technology and infrastructure may be vulnerable to attacks by hackers or breached due to employee error, malfeasance or other disruptions. Any such breach could compromise information stored on our networks or those of our partners. Any access, disclosure or other loss of information could result in legal claims or proceedings, liability under laws that protect the privacy of personal information, recovery costs, disruption of our operations, including delays in our regulatory approval efforts, and damage our reputation, which could adversely affect our business.

***Our future success depends on our ability to hire and retain our key executives and to attract, retain, and motivate qualified personnel. We may encounter difficulties in managing our business with limited resources.***

Our success depends upon retaining, recruiting and motivating key employees. Experienced employees in the biopharmaceutical and biotechnology industries are in high demand and competition for their talents can be intense. We have entered into employment agreements with our executive officers and certain other employees, but any employee may terminate his or her employment with us at any time. Recent management turnover, our recent headcount reduction, litigation and government investigations, including the JUXTAPID Investigations, the Settlement, potential financing and investment challenges and other risks facing our business may adversely affect our ability to attract, motivate and retain executives and other key employees and keep them focused on applicable strategies and goals. The loss of the services of any of these executives or key employees, or our inability to recruit desirable candidates, could impede the achievement of our development and commercialization objectives and could have a negative impact on our stock price. Aegerion's workforce reductions in 2016 in connection with its cost-reduction plan, and the attrition thereafter, along with Novelion's workforce reductions in January 2018, have resulted in the loss of longer-term employees, the loss of institutional knowledge and expertise and the reallocation and combination of certain roles and responsibilities across the organization, which have had and could continue to have an adverse effect on our operations. We may lose out on business opportunities, not be able to comply with business requirements, and face reduced productivity among remaining employees, if employees are distracted, if morale is low or, if workloads are too burdensome or if continuing employees are unfamiliar with the tasks at hand. As a result, our financial performance and our ability to commercialize lomitapide and metreleptin successfully, and to compete effectively, would be negatively affected.

***We have incurred significant operating losses since our inception, and have not yet achieved profitability for any fiscal year . We may never be profitable.***

We have incurred losses in each year since our inception. As of December 31, 2017 , we had an accumulated deficit of approximately \$714.0 million . Substantially all of our operating losses resulted from costs incurred in connection with our development programs and from selling, general and administrative costs associated with our operations. The losses we have incurred to date, combined with potential future losses, have had and may continue to have an adverse effect on our shareholders' (deficit) equity and working capital.

We expect to incur expenses related to the commercialization of lomitapide and metreleptin in the U.S. and in the key countries in which lomitapide is currently approved and being commercialized, including Japan, or in which lomitapide or metreleptin may be approved and commercialized in the future, and expected distribution of our products in Brazil and certain other countries as part of named patient supply or compassionate use; manufacturing costs for both lomitapide and metreleptin; the conduct of our observational cohort studies and other post-marketing commitments to the FDA for lomitapide and metreleptin, including the REMS program for each of our products; the conduct of any post-marketing commitments imposed by regulatory authorities in countries outside the U.S. and EU where our products are, or may be, approved; other possible clinical development activities for our products, including an anticipated clinical trial for metreleptin in the pediatric population or a subset thereof, and an anticipated clinical trial to assess metreleptin in HMD patients; regulatory activities for our products; and business development activities. We expect to incur significant royalties, sales, marketing, and outsourced manufacturing expenses, as well as research and development expenses. In addition, we expect to continue to incur additional costs associated with operating as a public company and in connection with servicing Aegerion's debt and compliance with the Settlement, our ongoing government investigations and the potential outcomes thereof, and litigation related thereto, as described in detail in the " *Legal Proceedings* " section of this Annual Report.

Our ability to become profitable depends upon our ability to generate significant revenue. Because of the numerous risks and uncertainties associated with developing and commercializing pharmaceutical products, we are unable to predict with certainty the extent of any future losses or when we will become profitable, if at all. We may not continue to generate substantial revenues from sales of lomitapide or metreleptin. If we are unable to continue to generate significant product revenues, we will not become profitable, and may be unable to continue operations without additional funding. Our ability to generate revenues sufficient to achieve profitability currently depends on a number of factors, including our ability to:

- continuing to sell JUXTAPID as a last-line treatment for adult HoFH patients in the U.S. despite the availability of PCSK9 inhibitor products, which have had a significant adverse impact on sales of JUXTAPID, and gaining market acceptance in the other countries, including Japan, where lomitapide is approved and being commercialized, or may in the future receive approval and be commercialized;
- minimize the expected negative impact of the availability of PCSK9 inhibitor products on sales of lomitapide outside the U.S., including in Japan, where we launched JUXTAPID in December 2016 and where a PCSK9 inhibitor product is available, and the degree to which the availability of PCSK9 inhibitor products outside the U.S., and the potential availability of named patient sales of PCSK9 inhibitor products outside the U.S., impact named patient sales of lomitapide outside the U.S., particularly in Brazil;
- effectively respond to requirements of insurance companies, managed care organizations, other private payers, and government entities that provide reimbursement for medical costs in the U.S. to require that newly diagnosed adult HoFH patients be treated with PCSK9 inhibitor products prior to JUXTAPID treatment, that current JUXTAPID patients switch to PCSK9 inhibitor products, and that potential JUXTAPID patients fail to adequately respond to PCSK9 inhibitor products before providing reimbursement for JUXTAPID at the prices at which we offer JUXTAPID;
- build and maintain market acceptance for MYALEPT in the U.S. for the treatment of complications of leptin deficiency in GL patients, and support named patient sales of metreleptin in GL in Brazil, particularly in light of local economic challenges, ongoing government investigations, an ongoing court proceeding reviewing the regulatory framework for named patient sales in Brazil, and recently implemented regulatory requirements for named patient sales which have added complexity to the process for named patient sales in Brazil and we believe have led a significant number of patients to discontinue therapy with our product, and supporting such sales in other key countries, including Turkey and France, where such sales are permitted;
- raise sufficient capital and service Aegerion's debt in a timely manner and on favorable terms to enable us to invest in the development, growth and commercialization of our products and to maintain our operations;
- maintain reimbursement policies for JUXTAPID and MYALEPT in the U.S. that do not impose significant restrictions on reimbursement and a payer mix that does not include significantly more Medicaid patients than the current payer mix;
- prepare for the launch of metreleptin in Europe as a treatment for complications of leptin deficiency in GL patients and a subset of PL, in the event we obtain regulatory, pricing and reimbursement approvals in the EU for metreleptin;
- execute on possible lifecycle management opportunities for metreleptin, including potential future clinical development of metreleptin in HMD and potential additional low-leptin mediated diseases;
- continue to have named patient sales of our products in Brazil and other key countries where such sales can occur as a result of the FDA approval, particularly in light of local economic challenges, ongoing government investigations, ongoing court proceedings in Brazil reviewing the regulatory framework for named patient sales, and negative media coverage in Brazil;
- obtain timely regulatory approval of lomitapide in Brazil as a treatment for patients with HoFH and metreleptin in the EU, Brazil and other key international markets as a treatment for patients with GL or a subset of PL, in each case without onerous restrictions, limitations, or post-marketing commitments in the resulting label, or conditions to approval;
- gain pricing and reimbursement approvals for our products in countries, including MYALEPTA in the EU for GL and the PL subset, in which we elect to seek, and eventually obtain, regulatory approval, at acceptable prices and without significant restrictions, discounts, caps or other cost containment measures, and to effectively launch our products in those countries where it makes business sense to do so;

- minimize the number of patients who are eligible to receive but decide not to commence treatment with our products, or who discontinue treatment, including with lomitapide, due to tolerability issues, and with metreleptin, due to its route of administration as a daily injection, through activities such as patient support programs, to the extent permitted in a particular country; and
- effectively estimate the size of the total addressable market for our products.

In addition, as described above, federal enforcement agencies recently have shown interest in pharmaceutical companies' product and patient assistance programs, including manufacturer reimbursement support services and relationships with specialty pharmacies, and contributions to charitable organizations that assist patients in accessing treatment for certain diseases and conditions. In addition to the risks associated with the costs of responding to government investigation or enforcement actions (such as a False Claims Act action), federal enforcement agencies' increased attention to such programs and contributions may lead to changes that adversely affect our business. For example, we believe that investigations and enforcement actions by these agencies have resulted in a reduction in contributions to third-party 501(c)(3) organizations that assist patients in accessing treatment for certain diseases and conditions. If a lack of available funds prevents these third-party 501(c)(3) organizations from providing adequate financial assistance, including assistance with co-payment obligations, to individuals who would otherwise be unable to afford our products, our revenues may decline below our expectations.

***The market price of our common shares has been, and may continue to be, highly volatile.***

Our share price is highly volatile and is subject to wide fluctuations in price in response to various factors, many of which are beyond our control, including the following:

- the short-term or long-term success or failure of our commercialization efforts and the level of revenues generated from sales of our products in the U.S.;
- the level of revenues we receive from named patient sales of our products in Brazil and other key countries where a mechanism exists to sell the product on a pre-approval basis in such country based on U.S. approval of such products or EU approval of lomitapide, particularly in light of the regulatory approval of Amgen's PCSK9 inhibitor product in Brazil in April 2016, the potential availability of that and other PCSK9 inhibitor products on a named patient sales basis in Brazil, the additional requirements that have been recently imposed on named patient sales of pharmaceutical products in Brazil, including our products, and potential future additional requirements or limitations, and the ongoing court proceedings in Brazil reviewing the regulatory framework for named patient sales;
- our cash position;
- the August 2019 maturity of and the dilutive effect of Aegerion's Convertible Notes, the warrants issued in connection with the New Loan Agreement or the availability and terms of any other debt, equity or equity-linked financings or alternative strategic arrangements;
- the perception of the terms of the Settlement, and any adverse consequences that may result from the terms of the final approved Settlement, such as additional demands, litigation or investigations;
- the extent to which the JUXTAPID REMS program may negatively affect the ability or willingness of a physician to prescribe JUXTAPID, a patient to be willing to initiate or continue on JUXTAPID therapy, or insurance companies, managed care organizations, other private payers, and government entities that provide reimbursement for medical costs to continue to provide reimbursement for JUXTAPID;
- our ability to gain regulatory approvals to market our products in countries in which the products are not currently approved and/or reimbursed or for new indications, including obtaining approval of the MAA seeking marketing approval of metreleptin in the EU as a treatment for complications of leptin deficiency in GL patients and a subset of PL;
- the short-term or long-term success or failure of the commercialization of our products in key countries outside the U.S. in which we have or obtain approval, and the level of revenues we generate;
- our ability to accurately forecast net product sales and operating expenses, and to meet such forecasts;
- our ability, or lack thereof, to manage our costs and expenses to better align with our revenues, and strengthen our capital structure, while supporting approved products in a compliant manner;

- the timing and cost of seeking regulatory approvals and conducting potential future clinical development of metreleptin in HMD;
- the extent to which changes to the labeling for JUXTAPID instructing patients to cease therapy upon the occurrence of severe diarrhea may negatively affect the ability or willingness of a healthcare professional to prescribe JUXTAPID, a patient to be willing to initiate or continue on therapy, or insurance companies, managed care organizations, other private payers, and government entities that provide reimbursement for medical costs to continue to provide reimbursement for JUXTAPID;
- any issues that may arise with our supply chain for our products or in connection with our licensing arrangements;
- any adverse regulatory decisions, or regulatory issues that arise, made with respect to our products;
- any issues that may arise with respect to the safety of our products;
- the acceleration of any indebtedness or other financial obligations;
- additional changes in, or loss of, key personnel, especially in light of recent management changes;
- our ability, and the resources expended, to defend ourselves successfully against claims made in securities class action or similar lawsuits, and, if we are unsuccessful in such defense or decide to settle, the type and amount of any damages, settlement amounts, fines or other payments or adverse consequences that may result;
- any adverse actions or decisions related to our intellectual property or marketing or data exclusivity, or any action by a third party to gain approval of a generic or biosimilar product, including, for lomitapide, for which a generic challenge can be filed with the FDA in light of the expiration of exclusivity on December 21, 2017;
- fluctuations in stock market prices and trading volumes of similar companies;
- general market conditions and overall fluctuations in U.S. and Canadian equity markets;
- low trading volume and short interest positions in our common shares;
- international financial market conditions;
- variations in our quarterly operating results;
- changes in our financial guidance or securities analysts' estimates of our financial performance;
- announcements of investigations or litigation, and updates to the status of investigations and litigation, or other notifications from enforcement or regulatory authorities related to our business or business practices;
- announcements of clinical data, regulatory submissions, product launches, new products, services or technologies, commercial relationships, acquisitions or other events by us or our competitors;
- changes in or materially incorrect application of accounting principles;
- issuance by us of new securities, or sales of large blocks of our common shares, including sales by our executive officers, directors and significant shareholders;
- success or failure of products within our therapeutic areas of focus;
- discussion of us or our share price by the financial press and in online investor communities;
- our relationships with and the conduct of third parties on which we depend; and
- other risks and uncertainties described in these risk factors.

In particular, our forecasts and guidance as to our financial performance are predicated on many assumptions, most notably that we have correctly forecast our U.S. and non-U.S. revenues for both of our products, including for sales of JUXTAPID in Japan and for named patient sales of both products to the federal Ministry of Health in Brazil, and that sales continue as we have forecasted to those patients who have previously received JUXTAPID or MYALEPT and, in Brazil, to new patients who have obtained federal court orders for JUXTAPID or MYALEPT treatment, particularly in light of the ongoing investigations in Brazil and the lawsuits currently being heard by the Brazil Supreme Federal Court to decide whether the government has an obligation to provide drugs, such as JUXTAPID and MYALEPT, that have not received regulatory and/or pricing and reimbursement approval in Brazil. Such factors may cause additional delays or eventually the suspension of the ordering process in Brazil. We have also assumed that our forecasts concerning sales of metreleptin in other key markets are correct, including named patient sales in France and Turkey and, assuming we receive approval for MYALEPTA in the EU, post-approval commercial sales in Germany. If any of our assumptions turn out to be incorrect, including our assumptions with respect to our ability to build and maintain market acceptance for our products for HoFH and GL in territories in which they have been approved; are subject to approval, including GL and the PL subset in the EU, assuming regulatory approval; or are eligible for named patient sales, or the extent of the negative impact of the availability of PCSK9 inhibitor products in the U.S., the EU, Japan, and Brazil on our sales of lomitapide in those countries and in other countries where PCSK9 inhibitors are, in the future, approved or available on a named patient basis, our financial results could be weaker than expected, and the price of our common shares could decline, perhaps significantly.

In addition, the stock market in general, and the market for small pharmaceutical and biotechnology companies in particular, have experienced extreme price and volume fluctuations that have often been unrelated or disproportionate to the operating performance of these companies. Also, broad market and industry factors may negatively affect the market price of our common shares, regardless of our actual operating performance. In the past, following periods of volatility in the market in a company's stock, securities class-action litigation has often been instituted against such a company. For example, Aegerion recently settled the Class Action Litigation. See the "Legal Proceedings" section of this Annual Report for further information. These proceedings have, and similar litigation could, if instituted against us, result in substantial costs and diversion of management's attention and resources, which could materially and adversely affect our business and financial condition.

***Our internal controls over financial reporting could fail to prevent or detect misstatements or have material weaknesses.***

Our internal controls over financial reporting may not prevent or detect misstatements. Also, projections of any evaluation of effectiveness to future periods are subject to the risk that controls may become inadequate because of changes in conditions, or that the degree of compliance with the policies or procedures may deteriorate. Any failure to maintain effective internal controls or to timely effect any necessary improvement or remediate any lapse in our internal control and disclosure controls could, among other things, result in losses from fraud or error, require significant resources and divert management's attention, harm our reputation, causing investors to lose confidence in our reported financial and other information, and expose us to legal or regulatory proceedings, all of which could have a material adverse effect on our financial condition, results of operations and cash flows.

During 2016, a material weakness in internal control over our financial reporting process was identified as we did not design and maintain sufficiently precise or effective review and approval controls over the forecasts used to develop management estimates, including those related to balances acquired in the Aegerion business combination that occurred during fiscal year 2016. Although management, in 2017, implemented a number of remediation efforts, there has been a lack of instances to test the remediated controls in 2017; as a result, the material weakness remains un-remediated as of December 31, 2017. Management has performed additional analysis and procedures to conclude that the Consolidated Financial Statements included in this Annual Report fairly present, in all material respects, our financial condition as of December 31, 2017 and 2016 and results of operations for the three years ended December 31, 2017. See "Management's Annual Report On Internal Control Over Financial Reporting." Even if we remediated this material weakness effectively, we may in the future identify additional material weaknesses.

***Changes in our effective income tax rate could adversely affect our results of operations.***

We and our subsidiaries are subject to income and other taxes in Canada, the United States, and many other tax jurisdictions throughout the world. Tax laws and rates in these jurisdictions are subject to change. Our effective income tax rate can vary significantly between periods due to a number of complex factors, including, but not limited to: (i) interpretations of existing tax laws; (ii) the accounting for business combinations, including accounting for contingent consideration; (iii) the tax impact of existing or future legislation; (iv) changes in accounting standards; (v) changes in the mix of earnings in the various tax jurisdictions in which we operate; (vi) the outcome of examinations by the Canada Revenue Agency, the U.S. Internal Revenue Service ("IRS") and other foreign tax authorities; (vii) adjustments to income taxes upon finalization of income tax returns; (viii) the accuracy of our estimates for unrecognized tax benefits; and (ix) increases or decreases to valuation allowances recorded against deferred tax assets. For example, in December 2017, Congress passed a comprehensive tax reform bill that includes significant changes to taxation of business entities, including a permanent reduction to the federal corporate income tax rate, a partial limitation on the

deductibility of business interest expense, adopting elements of a “territorial” tax system, assessing a repatriation tax or “toll-charge” on undistributed earnings and profits of U.S.-owned foreign corporations, and introducing certain anti-base erosion provisions. The impact of this tax reform on us is uncertain; however, a reduction in the U.S. corporate income tax rate could impair the value of our deferred tax assets which are discussed below. If our effective tax rate increases, our operating results and cash flow could be adversely affected.

***Any changes to existing accounting pronouncements or taxation rules or practices may cause adverse fluctuations in our reported results of operations or affect how we conduct our business.***

A change in accounting pronouncements or taxation rules or practices can have a significant effect on our reported results and may affect our reporting of transactions completed before the change is effective. New accounting pronouncements, taxation rules and varying interpretations of accounting pronouncements or taxation rules have occurred in the past and may occur in the future. The change to existing rules, future changes, if any, or the need for us to modify a current tax or accounting position may adversely affect our reported financial results or the way we conduct our business.

***We may be treated as a U.S. domestic corporation for U.S. federal income tax purposes.***

Under current U.S. federal tax law, a corporation is generally considered for U.S. federal income tax purposes to be a tax resident in the jurisdiction of its organization or incorporation. Accordingly, under the generally applicable U.S. federal income tax rules, because we are incorporated under the laws of British Columbia, Canada, we would be classified as a non-U.S. corporation (and, therefore, not a U.S. tax resident) for U.S. federal income tax purposes. Section 7874 of the Internal Revenue Code of 1986, as amended (the “Code”) provides an exception to this general rule, under which a non-U.S. incorporated entity will nevertheless be treated as a U.S. corporation for U.S. federal income tax purposes (and, therefore, as a U.S. tax resident subject to U.S. federal income tax on its worldwide income) if each of the following three conditions are met: (i) the non-U.S. corporation, directly or indirectly, acquires substantially all of the properties held directly or indirectly by a U.S. corporation (including through the acquisition of all of the outstanding shares of the U.S. corporation), (ii) the non-U.S. corporation’s “expanded affiliated group” does not have “substantial business activities” in the non-U.S. corporation’s country of organization or incorporation and tax residence relative to the expanded affiliated group’s worldwide activities and (iii) after the acquisition, the former shareholders of the acquired U.S. corporation hold at least 80% (by either vote or value) of the shares of the non-U.S. acquiring corporation by reason of holding shares in the U.S. acquired corporation (taking into account the receipt of the non-U.S. corporation’s shares in exchange for the U.S. corporation’s shares) as determined for purposes of Section 7874 (this test is referred to as the “80% ownership test”).

On April 4, 2016, the U.S. Treasury Department (the “Treasury”) and the IRS issued the Temporary Section 7874 Regulations, which, among other things, require certain adjustments that generally increase, for purposes of the 80% ownership test, the percentage of the shares of the acquiring non-U.S. corporation deemed owned (within the meaning of Section 7874) by the former shareholders of the acquired U.S. corporation by reason of holding shares in such U.S. corporation. It is possible that Aegerion shareholders could be deemed to have acquired for purposes of Section 7874 more than 80% of Novelion in the merger (the “Merger”) of the indirect, wholly-owned subsidiary of the Company, Isotope Acquisition Corp. with and into Aegerion, pursuant to an Agreement and Plan of Merger, effective as of November 29, 2016, and that as a result we would be treated as a U.S. corporation for U.S. federal income tax purposes. If we were to be treated as a U.S. corporation for U.S. federal tax purposes, we could suffer adverse tax consequences, including potential U.S. income taxes on future profits distributed from non-U.S. subsidiaries and loss of eligibility for benefits under the income tax treaty between Canada and the U.S.

If the Section 7874 percentage is calculated to be at least 60% or more (but less than 80%), Section 7874 can limit the ability of the acquired U.S. corporation and its U.S. affiliates to utilize U.S. tax attributes such as net operating losses to offset U.S. taxable income resulting from certain transactions. Additionally, related rules may impose an excise tax under Section 4985 of the Code on the gain recognized by certain “disqualified individuals” (including officers and directors of a U.S. company) on certain stock-based compensation held by such individuals at a rate equal to 15%. We may, if we determine that it is appropriate, provide disqualified individuals with payments to offset this excise tax, so that, on a net after-tax basis, they would be in the same position as if no such excise tax had been applied.

After taking into account the relevant adjustments under the temporary 7874 regulations and based on the facts and circumstances as of the date of the Merger, the Section 7874 percentage following the Merger was calculated to be less than 60% and, as a result, we do not believe the excise tax applies to “disqualified individuals.” However, if the IRS determines that the excise tax does apply, the payment of such excise tax will be costly to us.

Further, even if we are not treated as a U.S. corporation for U.S. federal income tax purposes under the inversion rules, there can be no assurance as to the effective tax rates applicable to our future revenues. For example, the ability of the companies

to locate personnel and to integrate and manage operations in a manner that supports and protects the tax benefits that potentially may be realized from Novellion's Canadian tax domicile is uncertain and complex.

Potential improvements to our effective tax rate that may result from Novellion's Canadian tax domicile have not been reflected in the pro forma financial information.

***We may not be able to use our net operating loss carryforwards ("NOLs") to offset future taxable income for U.S. or Canadian federal income tax purposes.***

As of December 31, 2017, we had NOLs for U.S. federal income tax purposes of approximately \$60.5 million, which expire at various dates through 2037.

Under Section 382 of the Code, if a corporation subject to the Code undergoes an "ownership change," generally defined as a greater than 50% change (by value) in its equity ownership over a three-year period, the corporation's ability to use its pre-change U.S. NOLs, and other pre-change tax attributes (such as research tax credits) to offset its post-change income may be limited. We underwent an "ownership change" within the meaning of Section 382 and 383 of the Code as a result of the Merger, and therefore an annual limit may be imposed on the amount of NOLs that may be used to offset future taxable income. Such annual limit is generally the product of the total value of a company's outstanding equity immediately prior to an "ownership change" (subject to certain adjustments) and the applicable federal long-term tax exempt interest rate. Certain of our U.S. subsidiaries underwent an "ownership change" (as defined above) and triggered the limitation on use of NOLs in 2005, 2012, and 2016. Due to the ownership changes, we have determined that our U.S. subsidiaries, including Aegerion, will only be able to utilize a small percentage of their NOLs and tax attributes. In connection with the Merger, we have determined that Aegerion had a net unrealized built-in-loss (an "NUBIL"). The NUBIL was determined based on the difference between the fair market value of Aegerion's assets and their tax basis as of the ownership change date. Because of the NUBIL, certain deductions recognized during the five-year period beginning on the date of the Section 382 ownership change are subject to the same limitation as the NOL carryforwards. Our U.S. subsidiaries may also experience ownership changes in the future as a result of subsequent shifts in share ownership. As a result, if our U.S. subsidiaries earn net taxable income, their ability to use their pre-change NOL carryforwards to offset U.S. federal taxable income may be subject to limitations, which could potentially result in increased future tax liabilities. In addition, at the U.S. state level, there may be periods during which the use of NOLs is suspended or otherwise limited, which could accelerate or permanently increase state taxes owed.

As of December 31, 2017, we had NOLs for Canadian federal income tax purposes of approximately \$176.9 million, which expire at various dates through 2037. The extent to which we can utilize any or all of our NOLs will depend on many factors, including the jurisdiction applicable to any future taxable revenue of Novellion.

Our ability to use NOLs will also depend on the amount of taxable income generated in future periods. The NOLs may expire before we can generate sufficient taxable income to use the NOLs. NOLs generated after December 31, 2017 will not be subject to expiration. As described above, the reduction in the U.S. corporate income tax rate could impair the value of our deferred tax assets.

***Our business could be negatively affected as a result of proxy contests and other actions of activist shareholders.***

Proxy contests have been waged against many companies in the biopharmaceutical industry over the last few years. If faced with a proxy contest, we may not be able to successfully defend against the contest, which would be disruptive to our business. Even if we are successful, our business could be adversely affected by a proxy contest because:

- responding to proxy contests and other actions by activist shareholders may be costly and time-consuming and may disrupt our operations and divert the attention of our management and our employees;
- perceived uncertainties as to our future direction may result in our inability to consummate potential acquisitions, collaborations or in-licensing opportunities and may make it more difficult to attract and retain qualified personnel and business partners; and
- if individuals are elected to our Board of Directors with a specific agenda different from our strategy for creating long-term shareholder value, it may adversely affect our ability to effectively and timely execute on our strategic plans and create additional value for our shareholders.



***Anti-takeover provisions in our Articles, certain provisions under the BCBCA, the Canadian take-over bid rules and our advance notice policy could prevent or delay transactions that our shareholders may favor and may prevent shareholders from changing the direction of our business or management.***

Provisions of our Articles and certain provisions under the *British Columbia Business Corporations Act* (the “BCBCA”) may discourage, delay or prevent a merger or acquisition that our shareholders may consider favorable, including transactions in which shareholders might otherwise receive a premium for their shares, and may also frustrate or prevent any attempt by shareholders to change our direction or management. For example, these provisions:

- require a 66 2/3% majority of shareholder votes cast in favor of a resolution to effect various amendments to our Articles; and
- require shareholder proposals for matters to be acted upon by shareholders at shareholder meetings to be submitted pursuant to, and in accordance with, the applicable provisions of the BCBCA for inclusion in the Company’s proxy materials by a date that is not later than three months prior to the anniversary date of the prior year’s shareholder meeting.

Canada’s take-over bid rules provide that take-over bids for Canadian issuers will be subject to a minimum 105-day deposit period, subject to certain exceptions. The take-over bid rules, together with the above-noted provisions, among others, whether alone or together, could delay or impede hostile takeovers and changes in control. Any provision of our organizational documents that has the effect of delaying or deterring a change in control could limit the opportunity for our shareholders to receive a premium for their common shares and could also affect the price that some investors are willing to pay for our common shares.

We also have a shareholder-approved advance notice policy which establishes advance notice requirements for nominations for election to our Board of Directors. This policy may delay or impede changes to the composition of our Board of Directors or management.

***Provisions of Canadian law may delay, prevent or make undesirable an acquisition of all or a significant portion of our common shares or assets.***

The Investment Canada Act subjects an acquisition of control of us by a non-Canadian to government review if our enterprise value as calculated pursuant to the legislation exceeds a threshold amount. A reviewable acquisition may not proceed unless the relevant Minister is satisfied that the investment is likely to be of net benefit to Canada. This could prevent or delay a change of control and may eliminate or limit strategic opportunities for shareholders to sell their common shares.

***The rights of our shareholders may differ from the rights typically offered to shareholders of a U.S. corporation, including the right to bring actions or enforce judgments against us and certain of our directors, and these differences may make our common shares less attractive to investors.***

We are incorporated under the laws of the Province of British Columbia, Canada, and therefore certain of the rights of holders of its shares are governed by Canadian law, including the provisions of the BCBCA, and by our Notice of Articles and Articles. These rights differ in certain respects from the rights of shareholders in typical U.S. corporations and these differences may make our common shares less attractive to investors. For example, it may not be possible for an investor to enforce judgments obtained in U.S. courts based upon the civil liability provisions of U.S. federal securities laws or other laws of the U.S. against us.

***Future sales or issuances of our common shares may cause our share price to decline.***

Sales or issuances of a substantial number of our common shares in the public market, or a sale or issuance of securities convertible into common shares, or the perception that these sales or issuances might occur, could significantly reduce the market price of our common shares and impair our ability to raise adequate capital through the sale of additional equity or equity linked securities. If our existing shareholders sell, or if the market believes our existing shareholders will sell, substantial amounts of our common shares in the public market, the trading price of our common shares could decline significantly. If additional shares are sold or otherwise enter the market, or if it is perceived that they will be sold in or otherwise enter the public market, the price of our common shares could decline substantially. In addition to the common shares underlying Aegerion’s Convertible Notes and the warrants issued in connection with the New Loan, there are also approximately 2,500,000 common shares that are subject to outstanding options to purchase common shares and restricted stock unit awards and reserved for issuance under our equity plans, and will need additional shares for equity awards in the near future. The common shares underlying such currently outstanding options and restricted stock unit awards can be, and any shares underlying equity awards that we issue, or that are reserved for

issuance, in the future are expected to be able to be, registered and freely saleable in the public market upon issuance, subject to vesting restrictions.

**Item 1B. Unresolved Staff Comments.**

Not applicable.

**Item 2. Properties.**

During 2016, we exercised a one-year renewal option on our office lease in Vancouver, British Columbia. The lease term applicable to this space expired on August 31, 2017.

Our U.S. operational office, which is located at One Main Street in Cambridge, Massachusetts, consists of approximately 31,571 square feet of office space under a lease that expires in April 2019.

We also lease office spaces in Argentina, Brazil, Canada, Colombia, France, Germany, Italy, Japan, Spain, Switzerland, the UK, and Turkey, with approximately 9,540 square feet of office space in the aggregate. Our international lease agreements expire at various dates through the year 2023.

In addition to the locations listed above, we hold inventory at various locations, including international locations, managed by third parties.

**Item 3. Legal Proceedings.**

In late 2013, Aegerion received a subpoena from the DOJ, represented by the U.S. Attorney's Office in Boston, requesting documents regarding its marketing and sale of JUXTAPID in the U.S., as well as related public disclosures. In late 2014, Aegerion received a subpoena from the SEC requesting certain information related to Aegerion's sales activities and disclosures related to JUXTAPID. The SEC also requested documents and information on a number of other topics, including documents related to the investigations by government authorities in Brazil into whether Aegerion's activities in Brazil violated Brazilian anti-corruption laws, and whether Aegerion's activities in Brazil violated the FCPA.

In May 2016, Aegerion reached preliminary agreements in principle with the DOJ and the SEC to resolve their investigations into U.S. commercial activities and disclosures relating to JUXTAPID. On September 22, 2017, Aegerion entered into a series of agreements in an effort to resolve investigations being conducted by the DOJ and the SEC regarding these topics. The terms of these agreements were substantially similar to the preliminary agreements in principle.

In connection with the SEC investigation, Aegerion consented to the entry of a final judgment, on September 25, 2017, in connection with a complaint filed by the SEC on September 22, 2017 without admitting or denying the allegations set forth in the complaint (the "SEC Judgment"). The complaint alleged negligent violations of Sections 17(a)(2) and (3) of the Securities Act of 1933, as amended, related to certain statements made by Aegerion in 2013 regarding the conversion rate for JUXTAPID prescriptions. The SEC Judgment provides that Aegerion must pay a civil penalty in the amount of \$4.1 million, to be paid in installments over three years, plus interest on any unpaid balance at a rate of 1.75% per annum. Aegerion's payment of this civil penalty is subject to acceleration in the event of certain change of control transactions or certain transfers of Aegerion's rights in JUXTAPID or MYALEPT. Aegerion's payment schedule is also subject to acceleration in the event that Aegerion fails to satisfy its payment obligations under the SEC Judgment. The SEC Judgment was approved by a U.S. District Court judge on September 25, 2017.

In connection with the DOJ investigation, Aegerion entered into a Plea Agreement, a Deferred Prosecution Agreement ("DPA"), a Civil Settlement, certain State Settlement Agreements, and a Consent Decree of Permanent Injunction ("FDA Consent Decree"). Under the DOJ Plea Agreement, Aegerion agreed to plead guilty to two misdemeanor misbranding violations of the FDCA. On November 20, 2017, the U.S. District Court rejected Aegerion's Plea Agreement. On January 12, 2018, Aegerion entered into a new Plea Agreement with the DOJ. On January 30, 2018, a U.S. District Court Judge sentenced Aegerion after accepting Aegerion's guilty criminal plea. The Court did not impose a criminal fine and instead ordered Aegerion to pay restitution, in the amount of \$7.2 million payable over three years, plus interest on any unpaid balance at a rate of 1.75% per annum, into a fund managed by an independent claims administrator. As contemplated by the Plea Agreement, Aegerion was further sentenced to a three-year term of probation. Among the terms of probation, Aegerion must (i) comply with federal, state and local laws, (ii) pay restitution in accordance with the payment schedule set by the Court, (iii) notify its probation officer of any prosecution, major civil litigation or administrative proceeding, (iv) seek permission of its probation officer prior to selling, assigning or transferring assets, (v) notify its probation officer of any material change in its economic circumstances, (vi) forbear from disparaging the

factual basis of Aegerion's plea or denying that Aegerion itself is guilty, and (vii) comply with the DPA and CIA. Reports prepared by the independent review organization and FDA Auditor, as discussed below, must be provided to its probation officer. Under the terms of the DPA, Aegerion admitted it engaged in conduct that constituted a conspiracy to violate the HIPAA. The DPA provides that Aegerion must continue to cooperate fully with the DOJ concerning its investigation into other individuals or entities. The DPA provides that Aegerion must maintain a robust Compliance and Ethics Program (as defined in the DPA) that requires, among other things, a designated Compliance Officer and Compliance Committee; written compliance policies and procedures; a training program focused on Aegerion's compliance policies and procedures; a disclosure program to allow individuals to report potential legal and/or compliance violations, including violations of HIPAA; a non-retaliation policy; and a monitoring and auditing program. Under the DPA, Aegerion, as well as the Board of Directors of the Company (or a designated committee thereof), must also conduct regular reviews of its Compliance and Ethics Program, provide certifications to the DOJ that the program is believed to be effective and notify the DOJ of any probable violations of HIPAA. In the event Aegerion breaches the DPA, there is a risk the government would seek to impose remedies provided for in the DPA, including instituting criminal prosecution against Aegerion and/or seeking to impose stipulated penalties against Aegerion. The DPA is subject to review and supervision by a U.S. District Court judge.

Aegerion also entered into the DOJ Civil Settlement Agreement to resolve allegations by the DOJ that false claims for JUXTAPID were submitted to governmental healthcare programs. The DOJ Civil Settlement Agreement requires Aegerion to pay a civil settlement in the amount of \$28.8 million, which includes \$2.7 million designated for certain U.S. states relating to Medicaid expenditures for JUXTAPID, to be paid in installments over three years. Aegerion's payment of this civil settlement amount is subject to acceleration in the event of certain change of control transactions or certain transfers of Aegerion's rights in JUXTAPID or MYALEPT. In the event that Aegerion fails to satisfy its obligations under the DOJ Civil Settlement Agreement, Aegerion could be subject to additional penalties or litigation.

Aegerion also agreed to enter into the State Settlement Agreements to resolve claims under state law analogues to the federal False Claims Act. The terms of the State Settlement Agreements are substantially similar to those set forth in the DOJ Civil Settlement Agreement. As noted above, participating states will receive up to \$2.7 million in the aggregate from the \$28.8 million amount to be paid pursuant to the DOJ Civil Settlement Agreement.

Aegerion also agreed to the FDA Consent Decree with the DOJ and the FDA to resolve a separate civil complaint alleging that the company violated the FDCA by failing to comply with the JUXTAPID REMS program and the requirement to provide adequate directions for all of the uses for which it distributed JUXTAPID. The FDA Consent Decree requires Aegerion, among other things, to comply with the JUXTAPID REMS program; retain a qualified independent auditor to conduct annual audits of its compliance with the JUXTAPID REMS program; and remediate any noncompliance identified by the auditor within specified timeframes. In the event Aegerion fails to comply with the JUXTAPID REMS program or any other provisions of the FDA Consent Decree, Aegerion could be subject to additional administrative remedies, civil or criminal penalties and/or stipulated damages. Aegerion is required to notify the FDA in advance of certain changes in control, or changes in its business that may affect its operations, assets, rights or liabilities in the United States. The FDA Consent Decree does not take effect until it is approved by the Court and the injunction order is issued.

Separately, Aegerion entered into a CIA with the Department of Human Services Office of the Inspector General ("OIG"). The CIA requires Aegerion, among other things, to maintain a Compliance Program (as defined in the CIA) that includes: the designation of a Compliance Officer and a Compliance Committee; comprehensive written policies and procedures regarding the operation of the Compliance Program and appropriate conduct related to sales, marketing, reimbursement, incentive compensation and other matters; training and education regarding the Compliance Program and requirements of the CIA; a centralized annual risk assessment and mitigation process; an independent review and analysis of Aegerion's systems, transactions, risk assessment and mitigation process and other compliance activities; a disclosure program that allows individuals to report issues or questions associated with Aegerion's policies, conduct, practices or procedures; a field force monitoring program to evaluate and monitor sales personnel's interactions with healthcare professionals; monitoring of non-promotional activities, including consultants, donations to independent third-party patient assistance programs and other types of grants; certain requirements for the variable compensation programs for its U.S. sales personnel; and an executive financial recoupment program that puts at risk of forfeiture and recoupment performance pay for certain of Aegerion's and the Company's executives. Under the CIA, Aegerion, as well as the Board of Directors of the Company (or a designated committee thereof), must also conduct regular reviews of Aegerion's Compliance Program and provide an annual resolution or certification to OIG that the program is believed to be effective. Additionally, Aegerion must obtain management certifications from certain employees who are expected to monitor and oversee Aegerion's activities, which must be provided to OIG. Aegerion has reporting obligations under the CIA, including with respect to any ongoing investigation or legal proceeding involving an allegation that Aegerion has engaged in any fraudulent activities or committed a crime, any communications with FDA regarding improper promotion or marketing of Aegerion's products and any probable violations of criminal, civil or administrative laws applicable to federal healthcare programs. In the event Aegerion

breaches the CIA, there is a risk the government would seek to impose remedies provided for in the CIA, including seeking to impose stipulated penalties against Aegerion and/or seeking to exclude Aegerion from participation in federal healthcare programs.

In March 2014, an amended qui tam complaint was filed under seal in the District of Massachusetts against Aegerion, two former executive officers and a former employee. *United States ex rel Clarke v. Aegerion Pharm. Inc.*, No. 13-cv-11785-IT. On September 22, 2017, the U.S. filed a notice of intervention as to Aegerion. On September 27, 2017, the qui tam relators filed a second amended complaint naming additional parties, including a former board member, former executives, and former employees of Aegerion, as well as other third parties. The second amended complaint noted that the relators would file a joint stipulation of dismissal with respect to Aegerion upon the completion of certain conditions set forth in the Civil Settlement Agreement. On October 27, 2017, the court granted Aegerion and relators' joint motion to stay proceedings until sentencing in the criminal matter is complete and on February 20, 2018, the DOJ filed a stipulation of dismissal with respect to Aegerion in the civil qui tam matter.

Aegerion continues to cooperate with the DOJ and the SEC with respect to their investigations into the conduct of other individuals regarding commercial activities and disclosures related to JUXTAPID. As part of this cooperation, the DOJ requested documents and information related to donations Aegerion made in 2015 and 2016 to 501(c)(3) organizations that provide financial assistance to patients. In connection with this inquiry, the DOJ may pursue theories that were not resolved pursuant to the Settlement. Additionally, the Settlement does not resolve the DOJ and SEC inquiries concerning Aegerion's operations in Brazil.

In addition, federal and state authorities in Brazil are conducting an investigation to determine whether there have been violations of Brazilian laws related to the sales of JUXTAPID in Brazil. In July 2016, the Ethics Council of Interfarma fined Aegerion Brazil approximately \$0.5 million for violations of the industry association's Code of Conduct, to which Aegerion Brazil is bound due to its affiliation with Interfarma. Also, the Board of Directors of Interfarma imposed an additional penalty of suspension of Aegerion Brazil's membership, without suspension of Aegerion Brazil's membership contribution, for a period of 180 days for Aegerion Brazil to demonstrate the implementation of effective measures to cease alleged irregular conduct, or exclusion of its membership in Interfarma if such measures are not implemented. Aegerion Brazil paid approximately \$0.5 million related to this fine during the third quarter of 2016. In March 2017, after the suspension period ended, Interfarma's Board of Directors decided to reintegrate Aegerion Brazil, enabling it to participate regularly in Interfarma activities, subject to meeting certain obligations. Also, in July 2016, Aegerion Brazil received an inquiry from a Public Prosecutor Office of the Brazilian State of Paraná asking it to respond to questions related to media coverage regarding JUXTAPID and its relationship with a patient association to which Aegerion made donations for patient support. This preliminary inquiry was later reclassified as a civil inquiry, which is a preliminary procedure by the Public Prosecutor's Office that aims to verify if there are enough elements for it to file a formal lawsuit or to dismiss the inquiry. In June 2017, the Federal Public Prosecutor of the City of São José dos Campos, State of São Paulo, requested that a Brazilian federal court provide federal investigators with access to the bank records of certain individuals and entities, including Aegerion's subsidiary in Brazil ("Aegerion Brazil"), certain former Aegerion Brazil employees, a Brazilian patient association, and certain Brazilian physicians. The Court has not yet ruled on the Federal Public Prosecutor's request. The Public Prosecutors in Paraná and São José dos Campos continue to gather information in connection with their respective investigations. At this time, we do not know whether the inquiries of the Public Prosecutors in Paraná or São José dos Campos will result in the commencement of any formal proceeding against Aegerion, but if Aegerion's activities in Brazil are found to violate any laws or governmental regulations, Aegerion may be subject to significant civil lawsuits to be filed by the Public Prosecution office, and administrative penalties imposed by Brazilian regulatory authorities and additional damages and fines. Under certain circumstances, Aegerion could be barred from further sales to federal and/or state governments in Brazil, including sales of JUXTAPID and/or MYALEPT, due to penalties imposed by Brazilian regulatory authorities or through civil actions initiated by federal or state public prosecutors. As of the filing date of this Annual Report, we cannot determine if a loss is probable as a result of the investigations and inquiry in Brazil and whether the outcome will have a material adverse effect on our business and, as a result, no amounts have been recorded for a loss contingency.

In January 2014, a putative class action lawsuit was filed against Aegerion and certain of its former executive officers in the U.S. District Court for the District of Massachusetts alleging certain misstatements and omissions related to the marketing of JUXTAPID and Aegerion's financial performance in violation of the federal securities laws. On July 22, 2016, co-lead plaintiffs and defendants filed a joint motion to stay the briefing schedule while they pursued mediation, which the Court granted on August 10, 2016. Through mediation, the co-lead plaintiffs and defendants reached an agreement in principle to settle the litigation on November 29, 2016. On January 17, 2017, the co-lead plaintiffs filed a stipulation of settlement with the Court that contained the settlement terms as agreed upon by the parties, including that Aegerion and its insurance carriers would contribute \$22.3 million to a settlement fund for the putative class. The insurance carriers agreed to cover \$22.0 million of this amount, with Aegerion responsible for the remainder of \$0.3 million. On June 29, 2017, the Court entered an order preliminarily approving the settlement. Aegerion and its insurance carriers contributed their respective portions of the settlement fund as of July 14, 2017. Class members had until October 31, 2017, to object to or file objections or postmark requests to opt-out of the settlement. No class members filed objections to the settlement by the October 31 deadline. On November 30, 2017, the Court approved the settlement and dismissed the action in its entirety with prejudice.

On September 22, 2015, we commenced an action in the Supreme Court of British Columbia against Valeant Pharmaceuticals International, Inc. for breach of contract under the terms of the asset purchase agreement with Valeant, entered into on September 21, 2012, pursuant to which we sold all of our assets related to Visudyne<sup>®</sup>, including our Qcellus<sup>™</sup> laser and certain other photodynamic therapy intellectual property, with respect to failure to pay a \$5.0 million laser earn-out payment and failure to use commercially reasonable efforts to promptly obtain the laser registrations for the Qcellus laser in the U.S. In December 2017, we agreed to a settlement of this litigation in exchange for a settlement payment from Valeant of \$0.5 million, and such receivable was recorded as other income in our Consolidated Statements of Operations for the year ended December 31, 2017. For additional information, refer to Note 18 - *Commitments & Contingencies - Related to the Sale of Visudyne* in this Annual Report.

**Item 4. Mine Safety Disclosures.**

Not applicable.

## PART II

### Item 5. Market for Registrant’s Common Equity, Related Shareholder Matters and Issuer Purchases of Equity Securities.

Effective at the close of market on May 3, 2017, as part of a cost reduction process, we voluntarily delisted our common shares from the Toronto Stock Exchange ("TSX"), due to the low trading volume of our shares on the TSX over a sustained period, which we believed no longer justified the financial and administrative costs associated with maintaining a dual listing. Canadian shareholders continue to be able to trade our shares on the NASDAQ exchange through their brokers who have U.S. registered broker-dealer affiliates. Our common shares are currently traded in the U.S. on NASDAQ under the symbol "NVLN." The following table sets forth the high and low sales prices for our common shares in each of 2017 and 2016, as quoted on NASDAQ and TSX. Since we delisted from TSX in May 2017, only the high and low sales prices for our common shares for the first and second quarter of 2017 are presented, as adjusted to give effect to the Consolidation discussed in Part I, Item 1 - "Business - Recent Corporate and Securities Transactions" section of this Annual Report:

	Toronto Stock Exchange		NASDAQ Global Select Market	
	High (CAD\$)	Low (CAD\$)	High (U.S.\$)	Low (U.S.\$)
<b>2017</b>				
First Quarter	\$ 16.09	\$ 10.80	\$ 11.97	\$ 8.15
Second Quarter	14.47	13.30	10.82	8.35
Third Quarter	—	—	9.59	6.77
Fourth Quarter	—	—	7.17	3.12
<b>2016</b>				
First Quarter	\$ 19.40	\$ 12.55	\$ 11.85	\$ 8.19
Second Quarter	14.10	8.00	9.37	6.10
Third Quarter	14.10	8.50	10.95	6.52
Fourth Quarter	14.75	10.25	13.80	7.65

(1) Prior to completing the Merger with Aegerion on November 29, 2016, our shares traded under the symbol "QLTI" on NASDAQ.

On March 12, 2018, the closing price per share of our common shares was \$5.08, as reported on the NASDAQ, and there were 70 registered holders of our common shares.

#### Equity Compensation Plan Information as of December 31, 2017

Information regarding our equity compensation plans is included in the "Security Ownership of Certain Beneficial Owners and Management and Related Shareholder Matters" section of this Annual Report and incorporated in this Item 5 by reference.

#### Performance Graph

The following performance graph compares cumulative total shareholder return on the common shares of NVLN for the last five fiscal years with the total cumulative return of the NASDAQ Composite Index and the NASDAQ Biotechnology Index over the same period. Pursuant to applicable SEC rules, all values assume reinvestment of the full amount of all dividends, however no dividends have been declared on our common shares to date.

**Comparison of 5 Year Cumulative Total Return  
Assumes Initial Investment of \$100  
December 2017**



	Year Ended December 31,					
	2012	2013	2014	2015	2016	2017
Novelon Therapeutics Inc.	\$ 100	\$ 70.87	\$ 51.02	\$ 33.84	\$ 28.94	\$ 10.72
NASDAQ Composite Index	\$ 100	\$ 138.32	\$ 156.85	\$ 165.84	\$ 178.28	\$ 228.63
NASDAQ Biotechnology Index	\$ 100	\$ 165.97	\$ 223.06	\$ 249.32	\$ 196.09	\$ 238.50

The graph above assumes \$100 invested on December 31, 2012 in common shares of Novelon and in each index. The shareholder returns shown above are historical and not necessarily indicative of future price performance, and we do not make or endorse any predictions as to future shareholder returns.

The material in this section captioned Performance Graph is being furnished and shall not be deemed “filed” with the U.S. Securities and Exchange Commission (“SEC”) for purposes of Section 18 of the Securities Exchange Act of 1934, as amended or otherwise subject to the liability of that section, nor shall the material in this section be deemed to be incorporated by reference in any registration statement or other document filed with the SEC under the Securities Act of 1933, as amended, except to the extent we specifically and expressly incorporate it by reference into such filing.

**Dividend Policy**

We do not anticipate paying any dividends in the foreseeable future. We currently anticipate that we will retain all future earnings, if any, for use in the development of our business. The declaration of dividend payments is at the sole discretion of our Board of Directors. The Board of Directors may declare dividends in the future depending upon numerous factors that ordinarily affect dividend policy, including the results of our operations, our financial position and general business conditions.

**Use of Proceeds from Registered Securities**

Not applicable.

**Purchases of Equity Securities by the Issuer and Affiliated Purchasers**

During the year ended December 31, 2017, there were no common share repurchases.

## Exchange Controls and Other Limitations Affecting Holders of Common Shares

There is no limitation imposed by Canadian law or the Notice of Articles or Articles of the Company on the right of non-residents to hold or vote common shares in the Company, other than those imposed by the Investment Canada Act (Canada) (the "Investment Act"). Generally speaking, the Investment Act establishes the following two principal procedures for certain investments involving Canadian businesses, as defined by the Investment Act, by an individual, government or agency thereof, corporation, partnership, trust or joint venture that is not a "Canadian," as defined in the Investment Act (a non-Canadian): either the filing of an application for review which, except in certain limited circumstances, must be filed before closing and the non-Canadian cannot complete its investment until the Minister responsible for the Investment Act has determined that the investment is "likely to be of net benefit to Canada," or the filing of a notice, which must be filed within 30 days after the completion of the investment. A notice is not subject to substantive review and is required for investments by a non-Canadian that involve either the establishment of a new Canadian business or that involve an acquisition of control of a Canadian business but the prescribed thresholds for review are not exceeded. Subject to the possible application of the national security provisions, the Investment Act does not apply to investments in existing Canadian businesses that do not result in an acquisition of control, as defined under the Investment Act.

A direct investment by a non-Canadian to acquire control of a Canadian business is a reviewable investment where the value of the assets of the corporation, based on the corporation's fiscal year immediately preceding the investment, is CAD\$5 million or more. Higher limits apply for direct acquisitions by or from World Trade Organization ("WTO") member country investors, as described below.

The acquisition of a majority of the voting interests of an entity or of a majority of the undivided ownership interests in the voting shares of an entity that is a corporation is deemed to be acquisition of control of that entity. The acquisition of less than a majority but one-third or more of the voting shares of a corporation or of an equivalent undivided ownership interest in the voting shares of the corporation is presumed to be acquisition of control of that corporation unless it can be established that, on the acquisition, the corporation is not controlled in fact by the acquirer through the ownership of voting shares. The acquisition of less than one-third of the voting shares of a corporation or of an equivalent undivided ownership interest in the voting shares of the corporation is deemed not to be acquisition of control of that corporation. Certain transactions in relation to common shares in the Company would be exempt from review from the Investment Act, including:

- a. acquisition of common shares by a person in the ordinary course of that person's business as a trader or dealer in securities;
- b. acquisition of control of us in connection with the realization of security granted for a loan or other financial assistance and not for any purpose related to the provisions of the Investment Act; and
- c. acquisition of control of us by reason of an amalgamation, merger, consolidation or corporate reorganization following which the ultimate direct or indirect control in fact of the Company, through the ownership of voting interests, remains unchanged.

Under the Investment Act, a direct investment in common shares of the Company by a non-Canadian who is a WTO investor (as defined in the Investment Act) would be reviewable only if it were an investment to acquire control of the Company and the enterprise value of the Company was CAD\$1 billion or more, starting June 22, 2017. A different threshold applies to an acquisition by a state-owned enterprise ("SOE"). Currently, where the acquisition is by a SOE, the investment would be reviewable if the value of our assets was CAD\$379 million or more.

The Minister responsible under the Investment Act can, within a prescribed period, require the review of an investment by a non-Canadian (even one that does not amount to an acquisition of control, and/or does not meet the review thresholds set out above) on grounds that it is likely to be injurious to national security. Ultimately, the Cabinet can prohibit the completion of an investment, or require divestment of control of a completed investment, or impose terms and conditions on an investment where the investment is injurious to national security.

### Item 6. Selected Financial Data.

#### Annual Financial Data

The following selected financial data should be read in conjunction with our Consolidated Financial Statements and notes to our Consolidated Financial Statements and "Management's Discussion and Analysis of Financial Condition and Results of Operations" contained elsewhere in this Annual Report. All per-share data has been retrospectively adjusted to give effect to the



Consolidation discussed in Part I, Item 1 - " *Business - Recent Corporate and Securities Transactions* " section of this Annual Report. Historical results are not necessarily indicative of future results.

	Year Ended December 31,				
	2017	2016 <sup>(1)</sup>	2015 <sup>(2)</sup>	2014 <sup>(3)</sup>	2013 <sup>(5)</sup>
(in thousands, except per share amounts)					
<b>Consolidated statements of operations data:</b>					
Net revenues	\$ 138,438	\$ 13,574	\$ —	\$ —	\$ —
Cost of product sales	77,220	5,971	—	—	—
Selling, general and administrative expenses	96,472	29,525	16,222	17,682	6,986
Research and development expenses	49,008	14,784	9,790	13,803	18,509
Loss from operations	(86,798)	(36,706)	(23,345)	(3,829)	(28,490)
Net loss	(126,710)	(52,870)	(23,009)	(4,071)	(24,871)
Basic and diluted net (loss) income per common share					
Continuing operations	\$ (6.81)	\$ (4.69)	\$ (2.20)	\$ (0.40)	\$ (2.55)
Discontinued operations	—	—	—	—	0.10
Net loss per common share - basic and diluted	\$ (6.81)	\$ (4.69)	\$ (2.20)	\$ (0.40)	\$ (2.45)

	As of December 31,				
	2017	2016 <sup>(1)</sup>	2015 <sup>(2)</sup>	2014 <sup>(3)</sup>	2013 <sup>(4)(5)</sup>
(in thousands)					
<b>Consolidated balance sheets data:</b>					
Cash and cash equivalents	\$ 55,430	\$ 108,927	\$ 141,824	\$ 155,908	\$ 118,521
Total assets	369,322	480,782	145,166	160,371	163,867
Working capital	40,709	47,337	139,253	153,900	157,587
Convertible notes, net	258,538	225,584	—	—	—
Accumulated deficit	(713,974)	(587,208)	(534,338)	(511,329)	(507,258)
Total shareholders' equity	14,938	135,787	141,341	156,512	157,784

- (1) On November 29, 2016, we completed the Merger and our financial position after that date includes Aegerion's financial position. Our results of operations for 2016 included Aegerion's financial results from November 29, 2016 to December 31, 2016.
- (2) On September 15, 2015, the InSite Merger Agreement was terminated after InSite's board of directors notified QLT that they had reviewed a second unsolicited offer from Sun Pharmaceuticals Industries Ltd. ("Sun") and determined that it was superior to the proposed InSite Merger with QLT. As a result, InSite notified QLT that it was exercising its right to terminate the InSite Merger Agreement in order to enter into an agreement with Sun, and InSite paid QLT a termination fee of \$2.7 million. During the year ended December 31, 2015, QLT incurred consulting and transaction fees of \$9.4 million in connection with the pursuit of the InSite Merger and the strategic transactions as described in Note 4 - *Terminated Merger Transaction* in the Notes to Consolidated Financial Statements.
- (3) On October 8, 2014, the Auxilium Merger Agreement among QLT, Auxilium, HoldCo, and AcquireCo, terminated after Auxilium delivered a notice of termination to QLT informing QLT that Auxilium's board of directors had determined that the Endo Proposal was a superior proposal under the terms of the Auxilium Merger Agreement. On October 9, 2014 Auxilium paid QLT a termination fee of \$28.4 million. On October 22, 2014, pursuant to the terms of the financial advisory services agreement with Credit Suisse, QLT paid Credit Suisse a fee of \$5.7 million in connection with the termination of the Auxilium Merger Agreement. During the year ended December 31, 2014, QLT incurred consulting and transaction fees of \$10.2 million in connection with the pursuit of the Auxilium Merger.
- (4) On June 27, 2013, we completed a \$200.0 million special cash distribution, by way of a reduction of the paid-up capital of the Company's common shares (the "Cash Distribution"). The Cash Distribution was approved by the Company's shareholders at QLT's annual and special shareholders' meeting on June 14, 2013. All shareholders of record as of June

24, 2013 (the "Record Date") were eligible to participate in the Cash Distribution and received a payment of approximately \$3.92 per share based upon the 51,081,878 common shares issued and outstanding on the Record Date.

- (5) On April 3, 2013, we completed the sale of our punctal plug drug delivery system technology to Mati pursuant to an asset purchase agreement. During the year ended December 31, 2013, we recognized \$1.1 million gain from discontinued operations, which represented \$1.3 million of sale proceeds, net of the \$0.2 million carrying value of certain equipment sold and a negligible amount of other transaction fees.

## Item 7. Management's Discussion and Analysis of Financial Condition and Results of Operations.

### Cautionary Statement Concerning Forward-Looking Statements

All statements included or incorporated by reference into this Annual Report on Form 10-K ("Annual Report"), other than statements of historical fact, are "forward-looking statements" under, and are made pursuant to the safe harbor provisions of, the Private Securities Litigation Reform Act of 1995 and other applicable federal U.S. and Canadian laws, regulations and other legal principles. Forward-looking statements and information are often identified by words such as "anticipates," "expects," "intends," "plans," "predicts," "believes," "seeks," "estimates," "forecasts," "may," "will," "should," "would," "could," "potential," "guidance," "continue," "ongoing" and similar expressions, and variations or negatives of these words.

Examples of forward-looking statements and information include our statements regarding: the commercial potential for, and market acceptance of, our products; our estimates as to the potential number of patients with the diseases for which our products are approved or for which our product candidates are being developed; our expectations with respect to reimbursement of our products in the U.S. and elsewhere; our expectations with respect to named patient sales of our products in Brazil and in other countries where such sales are permitted; the potential for and possible timing of approval of our products in countries or regions where we have not yet obtained approval; our plans for further clinical development of our products; our efforts to out-license zuretinol; our expectations regarding future regulatory filings and interactions with regulatory agencies for our products, including potential marketing approval applications with respect to metreleptin to expand the indication for metreleptin in the U.S. and with respect to potential approval of the marketing authorization application for metreleptin in the European Union ("EU"); our plans for commercial marketing, sales, manufacturing and distribution of our products; our expectations with respect to the impact of competition on our future operations and results; our beliefs with respect to our intellectual property portfolio for our products and the extent to which it allows us to exclusively develop and commercialize our products and product candidates; our expectations regarding the availability of data and marketing exclusivity for our products in the U.S., the EU, Japan and other countries; our expectations regarding our ability to comply with Aegerion's settlement of the Department of Justice (the "DOJ") and SEC investigations, including the payment of the penalties, restitution, and settlement amounts and the obligations contained in the settlement agreements and resulting from criminal probation; our beliefs that the DOJ and SEC investigations and the settlement could give rise to additional third party demands, claims or litigation, further investigations, or could impact Aegerion's commercial operations, research and development activities, contracts and business; the final outcomes of investigations in Brazil, and the possible impact and additional consequences of them on our business and the other factors that are significantly impacting named patient sales in Brazil; our expectations regarding the impact on U.S. sales and patient attrition of JUXTAPID as a result of the modified JUXTAPID Risk Evaluation and Mitigation Strategy ("REMS") program; the anticipated results of our recent workforce reduction and other cost control measures; our future expectation of cash use and ability and plans to restructure Aegerion's convertible debt; our expectations regarding our global consolidated tax structure and planning, our ability to achieve tax savings or utilize net operating loss carryforwards and other tax and tax planning activities, including whether we are characterized as a U.S. domestic corporation for U.S. federal income tax purposes; our forecasts regarding sales of our products, our future expenses, our cash position and the timing of any future need for additional capital to fund operations and product development opportunities; and our ability to manufacture and supply sufficient amounts of our products to meet demand for commercial and clinical supplies.

The forward-looking statements contained in this Annual Report and in the documents incorporated into this Annual Report by reference are based on our current beliefs and assumptions with respect to future events, all of which are subject to change. Forward-looking statements are based on estimates and assumptions regarding, for example, our financial position and execution of our business strategy, resolution of litigation and investigations, future competitive conditions and market acceptance of products, the possibility and timing of future regulatory approvals, expectations regarding our core capabilities, the ability to restructure Aegerion's convertible debt, and the availability of sufficient liquidity, each made in light of current conditions and expected future developments, as well as other factors that we believe are appropriate in the circumstances. Forward-looking statements are not guarantees of future performance, and are subject to risks, uncertainties and assumptions that are difficult to predict, and should be considered in light of the factors discussed in the "Risk Factors" section of and elsewhere in this Annual Report. It is not possible for us to predict all risks, nor can we assess the impact of all factors on our business or the extent to which any factor, or combination of factors may impact our operations or results. New risks may emerge from time to time. Past financial or operating performance is not necessarily a reliable indicator of future performance. Given these risks and uncertainties, we can give no assurances that any of the events anticipated by the forward-looking statements will occur or, if any of them does occur, what impact such event will have on our results of operations and financial condition. Our actual results could differ materially and adversely from those expressed in any forward-looking statement in this Annual Report or in our other filings with the SEC.

This Annual Report also contains "forward-looking information" that constitutes "financial outlooks" within the meaning of applicable Canadian securities laws. This information is provided to give investors general guidance on management's current

expectations of certain factors affecting our business, including our financial results. Given the uncertainties, assumptions and risk factors associated with this type of information, including those described above, investors are cautioned that the information may not be appropriate for other purposes.

Except as required by law, we undertake no obligation to revise our forward-looking statements to reflect events or circumstances that arise after the date of this Annual Report or the respective dates of documents incorporated into this Annual Report by reference that include forward-looking statements. Therefore, you should not assume that our silence over time means that actual events are bearing out as expressed or implied in these forward-looking statements.

## Business Overview

We are a biopharmaceutical company dedicated to developing new standards of care for individuals living with rare diseases. We, through Aegerion, have two commercial products:

- Lomitapide is marketed in the United States ("U.S.") under the brand name JUXTAPID (lomitapide) capsules ("JUXTAPID"). JUXTAPID is approved in the U.S. as an adjunct to a low-fat diet and other lipid-lowering treatments, including low-density lipoprotein ("LDL") apheresis where available, to reduce low-density lipoprotein cholesterol ("LDL-C"), total cholesterol ("TC"), apolipoprotein B ("apo B") and non-high-density lipoprotein cholesterol ("non-HDL-C") in adult patients with homozygous familial hypercholesterolemia ("HoFH"). Lomitapide is approved in the EU, under the brand name LOJUXTA (lomitapide) hard capsules ("LOJUXTA") for the treatment of adult patients with HoFH, as well as in Japan, Canada, and a limited number of other countries. In December 2016, Aegerion launched JUXTAPID as a treatment for HoFH in Japan. Aegerion receives sales milestones and royalties on net sales of LOJUXTA in the EU and certain other jurisdictions from Amryt Pharma plc ("Amryt"), to whom Aegerion out-licensed the rights to commercialize LOJUXTA in those jurisdictions in December 2016. Lomitapide is also sold, on a named patient sales basis, in Brazil and in a limited number of other countries outside the U.S. where such sales are permitted before regulatory approval in such country as a result of the approval of lomitapide in the U.S. or the EU. We plan to file for regulatory approval for lomitapide for the treatment of HoFH in Brazil in 2018.
- Metreleptin, a recombinant analog of human leptin, is marketed in the U.S. under the brand name MYALEPT (metreleptin) for injection ("MYALEPT"). MYALEPT is approved in the U.S. as an adjunct to diet as replacement therapy to treat the complications of leptin deficiency in patients with congenital or acquired generalized lipodystrophy ("GL"). In December 2016, we submitted a marketing authorization application ("MAA") to the European Medicines Agency ("EMA") to seek approval for metreleptin in the EU, under the brand name MYALEPTA, as replacement therapy to treat complications of leptin deficiency in patients with GL and in a subset of patients with partial lipodystrophy ("PL"). In February 2018, we presented at an oral hearing of the EMA's Committee for Medicinal Products for Human Use ("CHMP"). Based on the CHMP's feedback at and after the hearing, we have requested a two-month "clock stop" of the CHMP's review to allow for the preparation of responses and additional data focused, primarily, on the PL patient subset. Subject to our ability to address the CHMP's feedback during the "clock stop" period, we anticipate that the CHMP will issue its opinion in the second quarter of 2018 and the European Commission ("EC") will issue its decision in mid-2018. We plan to file for regulatory approvals for metreleptin in GL and, subject largely to whether we receive EMA approval of any PL subset indication, such PL subset in other key markets, including Brazil in late 2018. We offer metreleptin through expanded access programs in countries where permitted by applicable regulatory authorities and under applicable laws, and generate revenues in certain markets where named patient sales are permitted based on the approval of metreleptin in the U.S. We plan to initiate, by late 2018, a phase 2 trial assessing metreleptin in hypoleptinemic metabolic disorder ("HMD"), a low leptin mediated metabolic disease, subject to approval of our protocol and statistical plan by applicable regulatory authorities. We also plan to continue to explore new opportunities for metreleptin to treat certain other low-leptin mediated metabolic diseases, and are reviewing options for raising capital to fund such opportunities, along with later-stage studies in HMD, upon which such opportunities are largely dependent.

We also have one product candidate, zuretinol acetate ("zuretinol"), an oral synthetic retinoid in development for the treatment of inherited retinal disease ("IRD") caused by underlying mutations in retinal pigment epithelium protein 65 ("RPE65") and lecithin: retinol acyltransferase ("LRAT") genes, comprising Leber Congenital Amaurosis ("LCA") and Retinitis Pigmentosa ("RP"). Following a comprehensive pipeline review, we are evaluating options for out-licensing zuretinol.

During the year ended December 31, 2017, net revenues from sales of lomitapide and metreleptin were \$138.4 million, of which \$97.4 million was derived from prescriptions for lomitapide and metreleptin written in the U.S., and \$41.0 million was derived from prescriptions written for and royalties on sales of lomitapide and metreleptin outside the U.S. As of December 31, 2017, we had approximately \$55.4 million in cash and cash equivalents. We have approximately \$325.0 million principal amount of 2.0% convertible senior notes due August 15, 2019 (the "Convertible Notes"). To resolve certain DOJ and SEC investigations

regarding Aegerion's U.S. commercial activities and disclosures related to JUXTAPID, Aegerion is, among other obligations, required to pay approximately \$40.1 million in civil penalties, restitution and settlement amounts (plus interest) over three years. See the " *Management's Discussion and Analysis of Financial Condition and Results of Operations - Liquidity and Capital Resources* " section and the " *Legal Proceedings* " section of this Annual Report for further information.

In the near-term, we expect that the majority of revenues will continue to be derived from sales of JUXTAPID and MYALEPT in the U.S. We also expect to generate revenues from (i) sales in those countries outside the U.S. in which we have or expect to receive marketing approval, are able to obtain pricing and reimbursement approval at acceptable levels, and elect to commercialize the products, and (ii) sales of both products in a limited number of other countries where they are, or may in the future be, available on a named patient sales basis as a result of approvals in the U.S. or EU. We expect that in the near-term, our largest sources of revenues after the U.S., on a country-by-country basis, will be sales of JUXTAPID in Japan and named patient sales of both of our products in Brazil. We have had, and expect to continue to have, named patient sales of metreleptin in Brazil, Colombia, Argentina, and a select number of other key markets, including France and Turkey. We expect net revenues from named patient sales to fluctuate significantly quarter-over-quarter given that named patient sales are derived from unsolicited requests from prescribers. In some countries, including Brazil, orders for named patient sales are for multiple months of therapy, which can lead to fluctuation in sales depending on the ordering pattern.

We expect that our near-term efforts will be focused on the following:

- continuing to sell JUXTAPID as a last-line treatment for adult HoFH patients in the U.S. despite the availability of PCSK9 inhibitor products, which have had a significant adverse impact on sales of JUXTAPID, and gaining market acceptance in the other countries, including Japan, where lomitapide is approved and being commercialized, or may in the future receive approval and be commercialized;
- reviewing our holding and capital structure with a view towards optimizing our assets and improving our balance sheet;
- managing our costs and expenses to better align with our revenues, while supporting approved products in a compliant manner;
- continuing to support patient access to and reimbursement for our products in the U.S. without significant restrictions, particularly given the availability of PCSK9 inhibitor products in the U.S., which has adversely impacted reimbursement of JUXTAPID, and given the considerable number of eligible JUXTAPID patients in the U.S. who are on Medicare Part D and the significant percentage of such patients who may not be able to afford their out-of-pocket co-payments for our products, given that prior sources of financial support for some of the patients through patient assistance programs operated by independent charitable 501(c)(3) organizations may no longer be available;
- continuing to support sales of lomitapide as a treatment for HoFH in Brazil on a named patient sales basis, particularly in light of local economic challenges, ongoing government investigations, an ongoing court proceeding reviewing the regulatory framework for named patient sales in Brazil, and recently implemented regulatory requirements for named patient sales which have added complexity to the process for named patient sales in Brazil and we believe have led a significant number of patients to discontinue therapy with lomitapide, and continuing to support named patient sales in other key countries where such sales are permitted, despite the availability of PCSK9 inhibitors on a named patient sales basis in such countries;
- initiating clinical development of metreleptin in HMD, and exploring potential new opportunities for metreleptin in additional indications, including certain other low-leptin mediated metabolic diseases, assuming we raise capital to fund such opportunities;
- building and maintaining market acceptance for MYALEPT in the U.S. for the treatment of complications of leptin deficiency in GL patients, and supporting named patient sales of metreleptin in GL in Brazil, particularly in light of local economic challenges, ongoing government investigations, an ongoing court proceeding reviewing the regulatory framework for named patient sales in Brazil, and recently implemented regulatory requirements for named patient sales which have added complexity to the process for named patient sales in Brazil and we believe have led a significant number of patients to discontinue therapy with metreleptin, and supporting such sales in other key countries, including Turkey and France, where such sales are permitted;
- gaining regulatory, pricing and reimbursement approvals to market our products in countries in which the products are not currently approved and/or reimbursed or for new indications, including obtaining approval of the MAA seeking marketing approval of metreleptin in the EU as a treatment for complications of leptin deficiency in GL patients and a

subset of PL, and seeking approval of metreleptin in Brazil and other key markets as a treatment for complications of leptin deficiency in GL patients, and subject largely to whether we receive EMA approval of any PL subset indication, PL subset patients;

- preparing for the launch of metreleptin in Europe as a treatment for complications of leptin deficiency in GL patients and a subset of PL, in the event we obtain regulatory, pricing and reimbursement approvals in the EU for metreleptin;
- minimizing the number of patients who are eligible to receive but decide not to commence treatment with our products, or who discontinue treatment, by supporting activities such as patient support programs, to the extent permitted in a particular country;
- Aegerion complying with the various agreements and judgments entered into with the DOJ and SEC in September 2017, and subsequently, and Aegerion's criminal sentencing in January 2018, including the payment of approximately \$40.1 million in civil penalties, restitution and settlement amount (plus interest) over three years, and three-years of criminal probation, a civil settlement agreement with the DOJ, separate civil settlement agreements with multiple U.S. states, a final judgment entered in connection with a complaint filed by the SEC, a three-year deferred prosecution agreement with the DOJ (the "DPA"), a five-year corporate integrity agreement with the Office of Inspector General of the Department of Health and Human Services (the "CIA"), and a pending civil consent decree with the U.S. Food and Drug Administration ("FDA") and the DOJ relating to the JUXTAPID Risk Evaluation and Mitigation Strategy ("REMS") program, and managing other ongoing government investigations and related matters pertaining to its products;
- engaging in possible further development efforts related to our existing products, and assessing, and possibly acquiring, potential new product candidates targeted at rare diseases where we believe we can leverage our infrastructure and expertise;
- continuing to reinforce a culture of compliance, ethics and integrity throughout Novilion, Aegerion and their subsidiaries; and
- defending challenges to the patents or our claims of exclusivity for our products in the U.S., including against potential generic submissions with the FDA with respect to lomitapide, and expanding the intellectual property portfolio for our products.

### **Investigations and Legal Proceedings**

As noted above, Aegerion has been the subject of certain investigations and other legal proceedings (some of which remain ongoing), including investigations of Aegerion's marketing and sales activities of JUXTAPID by the DOJ and the SEC, an investigation by federal and state authorities in Brazil to determine whether there have been violations of Brazilian laws related to sales of JUXTAPID, and a putative class action lawsuit alleging certain misstatements and omissions related to the marketing of JUXTAPID and the Company's financial performance in violation of the federal securities laws (the "Class Action Litigation"). Aegerion entered into agreements (the "Settlement") with the DOJ and the SEC in September 2017 that required Aegerion, in addition to paying certain penalties and Settlement amounts, to plead guilty to two misdemeanor misbranding violations of the Food, Drug and Cosmetics Act and to enter into a three-year DPA with regard to a charge that it engaged in a conspiracy to violate the Health Insurance Portability and Accountability Act ("HIPAA"). Aegerion was sentenced by the U.S. District Court on January 30, 2018 after the judge accepted Aegerion's guilty criminal plea. Under the terms of the Settlement, including the sentence, Aegerion is required to pay approximately \$40.1 million in aggregate penalties, plus interest, over three years, including \$7.2 million of restitution, a civil penalty of \$4.1 million to be paid to the SEC pursuant to an SEC Judgment, and \$28.8 million (including \$2.7 million designated for certain states), to be paid pursuant to the Civil Settlement Agreement, which is a significant financial burden given Aegerion's financial condition. Aegerion made an initial payment to the DOJ on February 12, 2018, and an initial payment to certain states on February 15, 2018. On February 20, 2018, the DOJ filed a stipulation of dismissal with respect to Aegerion in the civil qui tam matter. The FDA Consent Decree remains subject to approval by a U.S. District Court Judge. Aegerion also settled the Class Action Litigation for \$22.3 million. The insurance carriers agreed to cover \$22.0 million of this amount, with Aegerion responsible for the remainder of \$0.3 million. See Part I, Item 3 - "*Legal Proceedings*" for further information regarding these investigations and legal proceedings.

### **Recent Corporate and Securities Transactions**

*New Loan Agreement.* On March 15, 2018, Aegerion entered into a loan and security agreement (the "New Loan Agreement") with affiliates of Broadfin Capital, LLC ("Broadfin Capital") and Sarissa Capital Management LP ("Sarissa Capital" and, together with Broadfin Capital, the "Lenders"), pursuant to which the Lenders have made a single-draw term loan to Aegerion

in an aggregate amount of \$20 million, secured by substantially all of Aegerion's assets (the "New Loan"), including a pledge of 66% of its first-tier foreign subsidiaries' equity interests, and substantially all of the intellectual property and related rights in respect of MYALEPT and JXTAPID, subject to certain exceptions. Interest on the New Loan accrues at 9.0% per annum. The term loan made pursuant to the New Loan Agreement matures on the earliest of (i) August 1, 2019, (ii) 30 days prior to the maturity date of the Convertible Notes, (iii) the date that any restructuring or recapitalization of all or substantially all of the Convertible Notes, including any exchange offer or similar transaction, is substantially consummated, and (iv) upon acceleration of the obligations under the New Loan Agreement. Concurrently with the execution of the New Loan Agreement, Novilion, Aegerion and the Lenders entered into a subordination agreement to subordinate the New Loan to the obligations of Aegerion to Novilion under the Amended and Restated Senior Loan Agreement (described in the Part I, Item 1 - "Business - Recent Corporate and Securities Transactions" section of this Annual Report).

In connection with the New Loan Agreement, the Lenders were issued warrants to purchase 1,818,592 Novilion common shares. The warrants have an exercise price equal to \$4.40 per share, representing the volume weighted average price of Novilion common shares for the 20 trading days ending March 14, 2018, and have a term of four years.

*Merger Transaction with Aegerion.* On June 14, 2016, we entered into an Agreement and Plan of Merger (as amended, the "Merger Agreement") with Aegerion, pursuant to which on November 29, 2016 our indirect wholly-owned subsidiary, Isotope Acquisition Corp., merged with and into Aegerion, with Aegerion surviving as our wholly-owned subsidiary (the "Merger"). Upon completion of the Merger, on November 29, 2016, each outstanding share of Aegerion common stock was converted into a right to receive 1.0256 Novilion (pre-Consolidation) common shares and Aegerion's common stock was cancelled and delisted from the NASDAQ.

Pursuant to the Merger Agreement, we also issued certain warrants to the pre-closing shareholders of Novilion. These warrants (the "Merger Agreement Warrants") were exercisable for up to an aggregate of 11,301,791 Novilion common shares at an exercise price of \$0.05 per share if Aegerion's disclosed DOJ and SEC investigations or Aegerion's Class Action Litigation were settled for amounts in excess of a certain negotiated threshold. Given that the settlements of these matters did not exceed the negotiated threshold, the Merger Agreement Warrants have been cancelled subsequent to December 31, 2017 and will not be exercisable for any shares. Refer to Note 13 - *Share Capital* in the Notes to Consolidated Financial Statements included in this Annual Report for further details.

The aggregate consideration delivered to the former holders of Aegerion common stock in connection with the Merger was approximately 6,060,288 Novilion common shares. Shareholders of Novilion immediately prior to the Merger, including the participants in the private placement pursuant to the Unit Subscription Agreement (described below), owned approximately 68% of the outstanding Novilion common shares upon completion of the Merger and stockholders of Aegerion immediately prior to the Merger owned approximately 32% of the outstanding Novilion common shares upon completion of the Merger.

*Private Placement.* Also on June 14, 2016, we entered into a unit subscription agreement (the "Unit Subscription Agreement") with the investors party thereto (the "Investors"). Pursuant to the Unit Subscription Agreement, immediately prior to the Merger, the Investors acquired units, for \$8.80 per unit, on a post-Consolidation basis, consisting of (i) 2,472,727 Novilion common shares, which includes up to 568,181 Novilion common shares issuable upon exercise of fully paid-up warrants, and (ii) warrants (the "Unit Subscription Agreement Warrants") which were exercisable for up to an aggregate of 2,644,952 Novilion common shares at an exercise price of \$0.05 per share on the same terms and conditions as the Merger Agreement Warrants (collectively with the Merger Agreement Warrants, the "Warrants"). As with the Merger Agreement Warrants, these warrants have been cancelled subsequent to December 31, 2017 because the settlements of the matters described above did not exceed the negotiated threshold. The aggregate consideration paid under the Unit Subscription Agreement was approximately \$21.8 million.

*Share Consolidation.* On December 16, 2016, we completed a one-for-five (1:5) consolidation (the "Consolidation") of all of our issued and outstanding common shares, without par value, for shareholders of record as of December 16, 2016, resulting in a reduction in the issued and outstanding common shares from 92,653,562 to 18,530,323 as of that date. Each shareholder's percentage ownership in Novilion and proportional voting power remained unchanged after the Consolidation, except for minor changes resulting from the treatment of fractional shares. In connection with the Consolidation, the conversion rate of the Convertible Notes was automatically adjusted from 24.9083 common shares per \$1,000 principal amount of such Convertible Notes to 4.9817 common shares per \$1,000 principal amount of such Convertible Notes.

*Aralez Investment and Distribution.* On December 7, 2015, we entered into an Amended and Restated Share Subscription Agreement (the "Amended and Restated Subscription Agreement") with Tribute Pharmaceuticals Canada Inc. ("Tribute"), POZEN Inc. ("POZEN"), Aralez Pharmaceuticals plc, (formally known as "Aguono Limited") ("Aralez Ireland"), Aralez Pharmaceuticals Inc. ("Aralez Canada"), Deerfield Private Design Fund II, L.P., Deerfield International Master Fund, L.P., Deerfield Partners, L.P. (together "Deerfield"), Broadfin Capital, LLC ("Broadfin") and JW Partners, LP, JW Opportunities Fund, LLC and J.W.

Opportunities Master Fund, Ltd. (together the "JW Parties") (Deerfield, Broadfin and the JW Parties are referred to herein collectively as the "Co-Investors"). The Amended and Restated Subscription Agreement amended and restated a share subscription agreement entered into on June 8, 2015 among the Company, Tribute, POZEN, Aralez Ireland, the Co-Investors and certain other investors. Pursuant to the Amended and Restated Subscription Agreement, immediately prior to and contingent upon the consummation of the merger of Tribute and POZEN (the "Aralez Merger"), Tribute agreed to sell to us and the other Co-Investors \$75.0 million of the common shares of Tribute (the "Tribute Shares") in a private placement (the "Aralez Investment") at a purchase price per share equal to: (a) the lesser of (i) \$7.20, and (ii) a five percent discount off of the five-day volume weighted average price per share of POZEN common stock calculated over the five trading days immediately preceding the date of closing of the Aralez Merger, not to be less than \$6.25 per share; multiplied by (b) the Aralez Merger exchange ratio of 0.1455. Upon consummation of the Aralez Merger on February 5, 2016, the Tribute Shares were exchanged for common shares of Aralez Canada (the "Aralez Shares"). We entered into the transaction contemplated by the Amended and Restated Subscription Agreement for the purpose of returning capital to our shareholders pursuant to a special election distribution, payable, at the election of each shareholder of the Company, in either Aralez Shares (approximately 0.13629 of an Aralez Share for each common share of the Company) or cash, subject to pro-ration (the "Aralez Distribution"), up to a maximum of \$15.0 million funded pursuant to the terms of the Backstop Agreement (as described below).

In connection with the Aralez Distribution, on June 8, 2015, we entered into a share purchase agreement (as amended, the "Backstop Agreement") with Broadfin and the JW Parties, pursuant to which Broadfin and the JW Parties agreed to purchase up to \$15.0 million of the Aralez Shares from us at \$6.25 per share. This arrangement provided our shareholders with the opportunity to elect to receive, in lieu of Aralez Shares, up to an aggregate of \$15.0 million in cash, subject to proration among the shareholders. As a result, on April 5, 2016 (the "Distribution Date"), we distributed 4,799,619 Aralez Shares, with a fair value of \$19.3 million, and \$15.0 million of cash.

Upon consummation of the Aralez Merger on February 5, 2016, we purchased 7,200,000 Aralez Shares (representing 10.1% of the issued and outstanding Aralez Shares), for an aggregate price of \$45.0 million. We held the Aralez Shares from February 5, 2016 to the Distribution Date and the Aralez Shares were marked-to-market. As a result, we recognized a \$10.7 million loss during the fiscal year ended December 31, 2016, to reflect the change in value from the acquisition date to the Distribution Date.

*Terminated Merger Transaction.* On June 8, 2015, QLT entered into an Agreement and Plan of Merger (as amended and restated on each of July 16, 2015 and August 26, 2015) (the "InSite Merger Agreement") with InSite Vision Incorporated, a Delaware corporation ("InSite"). On September 15, 2015, the InSite Merger Agreement was terminated by InSite and InSite paid QLT a termination fee of \$2.7 million. Refer to Note 4 - *Terminated Merger Transaction* in the Notes to Consolidated Financial Statements included in this Annual Report for further details.

## Financial Overview

- Net revenues were \$138.4 million for the year ended December 31, 2017, representing revenues from sales of lomitapide and metreleptin.
- Cost of product sales was \$77.2 million for the year ended December 31, 2017, representing costs of selling lomitapide and metreleptin, as well as an \$18.8 million charge towards the reserves recorded for excess and obsolete inventory, which are derived from projected sales activities, respective product shelf-life and their respective fair values.
- Selling, general and administrative ("SG&A") expenses increased from \$29.5 million in 2016 to \$96.5 million in 2017. This increase was primarily due to our recognition of a full year of Aegerion's SG&A expenses in the year ended December 31, 2017, compared to approximately five weeks of such activities in 2016 following the Merger on November 29, 2016, partially offset by a one-time \$8.0 million advisory fee incurred in 2016 in connection with the completion of the Aralez Investment and the Merger.
- Research and development ("R&D") expenses increased from \$14.8 million in 2016 to \$49.0 million in 2017. This increase was primarily driven by our recognition of a full year of Aegerion's R&D expenses in the year ended December 31, 2017, partially offset by the lower spending on Novelson clinical activities.
- We used \$54.9 million of net cash for operating activities in 2017, primarily due to a \$126.7 million net loss, \$6.9 million in nonrecurring payments associated with the Merger, and \$11.0 million used in cash related to the changes in other assets and liabilities, offset by non-cash expenses of \$82.8 million. Cash and cash equivalents totaled approximately \$55.4 million as of December 31, 2017.



## **Critical Accounting Policies and Estimates**

Our management's discussion and analysis of our financial condition and results of operations are based on our consolidated financial statements, which have been prepared in accordance with accounting principles generally accepted in the U.S. ("GAAP"). The preparation of these consolidated financial statements requires us to make estimates and judgments that affect the reported amounts of assets, liabilities, revenues and expenses. On an ongoing basis, we evaluate these estimates and judgments, including those described below. We base our estimates on historical experience and on various other assumptions that we believe to be reasonable under the circumstances. These estimates and assumptions form the basis for making judgments about the carrying values of assets and liabilities that are not readily apparent from other sources. Actual results and experiences may differ materially from these estimates.

While our significant accounting policies are more fully described in Note 2 - *Summary of Significant Accounting Policies* in the Notes to Consolidated Financial Statements appearing in the " *Consolidated Financial Statements and Supplementary Data* " section of this Annual Report, we believe that the accounting policy related to revenue recognition is the most critical to aid you in fully understanding and evaluating our reported financial results, and affecting the more significant judgments and estimates that we use in the preparation of our consolidated financial statements.

### ***Revenue Recognition***

The Company recognizes revenue from product sales when there is persuasive evidence that an arrangement exists, title to product and associated risk of loss has passed to the customer, the price is fixed or determinable, collectability is reasonably assured and the Company has no further performance obligations.

The Company records distribution and other fees paid to its distributors as a reduction of revenue. Revenue is recorded net of estimated discounts and rebates, primarily including those provided to Medicare and Medicaid. Allowances are recorded as a reduction of revenue at the time revenues from product sales are recognized. Allowances for government rebates and discounts are established based on the actual payer information, which is reasonably estimable at the time of delivery. These allowances are adjusted to reflect known changes in the factors that may impact such allowances in the quarter those changes are known. To date, such adjustments have not been significant.

We receive royalty revenues on sales by our licensees. We record these revenues based on estimates of the sales occurred during the relevant period and the applicable royalty rate. Differences between actual and estimated royalty revenues are adjusted for in the period in which they become known, typically the following quarter. Historically, adjustments have not been material when compared to actual amounts paid by licensees.

### **Recently Issued and Recently Adopted Accounting Standards**

See Note 2 - *Summary of Significant Accounting Policies* in the Notes to Consolidated Financial Statements for a discussion of recently adopted and new accounting pronouncements.

## **Results of Operations**

### ***Comparison of the Years Ended December 31, 2017 and 2016***

The following table summarizes the results of our operations for each of the years ended December 31, 2017 and 2016 , together with the changes in those items in dollars and as a percentage. Results of operations for 2016 include Aegerion's results of operations from November 29, 2016 through December 31, 2016, whereas results of operations for 2017 include Aegerion's results of operations for the full year.

	Year Ended December 31,		\$ Change	% Change
	2017	2016		
	(in thousands)			
Net revenues	\$ 138,438	\$ 13,574	\$ 124,864	920 %
Cost of product sales	77,220	5,971	71,249	NM
Operating expenses:				
Selling, general and administrative	96,472	29,525	66,947	227 %
Research and development	49,008	14,784	34,224	231 %
Restructuring charges	2,536	—	2,536	N/A
Total operating expenses	148,016	44,309	103,707	234 %
Loss from operations	(86,798)	(36,706)	50,092	136 %
Interest expense, net	(39,037)	(2,960)	36,077	NM
Fair value loss on investment	—	(10,740)	(10,740)	(100)%
Other (expense) income, net	(292)	(1,999)	(1,707)	(85)%
Loss before provision for income taxes	(126,127)	(52,405)	73,722	141 %
Provision for income taxes	(583)	(465)	118	25 %
Net loss	\$ (126,710)	\$ (52,870)	\$ 73,840	140 %

N/A - Not applicable

NM - Not meaningful

#### *Net Revenues*

	Year Ended December 31,	
	2017	2016
	(in thousands)	
Lomitapide	\$ 72,130	\$ 8,621
Metreleptin	66,308	4,953
Total net revenues	\$ 138,438	\$ 13,574

Revenues reported for 2017 and 2016 represented net product sales from sales of lomitapide and metreleptin, and royalties from sales of lomitapide and metreleptin made by our sublicensees in the EU and other territories. Net revenues in 2017 include a full year of activities compared to approximately five weeks of sales activities in 2016, commencing on November 29, 2016, which was the date of the Merger.

We expect that in the near-term, our largest sources of revenues after the U.S., on a country-by-country basis, will be from sales of JUXTAPID in Japan and named patient sales of both of our products in Brazil. We have had, and expect to continue to have, named patient sales of metreleptin in Brazil, Colombia, Argentina, and a select number of other key markets, including France and Turkey. We expect net revenues from named patient sales to fluctuate significantly quarter-over-quarter given that named patient sales are derived from unsolicited requests from prescribers. In some countries, including Brazil, orders for named patient sales are for multiple months of therapy, which can lead to fluctuation in sales depending on the ordering pattern.

#### *Lomitapide*

We generated revenues from lomitapide of approximately \$72.1 million for the year ended December 31, 2017. This amount includes \$2.2 million related to royalty income on sales of LOJUXTA. Revenues were primarily generated from sales in the U.S., as well as in Japan, and on a named patient basis in Brazil and certain other foreign countries. Future revenues from lomitapide may be negatively affected by the availability of PCSK9 inhibitor products.

#### *Metreleptin*

We generated revenues from metreleptin of approximately \$66.3 million for the year ended December 31, 2017. Revenues were primarily from sales within the U.S., as well as sales made on a named patient basis in Brazil. Revenues for 2017 include

recognition of \$2.3 million in the second quarter resulting from changing revenue recognition on sales of MYALEPT within the U.S from the sell-through to the sell-in method. Under the sell-in method of revenue recognition, revenue is recognized when the product is shipped to the distributor, whereas under the sell-through method, revenue is recognized when the product is prescribed to the patient. Going forward, metreleptin revenue will be recognized under the sell-in method of revenue recognition. See Note 2 - *Summary of Significant Accounting Policies* in the Notes to Consolidated Financial Statements for more information. The future net revenues of metreleptin is highly dependent on our ability to continue to find GL patients, to continue to build market acceptance for MYALEPT in the U.S. and obtaining market authorization for MYALEPT in the EU and developing metreleptin in additional indications, which is dependent on our ability to raise capital to fund the costs of such development. In addition, we will continue to pay significant Medicaid rebates for MYALEPT, which will have a negative impact in future periods. The degree of such impact on our overall financial performance will depend on the percentage of MYALEPT patients that have Medicaid as their primary insurance coverage and the quantity of units ordered per patient.

#### *Cost of Product Sales*

We recorded cost of product sales of \$77.2 million in the year ended December 31, 2017 . Cost of product sales in the current year includes \$18.8 million reserves recorded for excess and obsolete inventory, which are derived from projected sales activities, respective product shelf-life and their respective fair values. Cost of product sales in 2016 totaled \$6.0 million and included activities for approximately five weeks commencing on November 29, 2016, the day of the Merger. Additionally, cost of product sales was also comprised of the cost of inventory sold, amortization of acquired product rights, which resulted from the acquisition of Aegerion, and estimated royalties payable related to sales of lomitapide and metreleptin. We expect cost of product sales for metreleptin to increase in 2018 and for the next several years, due primarily to an increasing time-based royalty rate on net sales of metreleptin in the U.S. payable to BMS. The time-based royalty rate to BMS ranges from mid-single digits to low double digits, increasing annually in years 2016 to 2019 from rates in the low single digits to low double digits, peaking in years 2019 to 2020 at a rate in the low double digits before decreasing in years 2022 through 2025 to rates in the high single digits to mid-single digits. The royalty obligation to BMS terminates in 2026. We also expect cost of product sales for both products to fluctuate consistently with expected changes in net revenues. To the extent our actual sales or subsequent sale forecasts decrease, we could be required to record additional inventory reserves in future periods, which could have a material negative impact on our gross margin.

#### *Selling, General and Administrative Expenses*

During the year ended December 31, 2017 , SG&A expenses increased by \$66.9 million to \$96.5 million , compared to \$29.5 million for 2016 . The increase is primarily attributable to our recognition of a full year of Aegerion's SG&A expenses in the current year, compared to approximately five weeks of selling and marketing expenses in the prior year following the closing of the Merger. SG&A expenses are primarily comprised of employee-related expenses, including stock-based compensation, as well as infrastructure, consulting, contractor services, and legal expense.

#### *Research and Development Expenses*

During the year ended December 31, 2017 , R&D expenditures were \$49.0 million compared to \$14.8 million for 2016 . The \$34.2 million increase is primarily attributable to our recognition of a full year of Aegerion's R&D expenses in the current year, compared to approximately five weeks of expenses in the prior year following the closing of the Merger. These expenses are primarily comprised of employee related expenses, including stock-based compensation, as well as contractor services and consulting expenses for the period. This increase was offset by an \$8.0 million decrease in expenses related to Novelson R&D operations due to lower spending on Novelson clinical activities in 2017.

#### *Restructuring charges*

During the year ended December 31, 2017 , we incurred \$2.5 million in restructuring charges, primarily related to the integration of the businesses subsequent to the Merger.

#### *Interest Expense, net*

Interest expense, net was \$39.0 million in the year ended December 31, 2017 , an increase of \$36.1 million compared to 2016 . Interest expense in the current year primarily relates to the Convertible Notes, for which interest is payable semi-annually in arrears on February 15 and August 15 of each year. The increase in interest expense, net is primarily attributable to our recognition of full year amortization of the debt discount and interest in the current year, compared to the amortization of the debt discount and interest incurred in the approximately five week period of 2016 following the Merger on November 29, 2016.

### *Fair Value Loss on Investment*

We recognized \$10.7 million fair value loss on investment in 2016, which represents a realized loss as a result of the mark-to-market of the Aralez shares held by QLT from February 5, 2016 to April 5, 2016. Refer to Note 5 - *Strategic Transactions* in the Notes to Consolidated Financial Statements for further details.

### *Other (expense) income, net*

Other expense, net was \$0.3 million in the year ended December 31, 2017, a decrease of \$1.7 million compared to 2016. The change was primarily due to the \$2.0 million write-off in 2016 related to the receivable of the earn-out payment due from Valeant Pharmaceuticals International, Inc.

### *Provision for Income Taxes*

Our provision for income taxes was \$0.6 million for the year ended December 31, 2017, an increase of \$0.1 million from 2016. The provision for income taxes consists primarily of current tax expense, which relates to profitable operations in foreign tax jurisdictions.

For the years ended December 31, 2017 and 2016, we considered it more likely than not that some portion of the recorded deferred tax assets will not be realized in a future period based on all available evidence. As a result of our evaluations, we concluded that there was insufficient positive evidence to overcome the more objective negative evidence related to our cumulative losses and other factors. Accordingly, for the years ended December 31, 2017 and 2016, we maintained a valuation allowance against our U.S., Canadian and Swiss deferred tax assets. The Company did not maintain a valuation allowance against its remaining foreign subsidiaries as these entities are generally profitable under the Company's transfer pricing model and those earnings are further considered permanently invested in the respective foreign jurisdictions. In future periods, we will continue to evaluate whether there is sufficient positive evidence to overcome the more objective negative evidence in determining whether we will continue to maintain a valuation allowance.

We believe that we were a passive foreign investment company ("PFIC") for the taxable years ended December 31, 2008 through 2015. Based on the price of our common shares and the composition of our assets (and those of our subsidiaries), we believe that we will not be a PFIC for U.S. federal income tax purposes for the taxable years ended December 31, 2016 and 2017. The determination of whether we are a PFIC is made annually and depends on the particular facts and circumstances (such as the valuation of our assets, including goodwill and other intangible assets) and also may be affected by the application of the PFIC rules, which are subject to differing interpretations.

On December 22, 2017, the President of the United States signed into law the Tax Cuts and Jobs Act (the "Act"). The Act includes significant changes in U.S. tax law, including a reduction in the corporate tax rates and creating a territorial tax system with a one-time mandatory tax on previously deferred foreign earnings of U.S. subsidiaries. The Act reduced the U.S. corporate tax rate from the current rate of 35% to 21% for tax years beginning after December 31, 2017. As a result of the Act, we were required to revalue its existing deferred tax assets and liabilities as of December 31, 2017 from the 35% federal rate in effect through the end of 2017, to the new 21% rate. As a result of the change in law, we recorded a current period tax expense of \$21.5 million and a corresponding reduction in the associated valuation allowance, for zero net impact to its consolidated statement of operations for the year ended December 31, 2017. The Act will require us to pay tax on the unremitted earnings of its foreign subsidiaries through December 31, 2017. We have estimated that its foreign subsidiaries are in an overall net earnings deficit and as such would have no incremental U.S. tax and therefore has recorded no tax liability on its unremitted earnings at December 31, 2017.

### ***Comparison of the Years Ended December 31, 2016 and 2015***

The following table summarizes the results of our operations for each of the years ended December 31, 2016 and 2015, together with the changes in those items in dollars and as a percentage. Results of operations for 2016 include Aegerion's results of operations from November 29, 2016 through December 31, 2016.

	Year Ended December 31,		\$ Change	% Change
	2016	2015		
	(in thousands)			
Net revenues	\$ 13,574	\$ —	\$ 13,574	N/A
Cost of product sales	5,971	—	5,971	N/A
Operating expenses:				
Selling, general and administrative	29,525	16,222	13,303	82 %
Research and development	14,784	9,790	4,994	51 %
Termination fee	—	(2,667)	(2,667)	(100)%
Total operating expenses	44,309	23,345	20,964	90 %
Loss from operations	(36,706)	(23,345)	13,361	57 %
Interest (expense) income, net	(2,960)	277	(3,237)	NM
Fair value loss on investment	(10,740)	—	10,740	N/A
Other (expense) income, net	(1,999)	81	(2,080)	NM
Loss before provision for income taxes	(52,405)	(22,987)	29,418	128 %
Provision for income taxes	(465)	(22)	443	NM
Net loss	\$ (52,870)	\$ (23,009)	\$ 29,861	130 %

N/A - Not applicable

NM - Not meaningful

#### *Net Revenues*

	Year Ended December 31,	
	2016	2015
	(in thousands)	
Lomitapide	\$ 8,621	\$ —
Metreleptin	4,953	—
Total net revenues	\$ 13,574	\$ —

Revenues reported for the year ended December 31, 2016 represented net product sales and royalties from sales of lomitapide and metreleptin from November 29, 2016 through December 31, 2016. In 2015, we did not have any commercial products and did not generate any revenues.

#### *Lomitapide*

We generated revenues from lomitapide of approximately \$8.6 million in the year ended December 31, 2016. This amount is comprised primarily of revenues generated from sales within the U.S.

#### *Metreleptin*

We generated revenues from net product sales of MYALEPT of approximately \$5.0 million for the year ended December 31, 2016. Sales generated were comprised primarily of revenues generated from sales within the U.S.

#### *Cost of Product Sales*

We recorded cost of product sales of \$6.0 million for the year ended December 31, 2016. During 2015, we did not have any net revenues, and therefore we did not recognize cost of product sales. Cost of product sales in 2016 was comprised of the cost of inventory sold, amortization of acquired product rights, which resulted from the acquisition of Aegerion, and estimated royalties payable related to sales of lomitapide and metreleptin from November 29, 2016 through December 31, 2016.

### *Selling, General and Administrative Expenses*

During the year ended December 31, 2016, SG&A expenses increased by \$13.3 million to \$29.5 million, compared to \$16.2 million for 2015. A portion of the increase was attributable to \$8.0 million in total advisory fees we paid to Greenhill for the completion of Novilion's \$45.0 million investment in Aralez and the completion of the Merger. The remaining increase was primarily due to our recognition, starting on November 29, 2016, of 100% of Aegerion's SG&A expenses which were primarily comprised of employee-related expenses, including stock-based compensation, infrastructure, and legal expense.

### *Research and Development Expenses*

During the year ended December 31, 2016, R&D expenditures were \$14.8 million compared to \$9.8 million for 2015. The \$5.0 million increase was primarily due to our recognition, starting on November 29, 2016, of 100% of Aegerion's R&D expenses. These expenses were primarily comprised of employee related expenses, including stock-based compensation, and consulting costs.

### *Termination Fees*

In 2015, we recognized termination fees totaling \$2.7 million in connection with the termination of the InSite Merger Agreement on September 15, 2015.

### *Interest Expense, net*

We recognized \$3.0 million of interest expense, net in 2016, which represents the amortization of the debt discount and interest incurred in December 2016 in relation to the issuance by Aegerion of the Convertible Notes for which interest is payable semi-annually in arrears on February 15 and August 15 of each year. Prior to the Merger, we financed our operations through equity and existing resources and did not hold any debt.

### *Fair Value Loss on Investment*

We recognized \$10.7 million fair value loss on investment in 2016, which represents a realized loss as a result of the mark-to-market of the Aralez shares held by QLT from February 5, 2016 to April 5, 2016. Refer to Note 5 - *Strategic Transactions* in the Notes to Consolidated Financial Statements for further details.

### *Other (expense) income, net*

In 2016, we wrote off \$2.0 million related to the receivable of the earn-out payment due from Valeant Pharmaceuticals International, Inc.

### *Income Taxes*

During the year ended December 31, 2016, the provision for income taxes was \$0.5 million, an increase of \$0.4 million over 2015. The provision for income taxes consisted primarily of current tax expense, which relates to our profitable operations in our foreign tax jurisdictions, offset by a current tax benefit for the reversal of interest related to our provision for UTP. During the year ended December 31, 2015, the provision for income taxes from continuing operations was insignificant and primarily relates to the accrual of interest on our provision for UTP.

For the years ended December 31, 2016 and 2015, we considered it more likely than not that some portion or all of the recorded deferred tax assets will not be realized in a future period based on all available evidence. As a result of our evaluations, we concluded that there was insufficient positive evidence to overcome the more objective negative evidence related to our cumulative losses and other factors. Accordingly, for the year ended December 31, 2015, we maintained a full valuation against all of our deferred tax assets. For the year ended December 31, 2016, we maintained a valuation allowance against our U.S., Canadian and Swiss deferred tax assets. The Company did not maintain a valuation allowance against its remaining foreign subsidiaries as these companies are generally profitable under the Company's transfer pricing model and those earnings are further considered permanently invested in the respective foreign jurisdictions. In future periods, we will continue to evaluate whether there is sufficient positive evidence to overcome the more objective negative evidence in determining whether we will continue to maintain a full valuation allowance.

## Liquidity and Capital Resources

We have historically financed our operating and capital expenditures through existing cash resources. As a result of the Merger, we now have, through Aegerion, two commercial products, lomitapide and metreleptin, which generate revenues, but we are not yet profitable. In connection with the Merger, we entered into the Unit Subscription Agreement with the Investors. The aggregate consideration received pursuant to the Unit Subscription Agreement was approximately \$21.8 million. In August 2014, Aegerion issued the Convertible Notes, for which interest is payable semi-annually in arrears on February 15 and August 15 of each year. Aegerion's ability to refinance this indebtedness, if it elects to do so, will depend on the capital markets and our financial condition on a consolidated basis.

On June 14, 2016, Aegerion entered into the Senior Loan Agreement pursuant to which Aegerion could initially borrow up to \$15.0 million used to fund Aegerion's working capital. Since the consummation of the business combination, Aegerion has continued to borrow pursuant to the terms of the Senior Loan Agreement, which has been amended from time to time, including on March 15, 2018 pursuant to the Amended and Restated Senior Loan Agreement. As of March 15, 2018, there was approximately \$38.1 million outstanding under the Amended and Restated Senior Loan Agreement, including all accrued interest paid in kind. The Senior Loan accrues interest at the rate of 8.0% per annum (which increases by 3.0% in connection with an event of default), which accrues and compounds quarterly in arrears until July 1, 2019, the maturity date of the Senior Loan. Given that the Senior Loan is an intercompany loan, it has been eliminated upon consolidation.

Also on March 15, 2018, Aegerion entered into the New Loan Agreement with the Lenders, pursuant to which the Lenders provided a single-draw term loan to Aegerion in an aggregate amount of \$20.0 million, and, like the Senior Loan, is secured by substantially all of Aegerion's assets, subordinated to the Senior Loan. Interest on the New Loan accrues at 9.0% per annum, and the New Loan matures on the earliest of (i) August 1, 2019, (ii) 30 days prior to the maturity date of the Convertible Notes, (iii) the date that any restructuring or recapitalization of all or substantially all of the Convertible Notes, including any exchange offer or similar transaction, is substantially consummated, and (iv) upon acceleration of the obligations under the New Loan Agreement. See the Part I, Item 1 - "Business - Recent Corporate and Securities Transactions" section of this Annual Report for further information regarding this term loan.

In addition, as further described above in Part I, Item 3 - "Legal Proceedings," Aegerion entered into agreements (the "Settlement") with the DOJ and the SEC in September 2017 that requires Aegerion to pay approximately \$40.1 million in civil penalties, restitution and settlement amounts (plus interest) over three years.

During the year ended December 31, 2017, we generated \$138.4 million of net revenues, but incurred of a net loss of \$ 126.7 million and used \$54.9 million of cash for operating activities. As of December 31, 2017, we had \$55.4 million in cash and cash equivalents on hand, of which, \$12.1 million, \$39.0 million and \$4.3 million of cash and cash equivalents on hand was held at our U.S., Canadian, and other foreign subsidiaries, respectively.

Going forward, we expect to fund our current and planned operating requirements principally through our existing cash resources and other potential financing methods, including the possibility of utilizing equity. As described above, on March 15, 2018, the Company closed on and received \$20.0 million in proceeds from a new loan agreement, bringing the Company's unrestricted cash balance at March 15, 2018 to approximately \$57.6 million. The Company expects to fund its current and planned operating requirements principally through its existing cash resources, and other potential financing methods, including the possibility of utilizing equity. We believe that our existing funds are sufficient to satisfy our operating needs and our working capital, milestone payments, capital expenditures, debt service requirements and legal settlement expenditures for at least the next twelve months from the issuance of the financial statements. We may, from time to time, also seek additional funding, primarily for the purpose of developing potential additional indications of metreleptin, through strategic alliances, out-licensing activities, and additional equity and/or debt financings or from other sources, should we identify strategic needs or opportunities.

For information related to certain risks that could negatively impact our financial position or future results of operations and our ability to refinance the Convertible Notes or otherwise obtain financing, see the "Risk Factors" and "Quantitative and Qualitative Disclosures About Market Risk" sections of this Annual Report.

## Cash Flows

The following table sets forth the major sources and uses of cash and cash equivalents for the periods set forth below:

	Year Ended December 31,		
	2017	2016	2015
	(in thousands)		
Net cash (used in)/provided by:			
Operating activities	\$ (54,899)	\$ (34,356)	\$ (19,359)
Investing activities	(754)	25,327	34
Financing activities	109	(23,519)	5,508
Effect of exchange rates on cash	2,047	(349)	(267)
Net decrease in cash and cash equivalents	<u>\$ (53,497)</u>	<u>\$ (32,897)</u>	<u>\$ (14,084)</u>

#### ***Comparison of the Years Ended December 31, 2017 and 2016***

Changes in net cash (used in) provided by operating activities, investing activities and financing activities for the year ended December 31, 2017 compared to 2016 were primarily attributable to our recognition of the full year cash flow activities of Aegerion, including, among others, cash generated from the net revenues of lomitapide and metrelleptin, cash used to maintain inventory of those products, and cash used to support SG&A and R&D activities compared to approximately five weeks of such activities in 2016, subsequent to the Merger. In addition, in the prior year, we had \$45.0 million in cash outflows related to financing activities for the funding of our investment in Aralez, offset by cash inflows of \$15.0 million from the sale of Aralez shares.

#### ***Cash Used in Operating Activities***

Net cash used in operating activities was \$54.9 million for the year ended December 31, 2017 compared to \$34.4 million for 2016. The \$20.4 million increase was primarily attributable to the following:

- A significant increase in the net loss recognized year over year, offset by an increase in non-cash expenses, including non-cash interest expense of \$30.3 million, the amortization of intangible assets acquired of \$22.9 million, reserves for excess and obsolete inventory of \$18.8 million, stock-based compensation of \$3.7 million and depreciation expense of \$1.8 million.
- Changes in net working capital which used \$10.9 million more in 2017 than in 2016.

#### ***Cash Used in Investing Activities***

During the year ended December 31, 2017, cash flows used in investing activities, primarily for purchases of property and equipment, totaled \$0.8 million. During the year ended December 31, 2016, cash flows provided by investing activities totaled \$25.3 million, primarily attributable to \$28.3 million of cash we acquired as a result of the Merger, offset by a \$3.0 million advance to Aegerion.

#### ***Cash Provided By (Used in) Financing Activities***

During the year ended December 31, 2017, cash flows provided by financing activities were immaterial. During the year ended December 31, 2016, cash flows used in financing activities included \$45.0 million of cash used to fund our investment in Aralez. The sale of 2,400,000 Aralez Shares pursuant to the terms of the Backstop Agreement netted \$15.0 million of cash proceeds, which were distributed to our shareholders in the Aralez Distribution. The 2016 Private Placement provided net proceeds of approximately \$21.5 million.

#### ***Comparison of the Years Ended December 31, 2016 and 2015***

Changes in net cash (used in) provided by operating activities, investing activities and financing activities for the year ended December 31, 2016 compared to 2015 were primarily attributable to our recognition, starting on November 29, 2016, of 100% of the cash flow activities of Aegerion, including, among others, cash generated from the net product sales of JXTAPID and MYALEPT, cash used to maintain inventory of those products, and cash used to support Aegerion's SG&A and R&D activities.



### Cash Used in Operating Activities

During the year ended December 31, 2016, cash used in operating activities was \$34.4 million compared to \$19.4 million of cash used in operating activities in 2015. The \$15.0 million increase was primarily attributable to the following:

- A significant increase in the net loss recognized year-over-year.
- A negative operating cash flow variance of \$8.0 million related to advisory fees paid to Greenhill in connection with the completion of Novelion's \$45.0 million investment in Aralez and the completion of Novelion's acquisition of Aegerion.
- A positive operating cash flow variance in the year ended December 31, 2016 of \$10.7 million related to a loss recorded based on the mark-to-market adjustment on the Aralez Investment to reflect changes in value from the acquisition date of February 5, 2016 through the distribution date of April 5, 2016.
- Negative operating cash flows were noted as a result of the acquisition of Aegerion, which included cash flows from the acquisition date of November 29, 2016 through December 31, 2016. Significant items noted related to payments made for deal-related consulting fees and litigation during the period. These negative operating cash outflows were offset by period amortization of the JUXTAPID and MYALEPT intangible assets recognized in conjunction with the Merger.

### Cash Provided by Investing Activities

During the year ended December 31, 2016, cash flows provided by investing activities totaled \$25.3 million, primarily attributable to \$28.3 million of cash we acquired as a result of the Merger, partially offset by a \$3.0 million advance to Aegerion. During the year ended December 31, 2015, cash flows provided by investing activities were insignificant.

### Cash (Used in) Provided by Financing Activities

During the year ended December 31, 2016, cash flows used in financing activities totaled \$23.5 million and included \$45.0 million of cash used to fund our investment in Aralez. The sale of 2,400,000 Aralez Shares pursuant to the terms of the Backstop Agreement netted \$15.0 million of cash proceeds, which were distributed to our shareholders in the Aralez Distribution. The 2016 Private Placement provided net proceeds of approximately \$21.5 million. During the year ended December 31, 2015, cash flows provided by financing activities consisted of \$5.5 million of proceeds received in connection with the issuance of common shares for stock options exercised.

### Contractual Obligations

Our contractual obligations as of December 31, 2017 consisted primarily of debt service requirements from the Convertible Notes, operating lease commitments and legal settlement obligations and are as follows:

Contractual Obligations (1)	Payments due by period				
	Total	Less than 1 year	1 - 3 years	3 - 5 years	More than 5 years
			(in thousands)		
Convertible Notes (2)	\$ 337,998	\$ 6,500	\$ 331,498	\$ —	\$ —
Operating leases	4,219	2,973	1,144	77	25
Legal settlement	39,612	8,596	27,106	3,910	—
Total contractual cash obligations (3)	\$ 381,829	\$ 18,069	\$ 359,748	\$ 3,987	\$ 25

(1) The following contractual obligations have been excluded from the table above due to the reasons stated below:

i. *Uncertain Tax Positions*

As disclosed in Note 16 - *Income Taxes* in the Notes to Consolidated Financial Statements for the year ended December 31, 2017, we have identified certain potential long-term liabilities associated with uncertain tax positions. Given that we are unable to reasonably or reliably estimate the timing of these future payments, if any, due to uncertainties about the timing and/or future outcomes of tax audits that may arise, these uncertain tax liabilities have been excluded from the table above.

ii. *Purchase Orders*

As of December 31, 2017, we have certain open purchase orders related to potential and/or expected future expenditures. A total of \$27.9 million attributable to these purchase orders is not currently reflected on our consolidated balance sheets and has been excluded from the table above given that the amounts are not fixed contractual obligations and would only give rise to liabilities to the extent that goods and services are provided to Novilion. In addition, all of our material research contracts with third-parties have normal course termination and cancellation provisions. These purchase orders reflect estimated future expenditures based on existing arrangements and do not reflect any future modifications to, or terminations of, existing contracts or potential new contracts.

iii. *Contract Research Organization (CRO) Agreement*

We have engaged contract research organizations (“CROs”) to provide research, safety and project management services (the “Services”) in connection with the execution of our potential clinical trials and existing registries. The estimated amount of Services is excluded from the table above given that Services have not yet been performed as of the December 31, 2017 balance sheet date and they would only give rise to liabilities to the extent that Services are provided to us and pass through expenses are incurred. As of December 31, 2017, the Company had total potential commitments of approximately \$47.0 million under these agreements. The amount reflected is based on the existing contracts and does not reflect any inflation, future modification to, or termination of the existing contracts or anticipated or potential new contracts. The agreements with our selected CROs contain normal course termination and cancellation provisions. In the event of cancellation of these agreements, the Company would be obligated to pay for all direct fees, pass through costs, and services performed or incurred through the termination date. In addition, we would be required to reimburse the CROs for all future non-cancelable obligations to third parties, where such obligations were created in connection with services authorized by Novilion.

iv. *Milestone Obligations*

We have also committed to make potential future milestone payments to certain third parties as part of our licensing, development, and purchase agreements. Payments under these arrangements are generally contingent and payable upon achievement of certain developmental, regulatory or commercial milestones. During the year ended December 31, 2017, none of these payments were triggered by the achievement of specified developmental, regulatory or commercial milestones. For more information refer to Note 18 - *Commitments & Contingencies* in the Notes to Consolidated Financial Statements for the year ended December 31, 2017 and Item 1. *Business* of this Annual Report.

Under Aegerion's license agreement with UPenn, Aegerion will be required to make development milestone payments of up to an aggregate amount of \$2.6 million if we decide to develop lomitapide for indications within the licensed field other than HoFH and we achieve certain milestones in such development efforts. All such development milestone payments for these other indications are payable only once, no matter how many licensed products for other indications are developed. We have not initiated plans to develop lomitapide for indications within the licensed field other than HoFH.

(2) The amounts represent the payments, including interest, under our Convertible Notes, which can potentially be settled by using our common shares.

(3) This table does not include (i) any milestone payments which may become payable to third parties under license agreements as the timing and likelihood of such payments are not known; (ii) any royalty payments to third parties as the amounts, timing and likelihood of such payments are not known; and (iii) contracts that are entered into in the ordinary course of business which are not material in the aggregate in any period presented above.

In January 2015, Aegerion acquired metreleptin pursuant to the Asset Purchase Agreement with AstraZeneca. Metreleptin, a recombinant analog of human leptin, is currently marketed in the U.S. under the brand name MYALEPT. MYALEPT received marketing approval from the FDA in February 2014 as an adjunct to diet as replacement therapy to treat the complications of leptin deficiency in patients with GL. Under the terms of the Asset Purchase Agreement, Aegerion paid AstraZeneca \$325.0 million to acquire the global rights to develop, manufacture and commercialize metreleptin, subject to an existing distributor license with Shionogi covering Japan, South Korea and Taiwan. The distribution agreement with Shionogi was assigned to Aegerion as part of the transaction. Aegerion also assumed certain other assets and liabilities of AstraZeneca related to the metreleptin program. In connection with the acquisition, Aegerion assumed an agreement, as amended, with a contract manufacturer of MYALEPT. An amendment, which was disclosed to Aegerion after the closing of the MYALEPT acquisition, commits Aegerion to spend approximately 0.37 million Euros per week in contract manufacturing costs for a minimum of twelve weeks per year with a maximum of sixteen weeks per year. The amount does not reflect any inflation, future modification to, or termination of, the existing contract or anticipated or potential new contract.

In connection with the acquisition of MYALEPT in January 2015, Aegerion acquired a license agreement between Amgen Inc. ("Amgen") and Amylin Pharmaceuticals, Inc., dated February 7, 2006 (the "Amgen License") pursuant to which Aegerion obtained an exclusive worldwide license from Amgen to certain know-how and patents and patent applications covering the composition of matter and methods of use of metreleptin to develop, manufacture and commercialize a preparation containing metreleptin (the "Amgen Licensed Products").

As part of the Amgen License, Aegerion also obtained an exclusive sublicense of Amgen's exclusive rights to certain metreleptin-related patents and patent applications owned by the Rockefeller University and exclusively licensed to Amgen under a license agreement dated April 14, 1995, as amended (the "Rockefeller License") and an exclusive sublicense of Amgen's non-exclusive rights to certain metreleptin-related patents and patent applications owned by The Regents of the University of California and non-exclusively licensed to Amgen under a license agreement dated July 13, 2005 (the "UCSF License"). Amgen retains rights to conduct research, development, manufacturing and commercialization activities with respect to products other than the Amgen Licensed Products.

Aegerion may grant sublicenses under the licenses and sublicenses granted by Amgen, subject to certain limitations, including Amgen's right of first offer for any out-license, partnership, co-development, commercialization, co-promotion or similar agreement related to metreleptin or the Amgen Licensed Products, which expires in February 2021. Under this license agreement, Amgen must notify Aegerion of any potential third-party partnership regarding any intellectual property rights controlled by Amgen in the neurology field and we will have a right of first negotiation for any license, partnership, co-development, commercialization, co-promotion or similar agreement, which expires in February 2021.

Aegerion is required to make royalty payments to Amgen on net sales of each Amgen Licensed Product on a country-by-country basis (i) at a royalty rate in the low double digits where the Amgen Licensed Product has patent protection or market exclusivity granted by a regulatory authority at the time of regulatory approval in the applicable country during the applicable royalty term, which runs on a country-by-country basis until the later of (a) the expiration of the last-to-expire valid claim covering an Amgen Licensed Product in the applicable country, (b) expiration of any market exclusivity granted by a regulatory authority, and (c) ten years from the date on which an Amgen Licensed Product is first sold to a third party in a country after regulatory approval for the Amgen Licensed Product has been granted in such country ("Amgen Royalty Term") or (ii) at a royalty rate in the mid-single digits to low double digits where the Amgen Licensed Product receives patent protection or market exclusivity following the time of regulatory approval in the applicable country, in either case subject to a variety of customary reductions.

Under the Amgen License, Aegerion is also required to directly meet certain payment obligations under the Rockefeller License and UCSF License. Aegerion is required to make royalty payments to Rockefeller University on net sales of each product with patent rights or know-how in the field of obesity genes, obesity gene products, and molecules that modulate or mediate their action and/or regulation on a country-by-country basis at a range of royalty rates in the low single digits depending on whether the product has an orphan product designation or not until the later to occur of expiration of (i) patent protection, (ii) any market exclusivity period granted in the applicable country, or (iii) any data exclusivity period in the applicable country (with certain limitations related to the number of units sold). In February 2015, Aegerion paid a one-time \$5.0 million milestone payment to Rockefeller University, which was due twelve months following the receipt of marketing approval for MYALEPT in the U.S. Aegerion is also required to pay to Rockefeller University a percentage in the low double digits of any upfront license fees or one-time fees Aegerion receives in consideration for any sublicense of the licensed rights. There are no material payment obligations outstanding under the UCSF License. Also, in connection with the acquisition of metreleptin, Aegerion entered into a letter agreement with AstraZeneca pursuant to which Aegerion agreed to make royalty payments payable by AstraZeneca and its affiliates to BMS with respect to net sales of metreleptin in the U.S. The time-based royalty rate ranges from mid-single digits to low double digits, increasing annually in years 2016 to 2019 from rates in the low single digits to low double digits, peaking in years 2019 to 2020 at a rate in the low double digits before decreasing in years 2022 through 2025 to rates in the high single digits to mid-single digits. The royalty obligation to BMS terminates in 2026.

The Amgen License will terminate upon the expiration of the last Amgen Royalty Term for any Amgen Licensed Product. Aegerion has the right to terminate the Amgen License for convenience upon 90 days prior written notice to Amgen or for Amgen's uncured material breach of the Amgen License, or becoming subject to specified bankruptcy or liquidation events. Amgen may terminate the Amgen License for Aegerion's uncured failure to make payments to Amgen or if Aegerion is the subject of specified bankruptcy or liquidation events.

Aegerion made royalty payments to Amgen, Rockefeller University and BMS related to sales of MYALEPT through November 29, 2016. There were no royalty payments made to these parties from November 30, 2016 to December 31, 2016. In 2017, Aegerion made aggregate royalty payments of \$9.7 million to Amgen, Rockefeller University and BMS, and had \$2.9 million in aggregate royalties payable to these parties as of December 31, 2017.

In addition, Aegerion is required to make royalty payments at a range of royalty rates in the high single digits on net sales of lomitapide in countries where lomitapide has patent protection, and in respect of any other products covered by the license (subject to a variety of customary reductions), and share with UPenn specified percentages of sublicensing royalties and other consideration that Aegerion receives under any sublicenses that Aegerion may grant.

In December 2016, Aegerion entered into a license agreement with Amryt under which Amryt was granted an exclusive right to develop and commercialize LOJUXTA in the European Economic Area ("EEA"), Switzerland, Turkey and certain Middle Eastern and North African territories, including Israel. Under the license agreement, Aegerion's subsidiary maintains the marketing authorizations for LOJUXTA; however, Amryt is responsible for ongoing regulatory and post-marketing obligations and commitments for LOJUXTA. Amryt is also required to pay certain sales-related milestone payments and royalties on net product sales in the licensed territories.

### **Future Funding Requirements**

Our need to raise additional capital in the near term, the size of any such financings and the availability and terms of any such financings, will depend on many factors, including:

- the success of our commercialization efforts and the level of revenues generated from sales of lomitapide and metreleptin in the U.S., and of lomitapide in other key countries where it is approved and being commercialized, including Japan;
- the status of ongoing or recently concluded government investigations and lawsuits, such as the Settlement and the JUXTAPID Investigations, including relevant obligations, the disclosure of possible or actual outcomes, and the negative publicity surrounding such matters, and the costs associated with the resolution of these investigations and lawsuits, including the civil penalties, restitution and settlement amounts discussed in the " *Legal Proceedings* " section of this Annual Report and the cost of implementing and complying with the CIA, the DPA, and the FDA Consent Decree and criminal probation terms;
- the timing and costs of satisfying our debt obligations, including interest payments and any amounts due upon the maturity of such debt, including under Aegerion's Convertible Notes, the Senior Loan and Aegerion's other indebtedness;
- the level of revenues we receive from named patient sales of our products in Brazil and other key countries where a mechanism exists to sell the product on a pre-approval basis in such country based on U.S. approval of such products or EU approval of lomitapide, particularly in light of the regulatory approval of Amgen's PCSK9 inhibitor product in Brazil in April 2016, the potential availability of that and other PCSK9 inhibitor products on a named patient sales basis in Brazil, the additional requirements that have been recently imposed on named patient sales of pharmaceutical products in Brazil, including our products, and potential future additional requirements or limitations, and the ongoing court proceedings in Brazil reviewing the regulatory framework for named patient sales;
- the level of physician, patient and payer acceptance of lomitapide and metreleptin, and the extent of the negative impact of and other risks associated with the availability of PCSK9 inhibitor products on sales of JUXTAPID in the U.S.;
- our ability to continue to manage our costs and expenses to better align with our revenues and strengthen our capital structure, while supporting approved products in a compliant manner;
- our ability to provide security to collateralize any financings, which may be required by lenders as a condition to providing us with any funding, particularly given the fact that substantially all of Aegerion's assets have been pledged as collateral under the Senior Loan and the New Loan Agreement, including the intellectual property of metreleptin and lomitapide;
- gaining regulatory and pricing and reimbursement approvals to market our products in countries in which the products are not currently approved and/or reimbursed, where it makes business sense to seek such approval, without significant restrictions, discounts, caps or other cost containment measures, including regulatory and pricing and reimbursement approval of metreleptin in the EU for both GL and the PL subgroup, in connection with which we filed an MAA in the EMA in December 2016, and the timing and costs of seeking such approvals;
- the timing and cost of lifecycle management and clinical development activities, particularly our anticipated trial assessing metreleptin in patients with hypoleptinemic metabolic disorder ("HMD");

- the willingness of insurance companies, managed care organizations, other private payers, and government entities that provide reimbursement for medical costs in the U.S. to continue to provide reimbursement for our products at the prices at which we offer our products without imposing any additional major hurdles to access or other significant restrictions or limitations, and the ability and willingness of HoFH and GL patients to pay, or to arrange for payment assistance with respect to, any patient cost-sharing amounts for our products applicable under their insurance coverage, particularly in light of recent reductions in contributions to 501(c)(3) patient organizations by pharmaceutical companies;
- the cost of maintaining the sales and marketing capabilities necessary for the commercialization of our products for their targeted indications in the market(s) in which each has received regulatory approval and we elect to commercialize such products, to the extent reimbursement and pricing approvals are obtained, and certain other key international markets, if approved;
- the timing and costs of future business development opportunities;
- the cost of filing, prosecuting and enforcing patent claims, including the cost of defending any challenges to the patents or our claims of exclusivity;
- the costs of our manufacturing-related activities and the other costs of commercializing our products;
- the levels, timing and collection of revenues received from sales of our products in the future;
- whether we are successful in our efforts to defend ourselves in, or to settle on acceptable terms, any significant litigation, including litigation that may result, directly or indirectly from the Settlement; and
- the cost of our current and future observational cohort studies and other post-marketing commitments, including to the FDA, the EMA and in any other countries where our products are ultimately approved.

We may seek additional capital due to favorable market conditions or strategic considerations, even if we believe we have sufficient funds for our current or future operating plans. The source, timing and availability of any future financing will depend principally upon equity and debt market conditions, interest rates and, more specifically, on the extent of our commercial success and our continued progress in our regulatory and development activities. There can be no assurance that external funds will be available on favorable terms, if at all.

#### **Off-Balance Sheet Arrangements**

We have an operating lease for office space in Cambridge, Massachusetts, which expires in 2019. We do not currently have, nor have we ever had, any relationships with unconsolidated entities or financial partnerships, such as entities often referred to as structured finance or special purpose entities, which would have been established for the purpose of facilitating off-balance sheet arrangements or other contractually narrow or limited purposes. In addition, we do not engage in trading activities involving non-exchange traded contracts. Therefore, we are not materially exposed to any financing, liquidity, market or credit risk that could arise if we had engaged in these relationships.

#### **Item 7A. Quantitative and Qualitative Disclosures About Market Risk.**

Market risk is the potential loss arising from adverse changes in the financial markets, including interest rates and foreign currency exchange rates.

##### *Interest Rate Risk*

We have outstanding \$325.0 million 2.0% Convertible Notes due August 15, 2019. The Convertible Notes have a fixed annual interest rate of 2.0% and we, therefore, do not have economic interest rate exposure on the Convertible Notes. However, the fair value of the Convertible Notes is exposed to interest rate risk. Generally, the fair value of the Convertible Notes will increase as interest rates fall and decrease as interest rates rise. These Convertible Notes are also affected by the price and volatility of our common shares and will generally increase or decrease as the market price of our common shares changes. As of December 31, 2017, the fair value of the Convertible Notes was estimated by us to be \$258.3 million. For additional discussion on the Convertible Notes, refer to Note 10 - *Convertible Notes, Net* in the Notes to Consolidated Financial Statements for the year ended December 31, 2017. As of December 31, 2017 and 2016, we had no other assets or liabilities with significant interest rate sensitivity.

### *Foreign Currency Exchange Risk*

We are also exposed to risks associated with foreign currency exchange rate fluctuations related to our international subsidiaries in which we continue to help support operations with financial contributions. We do not currently hedge our foreign currency exchange rate risk and do not have any outstanding forward foreign currency contracts. We manage this foreign currency risk, in part, through operational means including managing foreign currency revenues in relation to same currency costs as well as managing foreign currency assets in relation to same currency liabilities. We are also exposed to the potential earnings effects from intercompany foreign currency assets and liabilities that arise from normal trade receivables and payables and other intercompany loans. These subsidiaries' financial statements are re-measured into their respective functional currencies using current or historical exchange rates. Such re-measurement adjustments could have an adverse effect on the Company's results of operations.

### *Investment Risk*

At December 31, 2017 and 2016, we did not have any investments in debt or equity securities and as such were not exposed to risks associated with any other-than-temporary decline in fair value of these investments. At December 31, 2017, the Company had \$20.0 million invested in treasury bills and less than \$0.1 million invested in money market funds, which collectively have a weighted average remaining maturity of approximately 21.0 days. Any fluctuation in fair value of these cash equivalents will be immaterial due to the short term timeframe until maturity.

**Item 8. Consolidated Financial Statements and Supplementary Data.**

	<u>Page</u>
<a href="#"><u>Report of Independent Registered Public Accounting Firm - Deloitte &amp; Touche LLP</u></a>	<a href="#"><u>117</u></a>
<a href="#"><u>Report of Independent Registered Public Accounting Firm - Deloitte LLP</u></a>	<a href="#"><u>118</u></a>
<a href="#"><u>Consolidated Balance Sheets as of December 31, 2017 and 2016</u></a>	<a href="#"><u>119</u></a>
<a href="#"><u>Consolidated Statements of Operations for the years ended December 31, 2017, 2016 and 2015</u></a>	<a href="#"><u>120</u></a>
<a href="#"><u>Consolidated Statements of Comprehensive Loss for the years ended December 31, 2017, 2016 and 2015</u></a>	<a href="#"><u>121</u></a>
<a href="#"><u>Consolidated Statements of Shareholders' Equity for the years ended December 31, 2017, 2016 and 2015</u></a>	<a href="#"><u>122</u></a>
<a href="#"><u>Consolidated Statements of Cash Flows for the years ended December 31, 2017, 2016 and 2015</u></a>	<a href="#"><u>123</u></a>
<a href="#"><u>Notes to Consolidated Financial Statements</u></a>	<a href="#"><u>124</u></a>

## Report of Independent Registered Public Accounting Firm

To the Board of Directors and Shareholders of  
Novelion Therapeutics Inc.

### Opinion on the Financial Statements

We have audited the accompanying consolidated balance sheet of Novelion Therapeutics Inc. and subsidiaries (the "Company") as of December 31, 2017, and the related consolidated statements of operations, comprehensive loss, shareholders' equity, and cash flows, for the year then ended, and the related notes (collectively referred to as the "financial statements"). In our opinion, the financial statements present fairly, in all material respects, the financial position of the Company as of December 31, 2017, and the results of its operations and its cash flows for the year then ended, in conformity with accounting principles generally accepted in the United States of America.

We have also audited, in accordance with the standards of the Public Company Accounting Oversight Board (United States) (PCAOB), the Company's internal control over financial reporting as of December 31, 2017, based on criteria established in *Internal Control - Integrated Framework (2013)* issued by the Committee of Sponsoring Organizations of the Treadway Commission and our report dated March 16, 2018, expressed an adverse opinion on the Company's internal control over financial reporting because of a material weakness.

### Basis for Opinion

These financial statements are the responsibility of the Company's management. Our responsibility is to express an opinion on the Company's financial statements based on our audit. We are a public accounting firm registered with the PCAOB and are required to be independent with respect to the Company in accordance with the U.S. federal securities laws and the applicable rules and regulations of the Securities and Exchange Commission and the PCAOB.

We conducted our audit in accordance with the standards of the PCAOB. Those standards require that we plan and perform the audit to obtain reasonable assurance about whether the financial statements are free of material misstatement, whether due to error or fraud. Our audit included performing procedures to assess the risks of material misstatement of the financial statements, whether due to error or fraud, and performing procedures that respond to those risks. Such procedures included examining, on a test basis, evidence regarding the amounts and disclosures in the financial statements. Our audit also included evaluating the accounting principles used and significant estimates made by management, as well as evaluating the overall presentation of the financial statements. We believe that our audit provides a reasonable basis for our opinion.

/s/ Deloitte & Touche LLP

Boston, Massachusetts  
March 16, 2018

We have served as the Company's auditor since 2017.



## Report of Independent Registered Public Accounting Firm

To the Board of Directors and Shareholders of  
Novelion Therapeutics Inc.

We have audited the accompanying consolidated balance sheet of Novelion Therapeutics Inc. and subsidiaries (the “Company”) as of December 31, 2016, and the consolidated statements of operations, comprehensive loss, shareholders’ equity, and cash flows for each of the two years in the period ended December 31, 2016. These financial statements are the responsibility of the Company’s management. Our responsibility is to express an opinion on these financial statements based on our audits.

We conducted our audits in accordance with the standards of the Public Company Accounting Oversight Board (United States). Those standards require that we plan and perform the audit to obtain reasonable assurance about whether the financial statements are free of material misstatement. An audit includes examining, on a test basis, evidence supporting the amounts and disclosures in the financial statements. An audit also includes assessing the accounting principles used and significant estimates made by management, as well as evaluating the overall financial statement presentation. We believe that our audits provide a reasonable basis for our opinion.

In our opinion, such consolidated financial statements present fairly, in all material respects, the financial position of Novelion Therapeutics Inc. and subsidiaries as of December 31, 2016, and the results of their operations and their cash flows for each of the two years in the period ended December 31, 2016, in conformity with accounting principles generally accepted in the United States of America.

/s/ Deloitte LLP

Chartered Professional Accountants  
March 30, 2017  
Vancouver, Canada

**Novelion Therapeutics Inc.**  
**Consolidated Balance Sheets**  
(in thousands)

	December 31,	
	2017	2016
<b>Assets</b>		
Current assets:		
Cash and cash equivalents	\$ 55,430	\$ 108,927
Restricted cash	253	390
Accounts receivable, net	22,191	9,339
Inventories - current	15,886	15,718
Insurance proceeds receivable	—	22,000
Prepaid expenses and other current assets	11,183	9,762
<b>Total current assets</b>	<b>104,943</b>	<b>166,136</b>
Inventories - non-current	33,940	59,003
Property and equipment, net	2,920	4,159
Intangible assets, net	225,272	250,324
Other non-current assets	2,247	1,160
<b>Total assets</b>	<b>\$ 369,322</b>	<b>\$ 480,782</b>
<b>Liabilities and shareholders' equity</b>		
Current liabilities:		
Accounts payable	\$ 13,800	\$ 17,609
Accrued liabilities	41,838	37,180
Provision for legal settlements - current	8,596	64,010
<b>Total current liabilities</b>	<b>64,234</b>	<b>118,799</b>
Convertible notes, net	258,538	225,584
Provision for legal settlements - non-current	31,016	—
Other non-current liabilities	596	612
<b>Total liabilities</b>	<b>354,384</b>	<b>344,995</b>
Commitments and contingencies (Note 18)		
Shareholders' equity:		
Common shares, without par value, 100,000 shares authorized; 18,702 and 18,530 shares issued and outstanding at December 31, 2017 and 2016, respectively	551,925	551,259
Additional paid-in-capital	73,185	69,149
Accumulated deficit	(713,974)	(587,208)
Accumulated other comprehensive income	103,802	102,587
<b>Total shareholders' equity</b>	<b>14,938</b>	<b>135,787</b>
<b>Total liabilities and shareholders' equity</b>	<b>\$ 369,322</b>	<b>\$ 480,782</b>

See accompanying Notes to Consolidated Financial Statements.

**Novelion Therapeutics Inc.**  
**Consolidated Statements of Operations**  
(in thousands, except per share amounts)

	Year Ended December 31,		
	2017	2016	2015
Net revenues	\$ 138,438	\$ 13,574	\$ —
Cost of product sales	77,220	5,971	—
Operating expenses:			
Selling, general and administrative	96,472	29,525	16,222
Research and development	49,008	14,784	9,790
Restructuring charges	2,536	—	—
Termination fee	—	—	(2,667)
Total operating expenses	148,016	44,309	23,345
Loss from operations	(86,798)	(36,706)	(23,345)
Interest (expense) income, net	(39,037)	(2,960)	277
Fair value loss on investment	—	(10,740)	—
Other (expense) income, net	(292)	(1,999)	81
Loss before provision for income taxes	(126,127)	(52,405)	(22,987)
Provision for income taxes	(583)	(465)	(22)
Net loss	\$ (126,710)	\$ (52,870)	\$ (23,009)
Net loss per common share—basic and diluted	\$ (6.81)	\$ (4.69)	\$ (2.20)
Weighted-average shares outstanding—basic and diluted	18,616	11,284	10,434

See accompanying Notes to Consolidated Financial Statements.

**Novelion Therapeutics Inc.**  
**Consolidated Statements of Comprehensive Loss**  
(in thousands)

	Year Ended December 31,		
	2017	2016	2015
Net loss	\$ (126,710)	\$ (52,870)	\$ (23,009)
Other comprehensive income (loss):			
Foreign currency translation	1,215	(382)	—
Other comprehensive income (loss)	1,215	(382)	—
Comprehensive loss	\$ (125,495)	\$ (53,252)	\$ (23,009)

See accompanying Notes to Consolidated Financial Statements.

**Novelion Therapeutics Inc.**  
**Consolidated Statements of Shareholders' Equity**  
(in thousands, except share information)

	Common		Additional	Accumulated	Accumulated	Total
	Stock				Paid-In	
	Shares	Amount	Capital	Deficit		Comprehensive
Balance at December 31, 2014	10,239,594	\$ 467,034	\$ 97,838	\$ (511,329)	\$ 102,969	\$ 156,512
Net loss	—	—	—	(23,009)	—	(23,009)
Stock-based compensation expense	—	—	2,330	—	—	2,330
Exercise of stock options	313,095	8,077	(2,569)	—	—	5,508
Shares issued in connection with vesting of RSUs	12,800	222	(222)	—	—	—
Balance at December 31, 2015	10,565,489	475,333	97,377	(534,338)	102,969	141,341
Net loss	—	—	—	(52,870)	—	(52,870)
Foreign currency translation adjustment	—	—	—	—	(382)	(382)
Stock-based compensation expense	—	—	797	—	—	797
Shares issued in connection with Acquisition of Aegerion	6,060,288	59,381	—	—	—	59,381
Shares issued in private placement, net of share issuance costs	1,904,546	16,545	4,936	—	—	21,481
Cash distribution to shareholders	—	—	(15,000)	—	—	(15,000)
Aralez shares distributed to shareholders	—	—	(19,296)	—	—	(19,296)
Uncertain tax position liability recovery	—	—	335	—	—	335
Balance at December 31, 2016	18,530,323	551,259	69,149	(587,208)	102,587	135,787
Net loss	—	—	—	(126,710)	—	(126,710)
Foreign currency translation adjustment	—	—	—	—	1,215	1,215
Stock-based compensation expense	—	—	4,537	—	—	4,537
Exercise of stock options	70,820	666	(167)	—	—	499
Vesting of restricted stock, net of shares withheld for taxes	100,792	—	(390)	—	—	(390)
Adoption of ASU 2016-09, <i>Compensation - Stock Compensation</i>	—	—	56	(56)	—	—
Balance at December 31, 2017	18,701,935	\$ 551,925	\$ 73,185	\$ (713,974)	\$ 103,802	\$ 14,938

See accompanying Notes to Consolidated Financial Statements.

**Novelion Therapeutics Inc.**  
**Consolidated Statements of Cash Flows**  
(in thousands)

	Year Ended December 31,		
	2017	2016	2015
<b>Cash used in operating activities</b>			
Net loss	\$ (126,710)	\$ (52,870)	\$ (23,009)
Adjustments to reconcile net loss to net cash used in operating activities:			
Depreciation	2,050	264	576
Amortization of intangible assets	25,052	2,134	—
Stock-based compensation	4,537	797	2,330
Non-cash interest expense	32,954	2,676	—
Provision for inventory excess and obsolescence	18,814	—	—
Fair value change in contingent consideration	—	2,042	—
Unrealized foreign exchange (gain) loss	(832)	118	(120)
Fair value loss on investment	—	10,704	—
Deferred income taxes	138	(214)	18
Other non-cash operating activities	83	50	(25)
Changes in assets and liabilities, excluding the effect of acquisition:			
Accounts receivable	(12,852)	(893)	10
Inventories	6,081	2,079	—
Prepaid expenses and other assets	19,541	705	442
Accounts payable	(3,932)	4,441	(184)
Accrued and other liabilities	(19,823)	(6,389)	603
Net cash used in operating activities	(54,899)	(34,356)	(19,359)
<b>Cash (used in) provided by investing activities</b>			
Purchase of property and equipment	(754)	(155)	(9)
Cash acquired in Merger	—	28,290	—
Loan receivable	—	(3,000)	—
Net proceeds from sale of long-lived assets	—	192	43
Net cash (used in) provided by investing activities	(754)	25,327	34
<b>Cash provided by (used in) financing activities</b>			
Issuance of common shares	109	21,481	5,508
Cash distribution to common shareholders	—	(15,000)	—
Settlement of Backstop Agreement	—	15,000	—
Aralez investment	—	(45,000)	—
Net cash provided by (used in) financing activities	109	(23,519)	5,508
Exchange rate effect on cash	2,047	(349)	(267)
Net decrease in cash and cash equivalents	(53,497)	(32,897)	(14,084)
Cash and cash equivalents, beginning of period	108,927	141,824	155,908
Cash and cash equivalents, end of period	\$ 55,430	\$ 108,927	\$ 141,824
<b>Supplemental disclosures of cash flow information</b>			
Cash paid for interest	\$ 6,500	\$ 33	\$ —
Cash paid for taxes	\$ 1,671	\$ 105	\$ —
<b>Non-cash investing activities</b>			
Purchases of property and equipment included in accounts payable	\$ 122	\$ 61	\$ —

See accompanying Notes to Consolidated Financial Statements.

**Novelion Therapeutics Inc.**  
**Notes to Consolidated Financial Statements**

## **1. Description of Business**

Novelion Therapeutics Inc. ("Novelion" or the "Company") is a rare disease biopharmaceutical company dedicated to developing new standards of care for individuals living with rare diseases. Novelion has international operations, two commercial products, lomitapide and metreleptin, and one orphan drug-designated product candidate, zuretinol acetate ("zuretinol"). Lomitapide, which is marketed in the United States ("U.S.") under the brand name JUXTAPID (lomitapide) capsules, is approved in the U.S. as an adjunct to a low-fat diet and other lipid-lowering treatments, including low-density lipoprotein ("LDL") apheresis where available, to reduce low-density lipoprotein cholesterol ("LDL-C"), total cholesterol ("TC"), apolipoprotein B ("apo B") and non-high-density lipoprotein cholesterol ("non-HDL-C") in adult patients with homozygous familial hypercholesterolemia ("HoFH"). Lomitapide is also approved in the European Union ("EU"), under the brand name LOJUXTA, for the treatment of adult patients with HoFH, as well as in Japan, Canada, and a limited number of other countries. Metreleptin, a recombinant analog of human leptin, is currently marketed in the U.S. under the brand name MYALEPT (metreleptin for injection). MYALEPT is approved in the U.S. as an adjunct to diet as replacement therapy to treat the complications of leptin deficiency in patients with congenital or acquired generalized lipodystrophy ("GL").

On November 29, 2016, QLT Inc. ("QLT") completed its acquisition of Aegerion Pharmaceuticals, Inc. ("Aegerion"), through the merger ("the Merger") of its indirect, wholly-owned subsidiary Isotope Acquisition Corp. ("MergerCo") with and into Aegerion, pursuant to an Agreement and Plan of Merger (as amended, the "Merger Agreement"), dated as of June 14, 2016, among QLT, Aegerion and MergerCo. Upon closing the acquisition, QLT changed its name to Novelion. As a result of the Merger, Aegerion became an indirect wholly-owned subsidiary of Novelion. Financial statements for the years ended December 31, 2017 and 2016 include the combined operations of Novelion and its wholly-owned subsidiaries, including the financial results of Aegerion.

The accompanying consolidated financial statements have been prepared assuming the Company will continue to operate as a going concern, which contemplates the realization of assets and the settlement of liabilities and commitments in the normal course of business. As presented in the consolidated financial statements, for the year ended December 31, 2017, the Company incurred a net loss of \$126.7 million and used \$54.9 million in cash to fund operating activities. As described in Note 19, on March 15, 2018, the Company closed on and received \$20.0 million in proceeds from a new loan agreement, bringing the Company's unrestricted cash balance at March 15, 2018 to approximately \$57.6 million. The Company expects to fund its current and planned operating requirements principally through its existing cash resources, and other potential financing methods, including the possibility of utilizing equity. The Company believes that its existing funds are sufficient to satisfy its operating needs and its working capital, milestone payments, capital expenditures, debt service requirements and legal settlement expenditures for at least the next twelve months from the issuance of the financial statements. The Company may, from time to time, also seek additional funding, primarily designed to fund potential additional indications for metreleptin, through strategic alliances and additional equity and/or debt financings or from other sources, should it identify a significant new opportunity.

## **2. Summary of Significant Accounting Principles**

### ***Basis of Presentation and Principles of Consolidation***

The accompanying consolidated financial statements have been prepared in accordance with accounting principles generally accepted in the United States of America ("GAAP"). All amounts herein are expressed in U.S. dollars ("USD") unless otherwise noted.

The accompanying consolidated financial statements include operations of Novelion Therapeutics Inc. and its wholly-owned subsidiaries. All intercompany transactions and balances have been eliminated.

### ***Use of Estimates***

The preparation of consolidated financial statements in accordance with GAAP requires management to make estimates and assumptions that affect the reported amounts of assets and liabilities, the disclosure of contingent assets and liabilities at the date of the consolidated financial statements, and the reported amounts of expenses during the reporting periods presented. The Company's estimates often are based on complex judgments, probabilities and assumptions that the Company believes to be reasonable but that are inherently uncertain and unpredictable. For any given individual estimate or assumption made by us, there may also be other estimates or assumptions that are reasonable. Actual results may differ from estimates made by management. Changes in estimates are reflected in reported results in the period in which they become known.

### ***Reporting and Functional Currency***

The Company's reporting currency is the USD and its operations utilize the USD or local currency as the functional currency, where applicable.

Transactions in other currencies are recorded in the functional currency at the rate of exchange prevailing when the transactions occur. Monetary assets and liabilities denominated in other currencies are re-measured into the functional currency at the rate of exchange in effect at the balance sheet date. Exchange gains and losses arising from re-measurement of foreign currency-denominated monetary assets and liabilities are included in income in the period in which they occur.

For foreign entities where the local currency is the functional currency, assets and liabilities denominated in local currencies are translated into USD at end-of-period exchange rates and the resulting translation adjustments are reported as a component of accumulated other comprehensive income (loss) within shareholders' equity.

### ***Cash and Cash Equivalents***

Cash and cash equivalents consist of highly liquid instruments purchased with an original maturity of three months or less at the date of purchase.

### ***Restricted Cash***

Restricted cash represents primarily amounts deposited to collateralize the Company's corporate credit card program and a letter of credit for the Company's facility lease in Cambridge, Massachusetts.

### ***Accounts Receivable, net***

The majority of the Company's accounts receivable arises from product sales and primarily represents amounts due from distributors, named patients, and other entities. The Company monitors the financial performance and creditworthiness of its customers to properly assess and respond to changes in their credit profile. The Company provides reserves against accounts receivable for estimated losses that may result from a customer's inability to pay. Amounts determined to be uncollectible are charged or written-off against the reserve. To date, the Company's historical reserves and write-offs of accounts receivable have not been significant.

### ***Inventories and Cost of Product Sales***

Inventories are stated at the lower of cost or net realizable value with cost determined on a first-in, first-out basis.

Inventory is maintained on the Company's consolidated balance sheets until the inventory is sold, donated as part of the Company's compassionate use program, or used for clinical development. Inventory that is sold is recognized as cost of product sales in the consolidated statements of operations, inventory that is donated as part of the Company's compassionate use program is recognized as a selling, general and administrative ("SG&A") expense in the consolidated statements of operations, expired inventory is disposed of and the related costs are recognized as cost of product sales in the consolidated statements of operations, and inventory used for clinical development is recognized as research and development expense in the consolidated statements of operations.

Inventories are reviewed periodically to identify slow-moving inventory based on sales activity, both projected and historical, as well as product shelf-life. The portion of the slow-moving inventory not expected to be sold within one year is classified as non-current inventory in the Company's accompanying consolidated balance sheets. At each balance sheet date, the Company evaluates its ending inventories for excess quantities and obsolescence. This evaluation includes an analysis of sales levels by product type, the expiration date of the Company's finished goods on-hand at each balance sheet date, as well as the anticipated unit forecast for demand for each product. Any changes to the demand of either product may result in a reserve for excess and obsolescence if the Company believes inventory will expire prior to being commercially sold, and such charge will be recorded to cost of product sales.

If the asset becomes impaired or is abandoned, the carrying value is written down to its net realizable value, and an impairment charge is recorded in the period in which the impairment occurs. In evaluating the recoverability of inventories produced, the Company considers the probability that revenue will be obtained from the future sale of the related inventory.



Cost of product sales includes the cost of inventory sold, manufacturing and supply chain costs, product shipping and handling costs, charges for excess and obsolete inventory, amortization of acquired intangibles, as well as royalties payable to The Trustees of the University of Pennsylvania ("UPenn") related to the sale of lomitapide and royalties payable to Amgen Inc. ("Amgen"), Rockefeller University and Bristol-Myers Squibb ("BMS") related to the sale of metreleptin.

### ***Prepaid Manufacturing Costs***

Cash advances paid by the Company prior to receipt of inventory are recorded as prepaid manufacturing costs and included in prepaid expenses and other current assets on the consolidated balance sheets. The cash advances are subject to forfeiture if the Company terminates the scheduled production. The Company expects the carrying value of the prepaid manufacturing costs to be fully realized. As of December 31, 2017 and 2016, \$4.1 million and \$1.4 million was recorded as prepaid manufacturing costs, respectively.

### ***Property and Equipment***

Property and equipment are stated at cost and depreciated using the straight-line method based on estimated economic lives of three to five years for office furniture, fixtures, research equipment and other equipment, and three years for computer software and hardware. Leasehold improvements are amortized over the shorter of the estimated useful lives of the improvements or the remaining lease term, which include lease extensions when reasonably assured. Repair and maintenance costs are expensed as incurred.

### ***Intangible Assets***

Intangible assets with definite useful lives are amortized, on a straight-line basis, to their estimated residual values over their estimated useful lives and reviewed for impairment if certain triggering events occur.

### ***Impairment of Long-lived Assets***

Impairment testing and assessments of remaining useful lives are performed when a triggering event occurs that could indicate a potential impairment or change in useful life. Such testing first entails comparison of the carrying value of the long-lived asset to the undiscounted cash flows expected from that asset. If impairment is indicated by this test, the long-lived assets are written down by the amount, if any, by which the discounted cash flows expected from the long-lived asset exceed its carrying value.

### ***Business Combinations***

The Company evaluates acquisitions of assets and other similar transactions to assess whether or not each such transaction should be accounted for as a business combination by assessing whether or not the Company has acquired inputs and processes that have the ability to create outputs. If the Company determines that an acquisition qualifies as a business, the Company applies the acquisition method of accounting which requires that the purchase price be allocated to the net assets acquired at their respective fair values. Goodwill is calculated as the excess of the consideration transferred over the net assets recognized and represents the future economic benefits arising from other assets acquired that could not be individually identified and separately recognized. Goodwill is not amortized and is not deductible for tax purposes. The Company has no goodwill on its consolidated balance sheets as December 31, 2017 or 2016. The Company reports provisional amounts when measurements are incomplete as of the end of the reporting period. Additionally, the Company completes the purchase price allocation within a measurement period which does not extend beyond one year after the acquisition date.

Contingent consideration in a business combination is included as part of the acquisition cost and is recognized at fair value as of the acquisition date. Fair value is generally estimated by using a probability-weighted discounted cash flow approach. Any liability resulting from contingent consideration is re-measured to fair value at each reporting date until the contingency is resolved. These changes in fair value are recognized on the consolidated statement of operations as a component of SG&A expenses.

The present-value models used to estimate the fair values of acquired inventories and intangibles incorporate significant assumptions, including, but not limited to: assumptions regarding the probability of obtaining marketing approval; estimated selling price, estimates of the timing and amount of future cash flows from potential product sales and related expenses; and the appropriate discount rate selected to measure the risks inherent in the future cash flows, the assessment of the asset's lifecycle and the competitive trends impacting the assets, including consideration of any technical, legal, regulatory or economic barrier.

Transaction costs associated with business combinations are expensed as incurred. The Company's consolidated financial statements include the results from operations of an acquired business after the transaction date.

### ***Contingencies***

The Company records a liability in the consolidated financial statements for litigation related matters when a loss is considered probable and the amount can be reasonably estimated. If the loss is not probable or a range cannot reasonably be estimated, no liability is recorded in the consolidated financial statements. Legal fees are expensed as incurred. Insurance recoveries related to potential claims are recognized up to the amount of the recorded liability when coverage is confirmed and the estimated recoveries are probable of payment. These recoveries are not netted against the related liabilities for financial statement presentation.

### ***Revenue Recognition***

The Company recognizes revenue from product sales when there is persuasive evidence that an arrangement exists, title to product and associated risk of loss has passed to the customer, the price is fixed or determinable, collectability is reasonably assured and the Company has no further performance obligations.

The Company records distribution and other fees paid to its distributors as a reduction of revenue. Revenue is recorded net of estimated discounts and rebates, primarily including those provided to Medicare and Medicaid. Allowances are recorded as a reduction of revenue at the time revenues from product sales are recognized. Allowances for government rebates and discounts are established based on the actual payer information, which is reasonably estimable at the time of delivery. These allowances are adjusted to reflect known changes in the factors that may impact such allowances in the quarter those changes are known. To date, such adjustments have not been significant.

We receive royalty revenues on sales by our licensees. We record these revenues based on estimates of the sales occurred during the relevant period and the applicable royalty rate. Differences between actual and estimated royalty revenues are adjusted for in the period in which they become known, typically the following quarter. Historically, adjustments have not been material when compared to actual amounts paid by licensees.

The Company also offers a branded co-pay assistance program for eligible patients with commercial insurance in the U.S. who are on JUXTAPID or MYALEPT therapy. The branded co-pay assistance program assists commercially insured patients who have coverage for JUXTAPID or MYALEPT, and is intended to reduce each participating patient's portion of the financial responsibility for JUXTAPID's or MYALEPT's purchase price up to a specified dollar amount of assistance. The Company records revenue net of amounts paid under the branded specific co-pay assistance program for each patient.

### ***Lomitapide***

In the U.S., JUXTAPID is only available for distribution through a specialty pharmacy. Until the November 2017 transition to the new specialty pharmacy described in the following paragraph, the product was shipped directly to the patient and prior authorization and confirmation of coverage level by a patient's private insurance plan or government payer were prerequisites to shipment. Revenue from sales in the U.S. covered by the patient's private insurance plan or government payer was recognized once the product had been received by the patient. For uninsured amounts billed directly to the patient, revenue was recognized at the time of cash receipt as collectability was not reasonably assured at the time the product was received by the patient. To the extent amounts were billed in advance of delivery to the patient, the Company deferred revenue until the product was received by the patient.

In the second quarter of 2017, to improve distribution efficiency, the Company's subsidiary signed a letter of intent for the distribution of JUXTAPID with the same specialty pharmacy that distributes MYALEPT in the U.S. The agreement was signed and finalized in October 2017 and the transition of this distribution model was completed in November 2017. Subsequent to completion of the transition, revenue from sales of JUXTAPID in the U.S. has been recognized upon product shipment to the distributor (sell-in method).

The Company also records revenue on sales in countries where lomitapide is available on a named patient basis, typically paid for by a government authority or institution. In many cases, these sales are processed through a third-party distributor that takes title to the product upon acceptance. Because of factors such as the pricing, the limited number of patients, the short period from product sale to delivery to the end-customer, and the limited contractual return rights, these distributors typically only hold inventory to supply specific orders for the product. The Company recognizes revenue for sales under these named patient programs

upon product acceptance by the third-party distributor. In the event the payer's creditworthiness has not been established, the Company recognizes revenue on a cash basis if all other revenue recognition criteria have been met.

### *Metreleptin*

Sales of metreleptin are facilitated through third-party distributors that take title to the product upon acceptance. Because of factors such as pricing, the limited number of patients, the short period from product sale to delivery to the end-customer, and the limited contractual return rights, these distributors typically only hold inventory to supply specific orders for the product. The Company recognizes revenue for sales upon product acceptance by the third-party distributor. In the event the payer's creditworthiness has not been established, the Company recognizes revenue on a cash basis if all other revenue recognition criteria have been met.

Prior to the second quarter of 2017, due to insufficient historical data to reasonably estimate the gross-to-net adjustments for rebates related to payors and insurance providers at the time of receipt by the distributor, the Company accounted for MYALEPT shipments using a deferred revenue recognition model (sell-through method). Under the deferred revenue recognition model, the Company did not recognize revenue upon product shipment. For product shipments, the Company invoiced the distributor, recorded deferred revenue at the gross invoice sales price, classified the cost basis of the product held by the distributor as a separate component of inventory, and recognized revenue when delivered to the patient.

Beginning in the second quarter of 2017, the Company determined that there was sufficient history to reasonably estimate expected rebates, and, to align its existing and anticipated revenue streams of products sold within the U.S., began recognizing sales of MYALEPT upon product shipment to the distributor (sell-in method). For the three-month period ended June 30, 2017, the Company recognized a one-time increase in net revenue of \$2.3 million resulting from this change in estimate, representing previously deferred product sales.

### ***Research and Development Expenses***

Research and development expenses are charged to expense as incurred. Research and development expenses comprise costs incurred in performing research and development activities, including personnel-related costs, stock-based compensation, facilities-related overhead, clinical trial costs, costs to support certain medical affairs activities, manufacturing costs for clinical and pre-clinical materials as well as other contracted services, license fees, and other external costs. Nonrefundable advance payments for goods and services that will be used in future research and development activities are expensed when the activity has been performed or when the goods have been received rather than when the payment is made.

### ***Income Taxes***

Income taxes are reported using the asset and liability method, whereby deferred tax assets and liabilities are recognized for the future tax consequences attributable to: (i) differences between the financial statement carrying amounts of existing assets and liabilities and their respective tax bases, and (ii) operating loss and tax credit carryforwards using applicable enacted tax rates. An increase or decrease in these tax rates will increase or decrease the carrying value of future net tax assets resulting in an increase or decrease to net income. Income tax credits, such as investment tax credits, are included as part of the provision for income taxes. Current income taxes are provided for in accordance with the laws of the relevant taxing authorities. Significant estimates are required in determining the Company's provision for income taxes and uncertain tax positions. Some of these estimates are based on interpretations of existing tax laws or regulations. Various internal and external factors may have favorable or unfavorable effects on the Company's future effective tax rate. These factors include, but are not limited to, changes in tax laws, regulations and/or rates, changing interpretations of existing tax laws or regulations, changes in estimates of prior years' items, results of tax audits by tax authorities, future levels of research and development spending, changes in estimates related to repatriation of undistributed earnings of foreign subsidiaries, and changes in overall levels of pre-tax earnings. The realization of the Company's deferred tax assets is primarily dependent on whether the Company is able to generate sufficient capital gains and taxable income prior to expiration of any loss carry forward balance. A valuation allowance is provided when it is more likely than not that a deferred tax asset will not be realized. The assessment of whether or not a valuation allowance is required often requires significant judgment including the long-range forecast of future taxable income and the evaluation of tax planning initiatives. Adjustments to the deferred tax valuation allowances are made to earnings in the period when such assessments are made.

The Company records tax benefits for all years subject to examination based upon management's evaluation of the facts, circumstances and information available at the reporting date. There is inherent uncertainty in quantifying income tax positions. The Company has recorded tax benefits for those tax positions where it is more likely than not that a tax benefit will result upon ultimate settlement with a tax authority that has all relevant information. For those income tax positions where it is not more likely

than not that a tax benefit will result, no tax benefit has been recognized in the consolidated financial statements. Penalties and interest are recorded as a component of income tax expense.

### ***Stock-Based Compensation***

For service-based awards, compensation expense is measured at the grant date based on the fair value of the award and is recognized on a straight-line basis over the requisite service period, which is typically the vesting period. For awards that vest or begin vesting upon achievement of a performance condition, the Company recognizes compensation expense when achievement of the performance condition is deemed probable using a straight-line model over the implicit service period. Certain of the Company's awards that contain performance conditions also require the Company to estimate the number of awards that will vest, which the Company estimates when the performance condition is deemed probable of achievement. For awards that vest upon the achievement of a market condition, the Company recognizes compensation expense over the derived service period. For equity awards that have previously been modified, any incremental increase in the fair value over the original award has been recorded as compensation expense on the date of the modification for vested awards or over the remaining service period for unvested awards.

The Company has a Directors' Deferred Share Unit Plan ("DDSU Plan") for the Company's directors. Given that vested Deferred Share Units ("DSUs") are convertible to cash only, the Company recognizes compensation expense for DSUs based on the market price of the Company's shares. The Company also records an accrued liability to recognize the expected financial obligation related to the future settlement of these DSUs as they vest. Each reporting period, the expected obligation is revalued for changes in the market value of Novilion's common shares.

The Company issues restricted stock units ("RSUs") to its employees and directors as consideration for their provision of future services. Restricted stock-based compensation expense is measured based on the fair value market price of Novilion's common shares on the grant date and is recognized over the requisite service period, which coincides with the vesting period. RSUs can only be exchanged and settled for Novilion's common shares, on a one-to-one basis, upon vesting.

### ***Comprehensive Loss***

Comprehensive loss combines net loss and other comprehensive items. Other comprehensive items represent certain amounts that are reported as components of shareholders' equity in the accompanying consolidated balance sheets, including currency translation adjustments.

### ***Recently Adopted Accounting Standards***

In the first quarter of 2017, the Company adopted Accounting Standards Update ("ASU") No. 2016-09, *Compensation - Stock Compensation (Topic 718)* ("ASU 2016-09"). ASU 2016-09 simplifies several aspects of the accounting for share-based payment transactions, including the income tax consequences, classification of awards as either equity or liabilities, and classification of related activity on the statement of cash flows. The adoption of this ASU did not have a material impact to the Company's consolidated financial statements.

In the first quarter of 2017, the Company adopted ASU No. 2015-11, *Simplifying the Measurement of Inventory* ("ASU 2015-11"), using the prospective method as required. ASU 2015-11 states that an entity should measure inventory at the lower of cost or net realizable value. Net realizable value is the estimated selling prices in the ordinary course of business, less reasonably predictable costs of completion, disposal, and transportation. The adoption of this ASU did not have a material impact to the Company's consolidated financial statements.

In the fourth quarter of 2017, the Company adopted ASU No. 2014-15, *Presentation of Financial Statements— Going Concern (Subtopic 205-40)* : Disclosure of Uncertainties about an Entity's Ability to Continue as a Going Concern ("ASU 2014-15"). ASU 2014-15 requires management to assess an entity's ability to continue as a going concern by incorporating and expanding upon certain principles of current U.S. auditing standards. Specifically, the amendments (1) provide a definition of the term "substantial doubt", (2) require an evaluation every reporting period, including interim periods, (3) provide principles for considering the mitigating effect of management's plans, (4) require certain disclosures when substantial doubt is alleviated as a result of consideration of management's plans, (5) require an express statement and other disclosures when substantial doubt is still present and (6) require an assessment for a period of one year after the date that the financial statements are issued (or available to be issued). ASU 2014-15 is effective prospectively for annual reporting periods ending after December 15, 2016, and for annual and interim periods thereafter. The adoption of ASU 2014-15 did not have a material effect on the Company's consolidated financial statements.

### ***Recent Accounting Pronouncements Not Yet Adopted***

In May 2014, the Financial Accounting Standards Board ("FASB") issued ASU No. 2014-09, *Revenue from Contracts with Customers* ("ASU 2014-09"). ASU 2014-09 represents a comprehensive new revenue recognition model that requires a company to recognize revenue to depict the transfer of promised goods or services to customers in an amount that reflects the consideration to which a company expects to be entitled in exchange for those goods or services. This ASU sets forth a new five-step revenue recognition model which replaces most existing revenue recognition guidance including industry-specific guidance. The Company adopted ASU 2014-09 and related ASUs, effective January 1, 2018, using the modified retrospective method applied to those contracts which were not completed as of that date.

The Company established an implementation team to assist with its assessment of the impact of the new revenue guidance on its operations, consolidated financial statements and related disclosures. The Company's assessment has included performing analysis for each revenue stream identified, assessing the potential differences in recognition and measurement that may result from adopting this standard and assessing whether the Company meets certain practical expedients. Based on the results of the assessment, the adoption of this standard will not have a material impact on the timing or amount of revenue recognized upon adoption and there is no cumulative prior period adjustment to be recorded to the opening balance of retained earnings upon adoption. The Company also anticipates changes to its disclosures to comply with the new disclosure requirements under the guidance. The Company is implementing the necessary changes to its revenue recognition accounting policies and controls to support recognition and disclosure under the new standard.

On February 25, 2016, the FASB issued ASU No. 2016-02, *Leases* ("ASU 2016-02"), its new standard on accounting for leases. The new guidance will require the Company to recognize the assets and liabilities associated with the rights and obligations created by those leases. ASU 2016-02 will be effective for annual periods beginning after December 15, 2018, and interim periods within those periods. The standard requires the application of the modified retrospective transition approach, and provides for certain expedients. The Company is currently assessing the impact ASU 2016-02 will have on its consolidated financial statements. Refer to additional details of the Company's lease obligations in Note 14.

On November 17, 2016, the FASB issued ASU No. 2016-18, *Statement of Cash Flows (Topic 230), Restricted Cash* ("ASU 2016-18"). ASU 2016-18 states that a statement of cash flows should explain the change during the period in the total of cash, cash equivalents, and amounts generally described as restricted cash or restricted cash equivalents. Therefore, amounts generally described as restricted cash and restricted cash equivalents should be included with cash and cash equivalents when reconciling the beginning-of-period and end-of-period total amounts shown on the statement of cash flows. ASU 2016-18 is effective for fiscal years beginning January 1, 2018, and interim periods within those fiscal years. All updates should be applied using a retrospective transition method. The Company does not anticipate that the adoption of ASU 2016-18 will have a material effect on its consolidated financial statements or related disclosures.

On May 10, 2017, the FASB issued ASU No. 2017-09, *Compensation—Stock Compensation (Topic 718) - Scope of Modification Accounting* ("ASU 2017-09"), to clarify when to account for a change to the terms or conditions of a share-based payment award as a modification. Under the new guidance, modification accounting is required only if the fair value, the vesting conditions, or the classification of the award (as equity or liability) changes as a result of the change in terms or conditions. ASU 2017-09 is effective prospectively for annual periods beginning on or after December 15, 2017. Early adoption is permitted. The Company is currently assessing the impact ASU 2017-09 will have on its consolidated financial statements.

### **3. Acquisition**

#### *Aegerion Pharmaceuticals, Inc.*

On November 29, 2016, Novelion completed its acquisition of Aegerion and each share of Aegerion's common stock was exchanged for 1.0256 Novelion ("pre-Consolidation") common shares (the "Exchange Ratio"). Immediately after the Merger, the Company had 18,530,323 common shares outstanding; former shareholders of Novelion held approximately 68% of the Company, and former stockholders of Aegerion held approximately 32% of the Company.

The Merger has been accounted for as a business combination under the acquisition method, with Novelion as the accounting acquirer and Aegerion as the "acquired" company. The operating results of Aegerion from November 29, 2016 are included in the accompanying Consolidated Statements of Operations for the years ended December 31, 2017 and 2016. The consolidated balance sheets as of December 31, 2017 and 2016 reflect the acquisition of Aegerion.

The acquisition consideration in connection with the Merger was approximately \$62.4 million, and the Company allocated the acquisition consideration to various tangible and intangible assets acquired and liabilities assumed, based on their estimated fair values, which were finalized during 2017.

	<u>Amount</u> (in thousands, except for share and per share information)
Number of Novelion common shares issued in connection with the acquisition of Aegerion	6,060,288
Novelion share price on November 29, 2016	\$ 9.75
Fair value of Novelion common shares issued to Aegerion stockholders	\$ 59,088
Liabilities assumed (2)	3,000
Stock compensation assumed (1)	293
Total acquisition consideration	<u>\$ 62,381</u>

(1) Represents the fair value of Aegerion in-the-money options and RSUs attributed to pre-combination services that were outstanding on November 29, 2016 and settled in connection with the Merger. All the outstanding out-of-the-money Aegerion stock options were cancelled as of the Merger date.

(2) Represents amounts borrowed under the term loan facility provided by QLT to Aegerion on June 14, 2016, concurrently with the execution of the Merger Agreement. At the time, Aegerion had borrowed \$3.0 million against the term loan and the loan remained outstanding as of November 29, 2016.

The following table summarizes the fair values of the assets acquired and liabilities assumed at the Merger date. There were no adjustments identified subsequent to the acquisition date.

	<u>November 29, 2016</u> (in thousands)
Cash and cash equivalents	\$ 28,290
Restricted cash	390
Accounts receivable (1)	8,182
Inventories	76,800
Prepaid expenses and other current assets	9,839
Insurance proceeds receivable	22,000
Property and equipment, net	4,020
Intangible assets	252,458
Other assets	1,352
Accounts payable	(11,459)
Accrued liabilities	(41,883)
Provision for legal settlement (2)	(63,968)
Convertible notes	(222,908)
Other liabilities	(732)
Net assets acquired	<u>\$ 62,381</u>

(1) As of the Merger date, the fair value of accounts receivable approximated the book value acquired. The amount not expected to be collected was insignificant.

(2) Legal Matters - Aegerion has been the subject of certain ongoing investigations and other legal proceedings. See Note 18 for further information regarding these and other legal proceedings.

The valuation of the intangible assets acquired and related amortization periods are as follows:

Developed Technology:	Amount	Amortization period
	(in thousands)	(in years)
Lomitapide	\$ 42,300	10.75
Metreleptin	210,158	9.25
<b>Total</b>	<b>\$ 252,458</b>	

The fair values of the intangibles were determined using a multi-period excess earnings approach. Under this method, an intangible asset fair value is equal to the present value of the after-tax cash flows attributable solely to the intangible asset over its remaining useful life. To calculate fair value, the Company used cash flows discounted at 11% .

The fair values of the purchased inventories were also determined using a discounted present value income approach. To calculate fair value, the Company used cash flows discounted at 11% . There was no goodwill recorded as part of the acquisition.

The Company recognized acquisition-related transaction costs associated with the Merger during the year ended December 31, 2016 totaling approximately \$4.0 million . These costs, which related primarily to bank fees, legal and accounting services, and fees for other professional services, were expensed as incurred, and reported as SG&A expenses in the accompanying consolidated statement of operations for the year ended December 31, 2016 .

#### *Actual and Pro Forma Impact of Acquisition*

The following table presents the amount of Aegerion net product sales and net loss included in the Company's consolidated statement of operations from November 29, 2016 through December 31, 2016 :

	November 29, 2016 - December 31, 2016
	<i>(in thousands, except for per share information)</i>
Net revenues	\$ 13,574
Net loss	\$ (6,276)
Basic and diluted net loss per share	\$ (0.34)

The following supplemental unaudited pro forma information presents the financial results as if the Merger had occurred on January 1, 2015 for the years ended December 31, 2016 and 2015 .

	Year Ended December 31,	
	2016	2015
	<i>(in thousands, except for per share information)</i>	
Net product sales	\$ 153,245	\$ 239,887
Net loss	\$ (207,773)	\$ (96,348)
Basic and diluted loss per share	\$ (18.41)	\$ (9.23)

This supplemental pro forma information has been prepared for comparative purposes and does not purport to reflect what the Company's results of operations would have been had the acquisition occurred on January 1, 2015, nor does it project the future results of operations of the Company or reflect the expected realization of any cost savings associated with the acquisition. The actual results of operations of the Company may differ significantly from the pro forma adjustments reflected here due to many factors. The unaudited supplemental pro forma financial information includes various assumptions, including those related to the purchase price allocation of the assets acquired and the liabilities assumed from Aegerion.

#### **4. Terminated Merger Transaction**

On June 8, 2015, QLT entered into an Agreement and Plan of Merger (as amended and restated on each of July 16, 2015 and August 26, 2015) (the "InSite Merger Agreement") with InSite Vision Incorporated, a Delaware corporation ("InSite"). On September 15, 2015, the InSite Merger Agreement was terminated by InSite's board of directors. As a result, InSite paid QLT a termination fee of \$2.7 million . In addition, in conjunction with the entry into the InSite Merger Agreement, on June 8, 2015 QLT granted InSite a secured line of credit (the "Secured Note") for up to \$9.9 million to fund continuing operations through to the

completion of the proposed InSite merger. Upon termination of the InSite Merger Agreement, InSite's repayment obligations under the Secured Note were accelerated and InSite paid QLT the full amount owed of \$5.8 million on September 15, 2015, which consisted of \$5.7 million of principal drawn from the Secured Note and \$0.1 million of accrued interest.

During the year ended December 31, 2015, QLT incurred \$10.2 million of consulting and transaction fees in connection with QLT's pursuit of the InSite Merger, and such fees have been reflected as part of SG&A expenses on the consolidated statement of operations for the year then ended.

## 5. Strategic Transactions

### *Aralez Investment and Distribution*

On December 7, 2015, QLT entered into an Amended and Restated Share Subscription Agreement (the "Amended and Restated Subscription Agreement") with Tribute Pharmaceuticals Canada Inc. ("Tribute"), POZEN Inc. ("POZEN"), Aralez Pharmaceuticals plc, (formally known as Aguono Limited) ("Aralez Ireland") and certain other investors for the purpose of returning capital to QLT's shareholders in either Aralez Shares (approximately 0.13629 of an Aralez Share for each common share of the Company) or cash, subject to pro-ration (the "Aralez Distribution"), up to a maximum of \$15.0 million funded pursuant to the terms of the Backstop Agreement as described below.

In connection with the Aralez Distribution, on June 8, 2015, QLT entered into a share purchase agreement (as amended, the "Backstop Agreement") with Broadfin Healthcare Master Fund, Ltd. ("Broadfin") and the JW Partners, LP, JW Opportunities Fund, LLC and JW Opportunities Master Fund, Ltd. (together the "JW Parties"), pursuant to which Broadfin and the JW Parties agreed to purchase up to \$15.0 million of the Aralez Shares from QLT at \$6.25 per share. This arrangement provided QLT's shareholders with the opportunity to elect to receive, in lieu of Aralez Shares, up to an aggregate of \$15.0 million in cash, subject to proration among the shareholders.

On February 5, 2016, QLT purchased 7,200,000 Aralez Shares (representing 10.1% of the issued and outstanding Aralez Shares), for an aggregate price of \$45.0 million. On April 5, 2016 (the "Distribution Date"), QLT distributed 4,799,619 Aralez Shares with a fair value of \$19.3 million, and \$15.0 million of cash to shareholders of record on February 16, 2016. At that time of the transaction, a member of QLT's Board of Directors was an affiliate of Aralez. QLT held the Aralez Shares from February 5, 2016 to the Distribution Date and the Aralez Shares were marked-to-market. As a result, QLT recognized a \$10.7 million loss during the year ended December 31, 2016, reflecting the change in value from the acquisition date to the Distribution Date.

Pursuant to QLT's financial advisory services agreement with Greenhill dated December 4, 2014 (as amended, the "Greenhill Agreement"), QLT paid Greenhill a \$4.0 million advisory fee in connection with the completion of QLT's \$45.0 million investment in Aralez and exploration of other strategic initiatives described in Note 4. The recognition and payment of the advisory fee was both contingent upon the satisfaction of various terms and conditions, which were met on February 5, 2016, and subject to the outcome of certain external factors and uncertainties, which were settled by February 5, 2016 but were beyond the Company's control.

### *Private Placement related to Aralez*

On June 8, 2015, QLT entered into a Share Purchase and Registration Rights Agreement (as amended, the "Share Purchase and Registration Rights Agreement") with Broadfin, JW Partners, LP, JW Opportunities Fund, LLC, EcoR1 Capital Fund Qualified, L.P. and EcoR1 Capital Fund, LP (the "QLT Investors"). The Share Purchase and Registration Rights Agreement provided that QLT would, following the completion of the Aralez Distribution, issue and sell to the QLT Investors a certain number of QLT common shares for an aggregate purchase price of \$20.0 million, reflecting a per share purchase price of \$1.87. In light of the termination of the InSite Merger Agreement and the board's determination that QLT's cash requirements at that time did not justify the dilution that would be caused by this private placement, on April 28, 2016, QLT and the QLT Investors mutually agreed to terminate the Share Purchase and Registration Rights Agreement.

## 6. Inventories

The components of inventories are as follows:



	December 31,	
	2017	2016
	(in thousands)	
Work-in-process	\$ 22,579	\$ 20,219
Finished goods	27,247	54,502
<b>Total</b>	<b>49,826</b>	<b>74,721</b>
Less: Inventories - current	(15,886)	(15,718)
<b>Inventories - non-current</b>	<b>\$ 33,940</b>	<b>\$ 59,003</b>

As part of the Merger, the Company acquired inventories with a value of \$76.8 million . A portion of inventory is classified as non-current as of December 31, 2017 and 2016 based on forecasted consumption exceeding one year. Based upon a review of projected sales, remaining product shelf-life and respective fair values, an \$18.8 million charge for excess and obsolete inventory was recognized during the year ended December 31, 2017 . There was no charge for excess or obsolete inventory in the consolidated statements of operations during the year ended December 31, 2016 or 2015 .

## 7. Property and Equipment

Property and equipment consist of the following:

	December 31,	
	2017	2016
	(in thousands)	
Leasehold improvements	\$ 1,686	\$ 1,869
Office furniture and equipment	1,743	539
Research equipment	—	1,962
Computer and office equipment	3,768	10,236
Construction in progress	123	339
Property, equipment and leasehold improvements, at cost	7,320	14,945
Less accumulated depreciation	(4,400)	(10,786)
<b>Property, equipment and leasehold improvements, net</b>	<b>\$ 2,920</b>	<b>\$ 4,159</b>

As a result of the expired office lease in Vancouver, British Columbia, we disposed of computer and office equipment in 2017 and removed the associated cost and accumulated depreciation from the related accounts. Less than \$0.1 million of loss was recognized for the year ended December 31, 2017 . Depreciation expense was \$2.0 million , \$0.3 million and \$0.6 million for the years ended December 31, 2017 , 2016 and 2015 , respectively.

## 8. Intangible Assets

The intangible assets are amortized over their estimated useful lives and reviewed for impairment when events and changes in circumstances indicate that the carrying amount may not be recoverable. During the year ended December 31, 2017 , there were no impairment charges recorded. Additionally, the Company reviewed the useful lives of the intangibles as of December 31, 2017 and believes the useful lives are still reasonable.

Intangible asset balances as of December 31, 2017 and 2016 are as follows:

	December 31, 2017		
	Gross Carrying Value	Accumulated Amortization	Net Carrying Value
	(in thousands)		
Developed technology - lomitapide	\$ 42,300	\$ (4,262)	\$ 38,038
Developed technology - metreleptin	210,158	(22,924)	187,234
<b>Total intangible assets</b>	<b>\$ 252,458</b>	<b>\$ (27,186)</b>	<b>\$ 225,272</b>

	December 31, 2016		
	Gross Carrying Value	Accumulated Amortization	Net Carrying Value
	(in thousands)		
Developed technology - lomitapide	\$ 42,300	\$ (328)	\$ 41,972
Developed technology - metreleptin	210,158	(1,806)	208,352
Total intangible assets	<u>\$ 252,458</u>	<u>\$ (2,134)</u>	<u>\$ 250,324</u>

Amortization expense was \$25.1 million and \$2.1 million for the years ended December 31, 2017 and 2016, respectively. There was no amortization expense recorded for the year ended December 31, 2015.

As of December 31, 2017, the estimated amortization expense related to these intangibles for future periods is as follows:

Years Ending December 31,	Amount
	(in thousands)
2018	\$ 25,095
2019	25,095
2020	25,095
2021	25,095
2022	25,095
Thereafter	99,797
Total intangible assets subject to amortization	<u>\$ 225,272</u>

## 9. Accrued Liabilities

Accrued liabilities as of December 31, 2017 and 2016 consist of the following:

	December 31,	
	2017	2016
	(in thousands)	
Accrued employee compensation and related costs	\$ 7,755	\$ 7,920
Accrued professional fees	4,118	3,466
Accrued sales allowances	13,471	7,849
Accrued royalties	3,588	3,301
Other accrued liabilities	12,906	14,644
Total	<u>\$ 41,838</u>	<u>\$ 37,180</u>

The following table summarizes combined activity for lomitapide and metreleptin in the sales allowance and reserve for the years ended December 31, 2017 and 2016. There was no sales allowance in 2015 as the Company did not have any commercial products and did not generate any revenues.

	December 31,	
	2017	2016
	(in thousands)	
Beginning balance	\$ 7,849	\$ —
Provision	23,087	7,849
Payments	(17,465)	—
Ending balance	<u>\$ 13,471</u>	<u>\$ 7,849</u>

During the year ended December 31, 2017, the Company incurred \$2.5 million in restructuring charges primarily related to the consolidation of similar positions during the integration of the business subsequent to the Merger. There were no restructuring

expenses incurred during the years ended December 31, 2016 and 2015. The restructuring charges consisted primarily of severance and benefits costs. The following table sets forth the components of the restructuring charge and payments made against the reserve for the year ended December 31, 2017 :

	<b>December 31, 2017</b>	
	<b>(in thousands)</b>	
Beginning restructuring balance	\$	—
Costs incurred		2,536
Cash paid		(2,788)
Other adjustments		262
Ending restructuring balance	<u>\$</u>	<u>10</u>

#### **10. Convertible Notes, net**

In August 2014, Aegerion issued Convertible Notes with an aggregate principal amount of \$325.0 million . The Convertible Notes are governed by the terms of an indenture and a supplemental indenture with The Bank of New York Mellon Trust Company, N.A., as the Trustee. The following are the key terms of the Convertible Notes:

- The Convertible Notes are senior unsecured obligations and bear interest at a rate of 2.0% per year, payable semi-annually in arrears on February 15 and August 15 of each year, beginning on February 15, 2015. The Convertible Notes will mature on August 15, 2019 , unless earlier repurchased or converted.
- After the Merger, the Convertible Notes are convertible into Novelon’s common shares at a conversion rate of 4.9817 common shares per \$1,000 principal amount of the Convertible Notes, as adjusted for the Exchange Ratio and the Consolidation. If the holders elect to convert the Convertible Notes, Aegerion can settle the conversion of the Convertible Notes through payment or delivery of cash, common shares, or a combination of cash and common shares, in its discretion.
- On or after February 15, 2019 until the close of business on the second scheduled trading day immediately preceding the maturity date, holders may convert all or any portion of their Convertible Notes, in multiples of \$1,000 principal amount, at the option of the holder.
- The indenture does not contain any financial covenants or restrict Aegerion’s ability to repurchase Aegerion’s securities, pay dividends or make restricted payments in the event of a transaction that substantially increases Aegerion’s level of indebtedness.
- The indenture contains customary terms and covenants and events of default. If an event of default (other than certain events of bankruptcy, insolvency or reorganization involving Aegerion) occurs and is continuing, the Trustee by notice to Aegerion, or the holders of at least 25% in principal amount of the outstanding Convertible Notes by written notice to Aegerion and the Trustee, may declare 100% of the principal of and accrued and unpaid interest, if any, on all of the Convertible Notes to be due and payable. Upon such a declaration of acceleration, such principal and accrued and unpaid interest, if any, will be due and payable immediately. Upon the occurrence of certain events of bankruptcy, insolvency or reorganization involving Aegerion, 100% of the principal and accrued and unpaid interest, if any, on the Convertible Notes will become due and payable automatically. Notwithstanding the foregoing, the indenture provides that, upon Aegerion’s election, and for up to 180 days , the sole remedy for an event of default relating to certain failures by Aegerion to comply with certain reporting covenants in the indenture consists exclusively of the right to receive additional interest on the Convertible Notes.

The Convertible Notes were re-measured at fair value at the Merger date, and the fair value of the Convertible Notes as of the Merger date was approximately \$222.9 million , which was determined by taking into consideration valuations obtained from third-party pricing services. The pricing services utilize pricing models incorporating variables such as coupon, maturity, conversion ratio, parity, corporate actions and equity market closing prices to calculate conversion premiums and sensitivity values to estimate the fair value of the Convertible Notes. As of the Merger date, management attributed the fair value entirely to the liability component of the Convertible Notes for the following reasons: (1) as of the Merger date, the conversion price ( \$200.74 ) was significantly higher than the price of Novelon common shares ( \$9.75 ), and (2) management did not expect the price of Novelon common shares to increase above the conversion price before the Convertible Notes mature in August 2019.

The Company's outstanding Convertible Note balances as of December 31, 2017 and 2016 consist of the following:

	December 31,	
	2017	2016
(in thousands)		
Liability component:		
Principal	\$ 324,998	\$ 324,998
Less: debt discount	(66,460)	(99,414)
Net carrying amount	<u>\$ 258,538</u>	<u>\$ 225,584</u>

The expected life of the debt was equal to the five year term on the Convertible Notes. The effective interest rate on the liability component was 16.42% for the period from the acquisition date through December 31, 2017. The following table sets forth total interest expense recognized related to the Convertible Notes during the years ended December 31, 2017 and 2016:

	Year Ended December 31,	
	2017	2016
(in thousands)		
Contractual interest expense	\$ 6,500	\$ 577
Amortization of debt discount	32,954	2,676
Total interest expense	<u>\$ 39,454</u>	<u>\$ 3,253</u>

Future minimum payments under the Convertible Notes as are as follows:

Years Ending December 31,	Amount
	(in thousands)
2018	\$ 6,500
2019	331,498
	<u>337,998</u>
Less amounts representing interest	(13,000)
Less debt discount, net	(66,460)
Net carrying amount of Convertible Notes as of December 31, 2017	<u>\$ 258,538</u>

## 11. Fair Value of Financial Instruments

Fair value is defined as the exchange price that would be received for an asset or paid to transfer a liability (an exit price) in the principal or most advantageous market for the asset or liability in an orderly transaction between market participants at the measurement date. A fair value hierarchy for those instruments measured at fair value is established that distinguishes between fair value measurements based on market data (observable inputs) and those based on the Company's own assumptions (unobservable inputs). This hierarchy maximizes the use of observable inputs and minimizes the use of unobservable inputs. The three levels of inputs used to measure fair value are as follows:

*Level 1* - Quoted prices in active markets for identical assets or liabilities that the Company has the ability to access at the measurement date.

*Level 2* - Inputs other than quoted prices in active markets that are observable for the asset or liability, either directly or indirectly.

*Level 3* - Inputs that are unobservable for the asset or liability based on management's best estimate of inputs market participants would use in pricing the asset or liability at the measurement date, including assumptions about risk.

The fair value measurements of the Company's financial instruments at December 31, 2017 are summarized in the table below:

	Quoted Prices in Active Markets for Identical Assets (Level 1)	Significant Other Observable Inputs (Level 2)	Significant Unobservable Inputs (Level 3)	Balance at December 31, 2017
(in thousands)				
Financial Instruments:				
Money market funds	\$ 20,046	\$ —	\$ —	\$ 20,046
Restricted cash	253	—	—	253
Total Financial Instruments	<u>\$ 20,299</u>	<u>\$ —</u>	<u>\$ —</u>	<u>\$ 20,299</u>

The fair value measurements of the Company's financial instruments at December 31, 2016 are summarized in the table below:

	Quoted Prices in Active Markets for Identical Assets (Level 1)	Significant Other Observable Inputs (Level 2)	Significant Unobservable Inputs (Level 3)	Balance at December 31, 2016
(in thousands)				
Financial Instruments:				
Money market funds	\$ 68,234	\$ —	\$ —	\$ 68,234
Restricted cash	390	—	—	390
Total Financial Instruments	<u>\$ 68,624</u>	<u>\$ —</u>	<u>\$ —</u>	<u>\$ 68,624</u>

The fair value of the Convertible Notes, which differs from their carrying values, is influenced by interest rates, the Company's share price and share price volatility and is determined by prices for the Convertible Notes observed in market trading which are Level 2 inputs. The estimated fair value of the Convertible Notes at December 31, 2017 and 2016 was \$258.3 million and \$240.4 million, respectively.

The Company's financial instruments include cash and cash equivalents, accounts receivable, accounts payable and accrued liabilities. The carrying amounts of cash and cash equivalents, accounts receivable, and accounts payable approximate fair value due to their immediate or short-term maturities.

These financial instruments are also exposed to credit risks. To limit the Company's credit exposure, cash and cash equivalents are deposited with high-quality financial institutions in accordance with its treasury policy goal to preserve capital and maintain liquidity. The Company's treasury policy limits investments to certain money market securities issued by governments, financial institutions and corporations with investment-grade credit ratings, and places restrictions on maturities and concentration by issuer. The Company maintains its cash, cash equivalents and restricted cash in bank accounts, which, at times, exceed federally insured limits. The Company has not experienced any credit losses in these accounts and does not believe it is exposed to any significant credit risk on these funds.

The Company is subject to credit risk from its accounts receivable related to product sales of lomitapide and metreleptin. The majority of the Company's accounts receivable arises from product sales and primarily represents amounts due from distributors, named patients, and other entities. The Company monitors the financial performance and creditworthiness of its customers to properly assess and respond to changes in their credit profile, and provides reserves against accounts receivable for estimated losses that may result from a customer's inability to pay. The Company does not recognize revenue for uninsured amounts billed directly to a patient until the time of cash receipt as collectability is not reasonably assured at the time the product is received by the customer. To date, the Company has not incurred any material credit losses.

## 12. Basic and Diluted Net Loss per Common Share

Basic net loss per common share is calculated by dividing the net loss by the weighted-average number of common shares outstanding for the period.

Diluted net loss per common share is computed by dividing the net loss by the weighted-average number of unrestricted common shares and dilutive common share equivalents outstanding for the period, determined using the treasury-stock and if-converted methods. Since the Company has had net losses for all periods presented, all potentially dilutive securities were determined to be anti-dilutive. Accordingly, basic and diluted net loss per common share are equal.

The following table sets forth potential common shares issuable upon the exercise of outstanding options, warrants, the vesting of restricted stock units and the conversion of the Convertible Notes (prior to consideration of the treasury stock and if-converted methods), which were excluded from the computation of diluted net loss per common share because such instruments were anti-dilutive:

	December 31,		
	2017	2016	2015
	(in thousands)		
Stock options	2,025	1,715	86
Unvested restricted stock units	445	1,029	—
Warrants	14,515	14,515	568
Convertible notes	1,619	1,619	—
<b>Total</b>	<b>18,604</b>	<b>18,878</b>	<b>654</b>

### 13. Share Capital

#### (a) Private Placement

On June 14, 2016, the Company entered into a unit subscription agreement (the "Unit Subscription Agreement") with the investors party thereto (the "Investors"). Pursuant to the Unit Subscription Agreement, immediately prior to the Merger, the Investors acquired units, for \$8.80 per unit, on a post-Consolidation basis, consisting of (i) 2,472,727 Novelson common shares, which include up to 568,181 Novelson common shares issuable upon exercise of fully paid-up warrants, and (ii) warrants (the "Unit Subscription Agreement Warrants") which were exercisable for up to an aggregate of 2,644,952 Novelson common shares at an exercise price of \$0.05 per share on the same terms and conditions as the Merger Agreement Warrants (collectively with the Merger Agreement Warrants, the "Warrants"). The net consideration received from the private placement was \$21.5 million ( \$21.8 million gross consideration net of \$0.3 million of share issuance costs), which was recorded as equity and allocated based on the relative fair values of the common shares and the paid-up warrants at the time of issuance. The Unit Subscription Agreement Warrants were issued on the same terms and conditions as the Merger Agreement Warrants.

The Company accounted for the paid-up warrants issued in the Private Placement in accordance with the guidance regarding the accounting for derivative financial instruments indexed to, and potentially settled in, a company's own stock. The paid-up warrants met the requirements to be accounted for as equity instruments. The proceeds related to the sale of the paid-up warrants are included in additional paid-in capital in the consolidated balance sheets.

#### (b) Cash, Share and Warrant Distributions

On April 5, 2016, based on shareholder election, Novelson distributed \$15.0 million of cash and 4,799,619 Aralez Shares, with a fair value of \$19.3 million , to its shareholders of record on February 16, 2016 in accordance with the Amended and Restated Subscription Agreement.

On November 23, 2016, the Company issued 10,565,879 Merger Agreement Warrants to its shareholders of record on November 17, 2016. As of December 31, 2017 , the Merger Agreement Warrants were recognized as a liability on the Company's consolidated balance sheet with a fair value of zero , as the exercise of such warrant was not deemed probable. The Merger Agreement Warrants have been cancelled subsequent to December 31, 2017 .

The Company accounts for the contingent warrants in accordance with the guidance regarding the accounting for derivative financial instruments indexed to, and potentially settled in, a company's own stock. The contingent warrants met the requirements to be accounted for as derivative instruments as the contingent warrants are variable and indexed to an event other than the fair value of the Company's shares.

## 14. Stock-based Payments

Under the Amended and Restated Novelion 2017 Equity Incentive Plan ("NVLN Plan"), under which it may grant non-qualified stock options, incentive stock options, and restricted stock units ("RSUs") to employees, directors and consultants of Novelion and its affiliates. Common shares of Novelion will be issued upon exercise of stock options and the vesting of RSUs. Under the terms of the NVLN Plan, Novelion is entitled to grant awards in respect of its unissued common shares up to a maximum of 4,760,000 shares. As of December 31, 2017, the Company has 2,277,417 shares of common shares available for issuance under its NVLN Plan.

In April 2017, the Board of Directors adopted the Company's 2017 Employee Stock Purchase Plan (the "ESPP"), to enable eligible employees of the Company and its designated subsidiaries to use payroll deductions to purchase shares of stock in offerings under the plan. A total of 278,710 shares of common stock have been reserved for issuance under the ESPP. Common shares of Novelion will be issued upon exercise of ESPP grants.

The Company issues stock options, RSUs and ESPP grants with service conditions, which are generally the vesting periods of the awards. The Company has also issued RSUs that vest upon the satisfaction of certain performance conditions or the satisfaction of certain market conditions. Generally, the stock options, RSUs and ESPP grants expire within 10 years of grant.

### *Determining the Fair Value of Stock Awards*

#### (a) Stock Options

The fair value of stock options is measured with service-based and performance-based vesting criteria to employees, consultants and directors on the date of grant using the Black-Scholes option pricing model. In general, the stock options vest over three to four years. The Black-Scholes option pricing model was developed for use in estimating the value of traded options that have no vesting restrictions and are fully transferable. In addition, option pricing models require the input of highly subjective assumptions, including the expected stock price volatility. The expected volatility and expected life of the Company's stock options are projected based upon historical and other economic data trended into future years. The risk-free interest rate assumption is based upon observed interest rates appropriate for the expected life of the Company's stock options. The risk-free interest rate is determined by reference to implied yields available from U.S. Treasury securities with a remaining term equal to the expected life assumed at the date of grant.

The weighted-average grant date fair values of stock options granted during the years ended December 31, 2017, 2016 and 2015 were \$3.15, \$7.12, and CAD \$10.60, respectively. The following weighted-average assumptions were used to value stock options granted in each of the years ended December 31, 2017, 2016 and 2015:

	Year Ended December 31,		
	2017	2016	2015
Expected stock price volatility	38.57%	38.23%	41.30%
Risk-free interest rate	2.04%	1.93%	1.40%
Expected life of options (years)	6.25	6.19	6.80
Expected dividend yield	—	—	—

The Company's stock option activity for the year ended December 31, 2017 is as follows:

	Number of Stock Options	Weighted- Average Exercise Price Per Share	Weighted Average Remaining Contractual Life (years)	Aggregate Intrinsic Value
Outstanding at December 31, 2016	1,714,593	\$ 9.01	9.6	\$ 528
Granted	1,391,125	7.70		
Exercised	(70,820)	7.15		
Forfeited/cancelled	(1,009,483)	9.48		
Outstanding at December 31, 2017	<u>2,025,415</u>	\$ 8.00	7.5	\$ —
Vested and expected to vest at December 31, 2017	2,025,415	\$ 8.00	7.5	\$ —
Exercisable at December 31, 2017	430,851	\$ 8.89	3.3	\$ —

As of December 31, 2017, the total future unrecognized compensation cost related to unvested stock options is \$4.4 million and is expected to be recorded over a weighted average period of 2.56 years.

During the years ended December 31, 2017, 2016 and 2015, the weighted average exercise price of stock options granted was \$7.70, \$8.27 and CAD \$4.84. The total intrinsic value of options exercised during the year ended December 31, 2015 was \$4.9 million; there was no intrinsic value for the options exercised in 2017 and 2016. The Company received \$0.5 million and \$5.5 million in cash proceeds from option exercises during the years ended December 31, 2017 and 2015, respectively; there was no cash proceeds received from option exercises in 2016.

#### (b) Deferred Share Units (DSUs)

The Company has a Directors' Deferred Share Unit Plan ("DDSU Plan") for the Company's directors. Under the Company's DDSU Plan, at the discretion of the Board of Directors, directors can receive all or a percentage of their equity-based compensation in the form of DSUs. DSUs vest in thirty-six (36) successive and equal monthly installments beginning on the first day of the first month after the grant date. A vested DSU can only be settled by conversion to cash (no shares are issued), and is automatically converted after the director ceases to be a member of the Board unless the director is removed from the Board for just cause. Prior to conversion, the value of each DSU, at any point in time, is equivalent to the latest closing price of Novelion's common shares on that trading day. When converted to cash, the value of a vested DSU is equivalent to the closing price of a common share of Novelion on the trading day immediately prior to the conversion date.

Given that vested DSUs are convertible to cash only, the Company recognizes compensation expense for DSUs based on the market price of the Company's shares. The Company also records an accrued liability to recognize the expected financial obligation related to the future settlement of these DSUs as they vest. Each reporting period, the expected obligation is revalued for changes in the market value of the Company's common shares.

The obligation to settle DSUs in cash is recorded as a liability in the Company's consolidated financial statements and is marked-to-market at the end of each reporting period. Cash payments under the DDSU Plan were immaterial and less than \$0.1 million during the years ended December 31, 2017 and 2016, respectively, and no cash payment was made under the DDSU Plan during the year ended December 31, 2015. The cash payments in 2016 related to two former members of the Novelion board of directors who departed from the board after the acquisition closed on November 29, 2016. The Company's obligation to settle the remaining vested and unsettled 28,400 common shares underlying DSUs held by former directors was not significant as of December 31, 2017 and 2016.

In connection with the Merger, the vesting provisions applicable to all of the outstanding and unvested DSUs were accelerated on November 29, 2016. The acceleration of the vesting provisions of all the outstanding and unvested DSUs during the year ended December 31, 2016 resulted in additional DSU compensation expense of \$0.06 million recognized in the Company's 2016 results of operations. There were no additional DSU activities in 2017.

#### (c) Restricted Stock Units (RSUs)

The Company issues restricted stock units ("RSUs") to its employees and directors as consideration for their provision of future services. Restricted stock-based compensation expense is measured based on the fair value market price of the Company's



common shares on the grant date and is recognized over the requisite service period, which coincides with the vesting period. RSUs can only be exchanged and settled for the Company's common shares, on a one -to-one basis, upon vesting.

The Company has outstanding time-vested, market-based and performance-based RSUs. Time-vested RSUs are awarded to eligible employees and entitle the grantee to receive common shares at the end of a vesting period, subject solely to the employee's continuing employment. The majority of time-vested RSUs vest over two to three years. All the market-based RSUs vest when the Company's stock price is equal to or greater than the value of the original new hire strike price and expire on July 29, 2019. The performance-based RSUs are awarded to eligible employees and entitle the grantee to receive shares of common stock if specified performance goals are achieved during the performance period and the grantee remains employed during the subsequent vesting period. The majority of performance-based RSUs vest in three equal annual installments beginning upon goal achievement. Upon vesting, each RSU represents the right to receive one common share of the Company.

The Company's RSU activity for the year ended December 31, 2017 is as follows:

	Number of RSUs	Weighted- Average Grant Date Fair Value
Outstanding at December 31, 2016	1,028,881	\$ 8.82
Granted	218,500	4.59
Vested	(150,556)	11.54
Forfeited/cancelled	(651,808)	9.21
Outstanding at December 31, 2017	445,017	\$ 8.29

RSUs are generally subject to forfeiture if employment terminates prior to the release of vesting restrictions. The compensation expense associated with RSUs with time-based and performance-based vesting conditions is measured based on the fair value market price of the Company's common shares on the grant date and is recognized on a straight-line basis over the period during which the vesting restrictions lapse.

As of December 31, 2017, the total future unrecognized compensation cost related to unvested RSUs is \$2.5 million and is expected to be recorded over a weighted average period of 1.35 years.

(d) Employee Stock Purchase Plan (ESPP)

The ESPP permits eligible employees to acquire shares of the Company's common stock through periodic payroll deductions of up to 15% of base compensation. The price at which the common stock may be purchased is 85% of the lesser of the fair market value of the Company's common stock on the first day of the applicable offering period or on the last day of the respective purchase period. The ESPP is deemed compensatory and compensation costs are recognized on a straight-line basis over the requisite service period.

The Company estimates the fair value of the ESPP shares at the date of grant using the Black-Scholes option pricing model. The assumptions are as follows:

	Year Ended December 31, 2017
Expected stock price volatility	48.51%
Risk-free interest rate	1.45%
Expected life of options (years)	0.50
Expected dividend yield	—

The weighted average per share fair value of purchase rights granted in 2017 was \$1.06. Total expense recognized for these purchase rights was less than \$0.1 million in 2017. As of December 31, 2017, no shares had been issued under the ESPP.

(e) Stock-based Compensation Expense

The Company recorded stock-based compensation expense in the consolidated statements of operations as follows:

	Year Ended December 31,		
	2017	2016	2015
	(in thousands)		
Selling, general and administrative	\$ 3,721	\$ 683	\$ 1,108
Research and development	816	155	1,267
Total stock-based compensation expense	\$ 4,537	\$ 838	\$ 2,375

### 15. Employee Benefit Plan

The Company maintains a defined contribution 401(k) plan (the “Plan”) in which substantially all of its and its subsidiaries' permanent U.S. employees are eligible to participate. Employee contributions are voluntary and are determined on an individual basis, limited by the maximum amounts allowable under the U.S. federal tax regulations. The Company makes matching contributions of 50% of the first 6% of employees' contributions to the Plan up to the maximum allowed by the Internal Revenue Service. Additionally, for certain employees outside of the U.S., the Company contributes amounts for retirement benefits required by applicable local laws. The Company recorded employer contribution expense of approximately \$0.8 million and \$0.03 million during the years ended December 31, 2017 and 2016, respectively. There was no employer contribution expense recorded during the year ended December 31, 2015.

### 16. Income Taxes

Loss before provision for income taxes, classified by source of (loss)/income, is as follow:

	Year Ended December 31,		
	2017	2016	2015
	(in thousands)		
U.S.	\$ (69,034)	\$ (9,521)	\$ —
Canada	(24,177)	(46,733)	(22,987)
Other Foreign	(32,916)	3,849	—
Loss before provision for income taxes	\$ (126,127)	\$ (52,405)	\$ (22,987)

(Provision) benefit for income taxes for the years ended December 31, 2017, 2016 and 2015 is as follows:

	Year Ended December 31,		
	2017	2016	2015
	(in thousands)		
Current (provision) benefit:			
U.S.	\$ 106	\$ (2)	\$ —
Canada	(3)	105	—
Other Foreign	(633)	(517)	—
	(530)	(414)	—
Deferred (provision):			
Canada	—	—	(22)
Other Foreign	(53)	(51)	—
	(53)	(51)	(22)
(Provision) for income taxes	\$ (583)	\$ (465)	\$ (22)

Differences between the Company's statutory income tax rates and its effective income tax rates, as applied to the loss from continuing operations before income taxes for the years ended December 31, 2017, 2016 and 2015 are reconciled as follows:

	December 31,		
	2017	2016	2015
	(in thousands)		
Canadian statutory tax rates	26%	26%	26%
Loss before income taxes	\$ (126,127)	\$ (52,405)	\$ (22,987)
Expected income tax benefit	32,793	13,625	5,977
Net increase in valuation allowance	(13,895)	(13,684)	(2,486)
Investment tax credits	(172)	868	(222)
Stock-based compensation	(270)	(133)	(606)
Foreign rate differential	1,198	532	—
U.S. and Canada rate change	(19,774)	—	—
Non-taxable expenditures	—	—	76
Change in uncertain tax positions	(20)	—	(1,784)
Adjustments to capital losses for settlement of uncertain tax positions	—	—	(560)
Other	(443)	(1,673)	(417)
(Provision) for income taxes	\$ (583)	\$ (465)	\$ (22)

Deferred taxes are recognized for temporary differences between the basis of assets and liabilities for financial statement and income tax purposes. The primary components of the Company's deferred tax assets and liabilities are comprised of the following:

	December 31,	
	2017	2016
	(in thousands)	
Deferred tax assets:		
Net operating loss carryforwards	\$ 68,915	\$ 51,808
Research and development credits	14,198	14,028
Stock-based compensation	761	108
Capitalized research expenses	1,028	2,124
Capital loss carryforwards	39,087	37,452
Depreciable and amortizable assets	10,859	13,597
Other temporary differences	11,761	13,736
Total gross deferred tax assets	146,609	132,853
Valuation allowance	(145,753)	(131,858)
Net deferred tax assets	\$ 856	\$ 995

As of December 31, 2017, the Company has a \$0.9 million net deferred tax asset attributable to its profitable foreign subsidiaries. Additionally, as of December 31, 2017, the Company has a \$145.8 million valuation allowance recorded against its U.S., Canadian and Swiss deferred tax assets. The valuation allowance increased approximately \$13.9 million during the year ended December 31, 2017, primarily due to the deferred tax assets established in connection with the Company's net operating loss carryforwards. If the Company is subsequently able to utilize all or a portion of the deferred tax assets for which the remaining valuation allowance has been established, the Company may be required to recognize these deferred tax assets through the reduction of the valuation allowance which could result in a material benefit to results of operations in the period in which the benefit is determined.

The Company has evaluated the positive and negative evidence bearing upon the realizability of its deferred tax assets and concluded that based on the Company's history of operating losses that it is more likely than not that the benefit of its U.S., Canadian and Swiss deferred tax assets will not be realized. Therefore, Company has provided a valuation allowance against its U.S., Canadian and Swiss deferred tax assets for all years presented.

As of December 31, 2017, the Company had approximately \$267.2 million of U.S., Canadian and foreign net operating losses ("NOL") of which \$60.5 million relate to the Company's U.S. subsidiaries, \$176.9 million relate to Canada and \$29.8 million relate to the Company's Swiss subsidiary. The U.S. federal NOL carryforwards will expire at various dates from 2025 through 2037, if not utilized. As of December 31, 2017, the Company also had approximately \$39.3 million of U.S. state NOL carryforwards that will expire at various dates from 2029 through 2037, if not utilized.

As of December 31, 2017, the Company also had approximately \$13.5 million of Canadian national and provincial research and development credits available for carryforward. The research and development credit carryforwards will expire at various dates through 2036. As of December 31, 2017, the Company's Canadian Scientific, Research and Experimental Development pool was \$11.4 million. Furthermore, as of December 31, 2017, the Company had approximately \$289.5 million of Canadian capital loss carryforwards, which carryforward indefinitely. The deferred tax benefit of these loss carryforwards and research and development credits is ultimately subject to final determination by the respective taxation authorities.

Upon acquiring a company that has U.S. federal and state net operating loss carryforwards and federal and state tax credits, the Company prepared an assessment to determine if it has a legal right to use the acquired net operating losses and tax credits. In performing this assessment, the Company followed the regulations within the Internal Revenue Code ("IRC") Sections 382 and 383. The Company determined that the U.S. net operating losses and tax credits acquired in the Aegerion acquisition are subject to limitations under IRC Sections 382 and 383. Due to the ownership changes, the Company determined that its U.S. subsidiaries, including Aegerion, will only be able to utilize approximately \$13.3 million of its pre-ownership change NOLs before expiration as a result of the annual Section 382 limitation. The Company has adjusted its NOL carryforward and the amounts above reflect the reduction for pre-ownership change NOLs that will expire before becoming available. The total available post-ownership change NOLs total \$47.2 million. The Company has not performed a formal analysis to determine if the post-ownership change NOLs are subject to limitation under IRC Sections 382 and 383. The U.S. subsidiaries may experience ownership changes in the future as a result of subsequent shifts in share ownership that could further limit the use of the available net operating losses and credits.

The Company recorded a provision for uncertain tax positions ("UTP") of approximately \$7.8 million offset by \$7.4 million of the Company's deferred tax assets at December 31, 2017, for a net of \$0.4 million for the provision for UTP as of December 31, 2017.

The following table summarizes the activity related to the Company's provision for UTP:

	Year Ended December 31,		
	2017	2016	2015
	(in thousands)		
Total provision for UTP as of January 1,	\$ 7,660	\$ 7,278	\$ 5,557
Current year acquisitions	—	911	—
Increases related to current year tax positions	62	—	347
Changes in tax positions of a prior period	55	—	1,934
Lapse due to statute of limitations	—	(342)	—
Settlements with taxing authorities	—	(187)	(560)
Total provision for UTP as of December 31,	7,777	7,660	7,278
Deferred tax assets available to offset provision for UTP	(7,360)	(7,279)	(6,936)
Total provision for UTP as of December 31,	\$ 417	\$ 381	\$ 342

As of December 31, 2017, 2016 and 2015, the Company has accrued an insignificant amount of interest as a result of the deferred assets available to offset its provision for UTP. The increase in provision for UTP during 2016 was the result of transfer pricing adjustments made at the Company's Swiss subsidiary as a result of its acquisition of Aegerion. During 2016, the Company settled a dispute with the Canadian Revenue Authority surrounding its capital loss carryover as a result of the remaining contingent consideration owed from the Company's previous sale of its subsidiaries, QLT USA, Inc., and Eligard® to TOLMAR Holding, Inc., which resulted in a decrease of unrecognized tax benefits. Additionally, during 2016, the period in which the 2008 Canadian income tax return was subject to reassessment expired, which resulted in a reduction of the provision for UTP surrounding its share buyback costs that arose during that tax year.

The Company and its subsidiaries file income tax returns in Canada, the U.S., and various U.S. states and in foreign jurisdictions. The Canadian income tax returns are generally subject to tax examination for the tax years ended December 31,

2011 through December 31, 2017. The U.S., U.S. state and foreign income tax returns are generally subject to tax examinations for the tax years ended December 31, 2014 through December 31, 2017. To the extent the Company has tax attribute carryforwards, the tax years in which the attribute was generated may still be adjusted upon examination by the tax authorities.

On December 22, 2017, the President of the United States signed into law the Tax Cuts and Jobs Act (the "Act"). This Act includes significant changes in U.S. tax law, including a reduction in the corporate tax rates and creating a territorial tax system with a one-time mandatory tax on previously deferred foreign earnings of U.S. subsidiaries. The Act reduced the U.S. corporate tax rate from the current rate of 35% to 21% for tax years beginning after December 31, 2017. As a result of the Act, the Company was required to revalue its existing U.S. deferred tax assets and liabilities as of December 31, 2017 from the 35% federal rate in effect through the end of 2017, to the new 21% rate. As a result of the change in law, the Company recorded a current period tax expense of \$21.5 million and a corresponding reduction in the associated valuation allowance for a net adjustment of zero to its consolidated statement of operations for the year ended December 31, 2017. The Act will require the Company to pay tax on the unremitted earnings of its foreign subsidiaries through December 31, 2017. The Company has estimated that its foreign subsidiaries are in an overall net earnings deficit and as such would have no incremental U.S. tax and therefore has recorded no tax liability on its unremitted earnings at December 31, 2017.

As a result of the enactment of the Act, the Company has reviewed the unremitted earnings of its foreign subsidiaries and estimated that because the foreign subsidiaries are in an overall earnings deficit at December 31, 2017 that there is no incremental amount of earnings to include in its U.S. taxable income, and no incremental U.S. tax due to the current year losses. As a result, the Company does not have any untaxed, undistributed earnings. However, the Company is indefinitely invested in the foreign subsidiaries, and has not provided deferred taxes for any other basis differences in its foreign subsidiaries.

The Act creates a new requirement that certain income earned by a foreign subsidiary must be included in the income of the foreign subsidiaries U.S. shareholder. This income (called Global Intangible Low-Taxed Income, or "GILTI") is defined as the excess of a foreign subsidiary's income over a nominal return on fixed assets. The Company expects to be subject to this inclusion in future years. The Company has elected to treat the effects of this provision as a period cost, and therefore has not considered the impacts of GILTI on its deferred taxes as of December 31, 2017.

The Company's preliminary estimate of the Act and the remeasurement of the Company's U.S. deferred tax assets and liabilities is subject to the finalization of the its analysis related to certain matters, such as developing interpretations of the provisions of the Act, changes to certain estimates and the filing of the Company's U.S. tax returns. U.S. Treasury regulations, administrative interpretations or court decisions interpreting the Act may require further adjustments and changes in the Company's estimates.

In conjunction with the tax law changes, the SEC staff issued Staff Accounting Bulletin No. 118 ("SAB 118") to address the application of GAAP in situations when a registrant does not have the necessary information available, prepared, or analyzed (including computations) in reasonable detail to complete the accounting for certain income tax effects of the Act. The ultimate impact may differ from these provisional amounts, possibly materially, due to, among other things, additional analysis, changes in interpretations and assumptions the Company has made, additional regulatory guidance that may be issued, and actions the Company may take as a result of the Act.

## **17. Segment Information**

The Company currently operates as one business segment, pharmaceuticals, and is focused on the development and commercialization of two commercial products. The Company's Chief Operating Officer is currently the Company's chief operating decision maker ("CODM"). The Company does not operate any separate lines of business or separate business entities with respect to its products. Accordingly, the Company does not accumulate discrete financial information with respect to separate service lines and does not have separate reportable segments. Enterprise-wide disclosures about net revenues and long-lived assets by geographic area and information relating to major customers are presented below.

### *Net Revenues*

The following table summarizes total net revenues from external customers by product and by geographic region, based on the location of the customer. In 2015, the Company did not have any commercial products and did not generate any revenues.

	Year Ended December 31, 2017			
	U.S.	Brazil	Other Foreign Countries	Total
	(in thousands)			
Lomitapide	\$ 46,431	\$ 6,659	\$ 19,040	\$ 72,130
Metreleptin	50,972	6,837	8,499	66,308
Total net revenues	<u>\$ 97,403</u>	<u>\$ 13,496</u>	<u>\$ 27,539</u>	<u>\$ 138,438</u>

  

	Year Ended December 31, 2016			
	U.S.	Brazil	Other Foreign Countries	Total
	(in thousands)			
Lomitapide	\$ 6,134	\$ —	\$ 2,487	\$ 8,621
Metreleptin	4,685	—	268	4,953
Total net revenues	<u>\$ 10,819</u>	<u>\$ —</u>	<u>\$ 2,755</u>	<u>\$ 13,574</u>

In 2017, net revenues generated from customers outside of the U.S. and Brazil, as listed in the column “Other Foreign Countries,” was primarily comprised of revenues from Japan, Argentina, Canada, Colombia, and Greece. In 2016, net revenues generated from customers outside of the U.S. were primarily derived from named patient sales in Argentina, Colombia and Italy.

#### *Significant Customers*

For the year ended December 31, 2017, two customers accounted for 70% of the Company’s net revenues; of these two customers, one customer accounted for 63% of the Company’s December 31, 2017 accounts receivable balance. For the year ended December 31, 2016, one customer accounted for 35% of the Company's net revenues, and 29% of the Company's December 31, 2016 accounts receivable balance.

#### *Long-lived Assets*

The Company’s long-lived assets are primarily comprised of intangible assets and property and equipment. As of December 31, 2017 and 2016, 100% of the Company's intangible assets were held by the Company's indirect wholly owned subsidiary, Aegerion. Of that, 65% and 66% of the intangible assets were attributable to Aegerion's U.S. business, with the remaining 35% and 34% attributable to Aegerion's European holding company as of December 31, 2017 and 2016, respectively.

As of December 31, 2017 and 2016, 75% and 93%, respectively, of the Company's property and equipment resided in the Company's U.S. subsidiaries, with the remaining 25% and 7% resided in the Company's Canadian and other foreign subsidiaries.

## **18. Commitments and Contingencies**

#### *Leases*

The Company leased certain office facilities and office equipment pursuant to operating leases during the year ended December 31, 2017. The future minimum payments for office space and office equipment over the next five years are summarized as follows:

Year Ending December 31,:	Lease Commitments	
	(in thousands)	
2018	\$	2,973
2019		949
2020		195
2021		52
2022		25
Thereafter		25
Total	\$	4,219

Rent expense pursuant to operating leases was approximately \$3.3 million , \$0.5 million and \$0.4 million for the years ended December 31, 2017 , 2016 and 2015 , respectively.

### **Other Commitments**

#### *Amgen Licensing Agreements*

*Metreleptin* . In connection with Aegerion's acquisition of MYALEPT in January 2015, Aegerion acquired a license agreement between Amgen Inc. ("Amgen") and Amylin Pharmaceuticals, Inc., dated February 7, 2006 (the "Amgen License") pursuant to which an exclusive worldwide license was obtained from Amgen to certain know-how and patents and patent applications covering the composition of matter and methods of use of metreleptin to develop, manufacture and commercialize a preparation containing metreleptin (the "Amgen Licensed Products").

As part of the Amgen License, an exclusive sublicense of Amgen's exclusive rights to certain metreleptin-related patents and patent applications owned by the Rockefeller University and exclusively licensed to Amgen under a license agreement dated April 14, 1995, as amended (the "Rockefeller License") and an exclusive sublicense of Amgen's non-exclusive rights to certain metreleptin-related patents and patent applications owned by The Regents of the University of California and non-exclusively licensed to Amgen under a license agreement dated July 13, 2005 (the "UCSF License") were obtained. Amgen retains rights to conduct research, development, manufacturing and commercialization activities with respect to products other than the Amgen Licensed Products.

Sublicenses under the licenses are permitted and are subject to certain limitations, including Amgen's right of first offer for any out-license, partnership, co-development, commercialization, co-promotion or similar agreement related to metreleptin or the Amgen Licensed Products, which expires in February 2021. Under this license agreement, Amgen must notify Aegerion of any potential third-party partnership regarding any intellectual property rights controlled by Amgen in the neurology field and Aegerion will have a right of first negotiation for any license, partnership, co-development, commercialization, co-promotion or similar agreement, which expires in February 2021.

Aegerion is required to make royalty payments to Amgen on net sales of each Amgen Licensed Product on a country-by-country basis (i) at a royalty rate in the low double digits where the Amgen Licensed Product has patent protection or market exclusivity granted by a regulatory authority at the time of regulatory approval in the applicable country during the applicable royalty term, which runs on a country-by-country basis until the later of (a) the expiration of the last-to-expire valid claim covering an Amgen Licensed Product in the applicable country, (b) expiration of any market exclusivity granted by a regulatory authority, and (c) ten years from the date on which an Amgen Licensed Product is first sold to a third party in a country after regulatory approval for the Amgen Licensed Product has been granted in such country ("Amgen Royalty Term") or (ii) at a royalty rate in the mid-single digits to low double digits where the Amgen Licensed Product receives patent protection or market exclusivity following the time of regulatory approval in the applicable country, in either case subject to a variety of customary reductions.

Under the Amgen License, Aegerion is also required to directly meet certain payment obligations under the Rockefeller License and UCSF License. Aegerion is required to make royalty payments to Rockefeller University on net sales of each product with patent rights or know-how in the field of obesity genes, obesity gene products, and molecules that modulate or mediate their action and/or regulation on a country-by-country basis at a range of royalty rates in the low single digits depending on whether the product has an orphan product designation or not until the later to occur of expiration of (i) patent protection, (ii) any market exclusivity period granted in the applicable country, or (iii) any data exclusivity period in the applicable country (with certain limitations related to the number of units sold). In February 2015, Aegerion paid a one-time \$5.0 million milestone payment to Rockefeller University, which was due twelve months following the receipt of marketing approval for MYALEPT in the U.S.

Aegerion is also required to pay to Rockefeller University a percentage in the low double digits of any upfront license fees or one-time fees Aegerion receives in consideration for any sublicense of the licensed rights. There are no material payment obligations outstanding under the UCSF License. Also, in connection with the acquisition of metreleptin, Aegerion entered into a letter agreement with AstraZeneca pursuant to which Aegerion agreed to make royalty payments payable by AstraZeneca and its affiliates to BMS with respect to net sales of metreleptin in the U.S. The time-based royalty rate ranges from mid-single digits to low double digits, increasing annually in years 2016 to 2019 from rates in the low single digits to low double digits, peaking in years 2019 to 2020 at a rate in the low double digits before decreasing in years 2022 through 2025 to rates in the high single digits to mid-single digits. The royalty obligation to BMS terminates in 2026.

The Amgen License will terminate upon the expiration of the last Amgen Royalty Term for any Amgen Licensed Product. Aegerion has the right to terminate the Amgen License for convenience upon 90 days prior written notice to Amgen or for Amgen's uncured material breach of the Amgen License, or becoming subject to specified bankruptcy or liquidation events. Amgen may terminate the Amgen License for Aegerion's uncured failure to make payments to Amgen or if Aegerion is the subject of specified bankruptcy or liquidation events.

Aegerion made royalty payments to Amgen, Rockefeller University and BMS related to the sales of MYALEPT through November 29, 2016. There were no royalty payments made to these parties from November 30, 2016 to December 31, 2016. During the year ended December 31, 2017, Aegerion made aggregate royalty payments of \$9.7 million to Amgen, Rockefeller University and BMS, and had \$2.9 million and \$2.0 million in aggregate royalties payable as of December 31, 2017 and 2016, respectively.

#### *University of Pennsylvania Licensing Agreements*

*Lomitapide*. In May 2006, Aegerion entered into a license agreement with The Trustees of the University of Pennsylvania ("UPenn") pursuant to which it obtained an exclusive, worldwide license from UPenn to certain know-how and a range of patent rights applicable to lomitapide. In particular, Aegerion obtained a license to certain patent and patent applications owned by UPenn relating to the dosing of microsomal triglyceride transfer protein inhibitors, including lomitapide, and certain patents and patent applications and know-how covering the composition of matter of lomitapide that were assigned to UPenn by BMS in the field of monotherapy or in combination with other dyslipidemic therapies, which are therapies for the treatment of patients, with abnormally high or low levels of plasma cholesterol or triglycerides.

Aegerion is obligated under this license agreement to use commercially reasonable efforts to develop, commercialize, market and sell at least one product covered by the licensed patent rights, such as lomitapide. In addition, Aegerion will be required to make specified royalty payments on net sales of products, at a range of royalty rates in the high single digits on net sales of lomitapide in countries where lomitapide has patent protection, and of any other products covered by the license (subject to a variety of customary reductions), and share with UPenn specified percentages of sublicensing royalties and certain other consideration that Aegerion receives under any sublicenses that Aegerion may grant. During the year ended December 31, 2017, Aegerion made \$3.1 million in royalty payments to UPenn. Aegerion made royalty payments to UPenn through November 29, 2016, and there were no royalty payments made from November 30, 2016 to December 31, 2016. Additionally, Aegerion accrued an additional \$0.7 million and \$1.3 million in royalties to UPenn as of December 31, 2017 and 2016.

This license agreement will remain in effect on a country-by-country basis until the expiration of the last-to-expire licensed patent right in the applicable country. Aegerion has the right to terminate this license agreement for UPenn's uncured material breach of the license agreement or for convenience upon 60 days' prior written notice to UPenn, subject to certain specific conditions and consequences. UPenn may terminate this license agreement for Aegerion's uncured material breach of the license agreement, its uncured failure to make payments to UPenn or if Aegerion is the subject of specified bankruptcy or liquidation events.

#### *Indemnities*

In connection with the sale of assets, the Company provided indemnities with respect to certain matters, including product liability, patent infringement, contractual breaches and misrepresentations, and the Company provides other indemnities to third parties under the clinical trial, license, service, supply and other agreements that it enters into in the normal course of its business. If the indemnified party were to make a successful claim pursuant to the terms of the indemnity, the Company would be required to reimburse the loss. These indemnities are generally subject to threshold amounts, specified claims periods and other restrictions and limitations. As of December 31, 2017 and 2016, no amounts have been accrued in connection with such indemnities.



### ***Development and Post Marketing Regulatory Commitments***

Novelion and Aegerion have engaged Contract Research Organizations ("CROs") to provide research, safety and project management services (the "Services") in connection with the execution of their potential clinical trials and existing registries. Services would only give rise to liabilities to the extent that services are provided to Novelion or Aegerion, as applicable, and pass through expenses are incurred. As of December 31, 2017, the Services have not yet been performed and the Company has potential commitments of approximately \$47.0 million under these agreements. The amount reflected is based on the existing contracts and does not reflect any inflation, future modification to, or termination of, the existing contracts or anticipated or potential new contracts.

### ***Legal Matters***

As of December 31, 2017 and 2016, the insurance proceeds receivable and provision for legal settlements are as follows:

	<b>December 31,</b>	
	<b>2017</b>	<b>2016</b>
	<b>(in thousands)</b>	
<b>Insurance Proceeds Receivable</b>		
Class action lawsuit insurance proceeds	\$ —	\$ 22,000
<b>Provision for Legal Settlements</b>		
Class action lawsuit settlement	\$ —	\$ (22,250)
DOJ and SEC settlement	(39,612)	(40,635)
Relator legal settlement	—	(620)
Relators legal fees	—	(405)
Other litigation settlement	—	(100)
Total provision for legal settlements	(39,612)	(64,010)
Less: Provision for legal settlements - current	(8,596)	(64,010)
Provision for legal settlements - non-current	\$ (31,016)	\$ —

### ***DOJ/SEC Investigations***

In late 2013, Aegerion received a subpoena from Department of Justice (the "DOJ"), represented by the U.S. Attorney's Office in Boston, requesting documents regarding its marketing and sale of JUXTAPID in the U.S., as well as related public disclosures. In late 2014, Aegerion received a subpoena from the Securities and Exchange Commission ("SEC") requesting certain information related to Aegerion's sales activities and disclosures related to JUXTAPID. The SEC also requested documents and information on a number of other topics, including documents related to the investigations by government authorities in Brazil into whether Aegerion's activities in Brazil violated Brazilian anti-corruption laws, and whether Aegerion's activities in Brazil violated the FCPA.

In May 2016, Aegerion reached preliminary agreements in principle with the DOJ and the SEC to resolve their investigations into U.S. commercial activities and disclosures relating to JUXTAPID. On September 22, 2017, Aegerion entered into a series of agreements in an effort to resolve investigations being conducted by the DOJ and the SEC regarding these topics. The terms of these agreements were substantially similar to the preliminary agreements in principle.

In connection with the SEC investigation, Aegerion consented to the entry of a final judgment, on September 25, 2017, in connection with a complaint filed by the SEC without admitting or denying the allegations set forth in the complaint (the "SEC Judgment"). The complaint alleged negligent violations of Sections 17(a)(2) and (3) of the Securities Act of 1933, as amended, related to certain statements made by Aegerion in 2013 regarding the conversion rate for JUXTAPID prescriptions. The SEC Judgment provides that Aegerion must pay a civil penalty in the amount of \$4.1 million, to be paid in installments over three years, plus interest on any unpaid balance at a rate of 1.75% per annum. As of December 31, 2017, \$1.2 million remains due as a current liability and \$1.6 million remains due as a non-current liability. Aegerion's payment of this civil penalty is subject to acceleration in the event of certain change of control transactions or certain transfers of Aegerion's rights in JUXTAPID or MYALEPT. Aegerion's payment schedule is also subject to acceleration in the event that Aegerion fails to satisfy its payment obligations under the SEC Judgment. The SEC Judgment was approved by a U.S. District Court judge on September 25, 2017.

In connection with the DOJ investigation, Aegerion entered into a Plea Agreement, a Deferred Prosecution Agreement (“DPA”), a Civil Settlement, certain State Settlement Agreements, and a Consent Decree of Permanent Injunction (“FDA Consent Decree”). Under the DOJ Plea Agreement, Aegerion agreed to plead guilty to two misdemeanor misbranding violations of the Federal Food, Drug, and Cosmetic Act (“FDCA”). On November 20, 2017, the U.S. District Court rejected Aegerion’s Plea Agreement. On January 12, 2018, Aegerion entered into a new Plea Agreement with the DOJ. On January 30, 2018, a U.S. District Court Judge sentenced Aegerion after accepting Aegerion’s guilty criminal plea. The Court did not impose a criminal fine and instead ordered Aegerion to pay restitution, in the amount of \$7.2 million payable over three years, plus interest on any unpaid balance at a rate of 1.75% per annum, into a fund managed by an independent claims administrator. Of the total \$7.2 million of restitution fund, \$2.9 million is recorded as a current liability and \$4.3 million is recorded as a non-current liability. As contemplated by the Plea Agreement, Aegerion was further sentenced to a three -year term of probation. Among the terms of probation, Aegerion must (i) comply with federal, state and local laws, (ii) pay restitution in accordance with the payment schedule set by the Court, (iii) notify its probation officer of any prosecution, major civil litigation or administrative proceeding, (iv) seek permission of its probation officer prior to selling, assigning or transferring assets, (v) notify its probation officer of any material change in its economic circumstances, (vi) forbear from disparaging the factual basis of Aegerion’s plea or denying that Aegerion itself is guilty, and (vii) comply with the DPA and CIA. Reports prepared by the independent review organization and FDA Auditor, as discussed below, must be provided to its probation officer. Under the terms of the DPA, Aegerion admitted it engaged in conduct that constituted a conspiracy to violate the HIPAA. The DPA provides that Aegerion must continue to cooperate fully with the DOJ concerning its investigation into other individuals or entities. The DPA provides that Aegerion must maintain a robust Compliance and Ethics Program (as defined in the DPA) that requires, among other things, a designated Compliance Officer and Compliance Committee; written compliance policies and procedures; a training program focused on Aegerion’s compliance policies and procedures; a disclosure program to allow individuals to report potential legal and/or compliance violations, including violations of HIPAA; a non-retaliation policy; and a monitoring and auditing program. Under the DPA, Aegerion, as well as the Board of Directors of the Company (or a designated committee thereof), must also conduct regular reviews of its Compliance and Ethics Program, provide certifications to the DOJ that the program is believed to be effective and notify the DOJ of any probable violations of HIPAA. In the event Aegerion breaches the DPA, there is a risk the government would seek to impose remedies provided for in the DPA, including instituting criminal prosecution against Aegerion and/or seeking to impose stipulated penalties against Aegerion. The DPA is subject to review and supervision by a U.S. District Court judge.

Aegerion also entered into the DOJ Civil Settlement Agreement to resolve allegations by the DOJ that false claims for JUXTAPID were submitted to governmental healthcare programs. The DOJ Civil Settlement Agreement requires Aegerion to pay a civil settlement in the amount of \$28.8 million , which includes up to \$2.7 million designated for certain U.S. states relating to Medicaid expenditures for JUXTAPID, to be paid in installments over three years. In addition, a \$0.8 million of interest under the preliminary agreements in principle with the DOJ has been paid subsequent to December 31, 2017. Of the \$28.8 million , \$3.7 million is recorded as a current liability and \$25.1 million is recorded as a non-current liability. Aegerion’s payment of this civil settlement amount is subject to acceleration in the event of certain change of control transactions or certain transfers of Aegerion’s rights in JUXTAPID or MYALEPT. In the event that Aegerion fails to satisfy its obligations under the DOJ Civil Settlement Agreement, Aegerion could be subject to additional penalties or litigation.

Aegerion also agreed to enter into the State Settlement Agreements to resolve claims under state law analogues to the federal False Claims Act. The terms of the State Settlement Agreements are substantially similar to those set forth in the DOJ Civil Settlement Agreement. As noted above, participating states will receive up to \$2.7 million in the aggregate from the \$28.8 million amount to be paid pursuant to the DOJ Civil Settlement Agreement.

Aegerion also agreed to the FDA Consent Decree with the DOJ and the FDA to resolve a separate civil complaint alleging that the company violated the FDCA by failing to comply with the JUXTAPID REMS program and the requirement to provide adequate directions for all of the uses for which it distributed JUXTAPID. The FDA Consent Decree requires Aegerion, among other things, to comply with the JUXTAPID REMS program; retain a qualified independent auditor to conduct annual audits of its compliance with the JUXTAPID REMS program; and remediate any noncompliance identified by the auditor within specified timeframes. In the event Aegerion fails to comply with the JUXTAPID REMS program or any other provisions of the FDA Consent Decree, Aegerion could be subject to additional administrative remedies, civil or criminal penalties and/or stipulated damages. Aegerion is required to notify the FDA in advance of certain changes in control, or changes in its business that may affect its operations, assets, rights or liabilities in the United States. The FDA Consent Decree does not take effect until it is approved by the Court and the injunction order is issued.

Separately, Aegerion entered into a Corporate Integrity Agreement (“CIA”) with the Department of Human Services Office of the Inspector General (“OIG”). The CIA requires Aegerion, among other things, to maintain a Compliance Program (as defined in the CIA) that includes: the designation of a Compliance Officer and a Compliance Committee; comprehensive written policies and procedures regarding the operation of the Compliance Program and appropriate conduct related to sales, marketing,

reimbursement, incentive compensation and other matters; training and education regarding the Compliance Program and requirements of the CIA; a centralized annual risk assessment and mitigation process; an independent review and analysis of Aegerion's systems, transactions, risk assessment and mitigation process and other compliance activities; a disclosure program that allows individuals to report issues or questions associated with Aegerion's policies, conduct, practices or procedures; a field force monitoring program to evaluate and monitor sales personnel's interactions with healthcare professionals; monitoring of non-promotional activities, including consultants, donations to independent third-party patient assistance programs and other types of grants; certain requirements for the variable compensation programs for its U.S. sales personnel; and an executive financial recoupment program that puts at risk of forfeiture and recoupment performance pay for certain of Aegerion's and the Company's executives. Under the CIA, Aegerion, as well as the Board of Directors of the Company (or a designated committee thereof), must also conduct regular reviews of Aegerion's Compliance Program and provide an annual resolution or certification to OIG that the program is believed to be effective. Additionally, Aegerion must obtain management certifications from certain employees who are expected to monitor and oversee Aegerion's activities, which must be provided to OIG. Aegerion has reporting obligations under the CIA, including with respect to any ongoing investigation or legal proceeding involving an allegation that Aegerion has engaged in any fraudulent activities or committed a crime, any communications with FDA regarding improper promotion or marketing of Aegerion's products and any probable violations of criminal, civil or administrative laws applicable to federal healthcare programs. In the event Aegerion breaches the CIA, there is a risk the government would seek to impose remedies provided for in the CIA, including seeking to impose stipulated penalties against Aegerion and/or seeking to exclude Aegerion from participation in federal healthcare programs.

#### *Investigations in Brazil*

Federal and state authorities in Brazil are conducting an investigation to determine whether there have been violations of Brazilian laws related to the sales of JUXTAPID in Brazil. In July 2016, the Ethics Council of Interfarma fined Aegerion's subsidiary in Brazil ("Aegerion Brazil") approximately \$0.5 million for violations of the industry association's Code of Conduct, to which Aegerion Brazil is bound due to its affiliation with Interfarma. Also, the Board of Directors of Interfarma imposed an additional penalty of suspension of Aegerion Brazil's membership, without suspension of Aegerion Brazil's membership contribution, for a period of 180 days for Aegerion Brazil to demonstrate the implementation of effective measures to cease alleged irregular conduct, or exclusion of the Company's membership in Interfarma if such measures are not implemented. Aegerion Brazil paid approximately \$0.5 million related to this fine during the third quarter of 2016. In March 2017, after the suspension period ended, Interfarma's Board of Directors decided to reintegrate Aegerion Brazil, enabling it to participate regularly in Interfarma activities, subject to meeting certain obligations. Also, in July 2016, Aegerion Brazil received an inquiry from a Public Prosecutor Office of the Brazilian State of Paraná asking it to respond to questions related to media coverage regarding JUXTAPID and its relationship with a patient association to which Aegerion made donations for patient support. This preliminary inquiry was later reclassified as a civil inquiry, which is a preliminary procedure by the Public Prosecutor's Office that aims to verify if there are enough elements for it to file a formal lawsuit or to dismiss the inquiry. In June 2017, the Federal Public Prosecutor of the City of São José dos Campos, State of São Paulo, requested that a Brazilian federal court provide federal investigators with access to the bank records of certain individuals and entities, including Aegerion Brazil, certain former Aegerion Brazil employees, a Brazilian patient association, and certain Brazilian physicians. The Court has not yet ruled on the Federal Public Prosecutor's request. The Public Prosecutors in Paraná and São José dos Campos continue to gather information in connection with their respective investigations. At this time, the Company does not know whether the inquiries of the Public Prosecutors in Paraná or São José dos Campos will result in the commencement of any formal proceeding against Aegerion, but if Aegerion's activities in Brazil are found to violate any laws or governmental regulations, Aegerion may be subject to significant civil lawsuits to be filed by the Public Prosecution office, and administrative penalties imposed by Brazilian regulatory authorities and additional damages and fines. Under certain circumstances, Aegerion could be barred from further sales to federal and/or state governments in Brazil, including sales of JUXTAPID and/or MYALEPT, due to penalties imposed by Brazilian regulatory authorities or through civil actions initiated by federal or state public prosecutors. As of the filing date of this Annual Report, the Company cannot determine if a loss is probable as a result of the investigations and inquiry in Brazil and whether the outcome will have a material adverse effect on the Company's business and, as a result, no amounts have been recorded for a loss contingency.

#### *Shareholder Class Action Lawsuit*

In January 2014, a putative class action lawsuit was filed against Aegerion and certain of its former executive officers in the U.S. District Court for the District of Massachusetts alleging certain misstatements and omissions related to the marketing of JUXTAPID and Aegerion's financial performance in violation of the federal securities laws. On July 22, 2016, co-lead plaintiffs and defendants filed a joint motion to stay the briefing schedule while they pursued mediation, which the Court granted on August 10, 2016. Through mediation, the co-lead plaintiffs and defendants reached an agreement in principle to settle the litigation on November 29, 2016. On January 17, 2017, the co-lead plaintiffs filed a stipulation of settlement with the Court that contained the settlement terms as agreed upon by the parties, including that Aegerion and its insurance carriers would contribute \$22.3 million to a settlement fund for the putative class. The insurance carriers agreed to cover \$22.0 million of this amount, with Aegerion

responsible for the remainder of \$0.3 million. On June 29, 2017, the Court entered an order preliminarily approving the settlement. Aegerion and its insurance carriers contributed their respective portions of the settlement fund as of July 14, 2017. Class members had until October 31, 2017, to object to or file objections or postmark requests to opt-out of the settlement. No class members filed objections to the settlement by the October 31 deadline. On November 30, 2017, the Court approved the settlement and dismissed the action in its entirety with prejudice.

#### *Qui Tam Litigation*

In March 2014, an amended qui tam complaint was filed under seal in the District of Massachusetts against Aegerion, two former executive officers and a former employee. *United States ex rel Clarke v. Aegerion Pharm. Inc.*, No. 13-cv-11785-IT. On September 22, 2017, the U.S. filed a notice of intervention as to Aegerion. On September 27, 2017, the qui tam relators filed a second amended complaint naming additional parties, including a former board member, former executives, and former employees of Aegerion, as well as other third parties. The second amended complaint noted that the relators would file a joint stipulation of dismissal with respect to Aegerion upon the completion of certain conditions set forth in the Civil Settlement Agreement. On October 27, 2017, the court granted Aegerion and relators' joint motion to stay proceedings until sentencing in the criminal matter is complete. On February 20, 2018, Aegerion was dismissed from the qui tam lawsuit.

#### **Contingent Consideration Receivable**

##### *Related to the Sale of Visudyne®*

On September 24, 2012, the Company completed the sale of its Visudyne business to Valeant Pharmaceuticals International, Inc. ("Valeant"). Subject to the achievement of certain future milestones, the Company was eligible to receive the following additional consideration: (i) a milestone payment of \$5.0 million if receipt of the registration required for commercial sale of the Qcellus™ laser in the U.S. (the Laser Registration) is obtained by December 31, 2013, \$2.5 million if the Laser Registration is obtained after December 31, 2013 but before January 1, 2015, and \$0 if the Laser Registration is obtained thereafter (the "Laser Earn-Out Payment"); (ii) up to \$5.0 million in each calendar year commencing January 1, 2013 (up to a maximum of \$15.0 million in the aggregate) for annual net royalties exceeding \$8.5 million pursuant to the Amended and Restated PDT Product Development, Manufacturing and Distribution Agreement with Novartis Pharma AG (the "Novartis Agreement") or from other third-party sales of Visudyne outside of the U.S.; and (iii) a royalty on net sales attributable to new indications for Visudyne, if any should be approved by the FDA.

On September 26, 2013, the FDA approved the premarket approval application ("PMA") supplement for the Qcellus laser and on October 10, 2013, the Company invoiced Valeant for the \$5.0 million Laser Earn-Out Payment. Valeant subsequently disputed payment on the basis that it believes the Laser Earn-Out Payment remains contingent upon receipt of additional governmental authorizations with regard to the Qcellus laser. As a result, on September 22, 2015 the Company commenced an action in the Supreme Court of British Columbia against Valeant for breach of contract. In December 2017, we agreed to a settlement of this litigation in exchange for a settlement payment from Valeant of \$0.5 million, and such receivable was recorded as other income in our Consolidated Statement of Operations for the year ended December 31, 2017.

##### *Related to the Sale of the PPDS Technology*

On April 3, 2013, Novelion completed the sale of its punctal plug drug delivery system technology for approximately \$1.3 million (the "PPDS Technology") to Mati Therapeutics Inc. ("Mati") pursuant to the terms of Novelion's asset purchase agreement with Mati (the "Mati Agreement"). Under the terms of the Mati Agreement, Novelion is eligible to receive future potential payments upon completion of certain product development and commercialization milestones that could reach \$19.5 million (or exceed that amount if more than two products are commercialized), a low single digit royalty on worldwide net sales of all products using or developed from the PPDS Technology and a fee on payments received by Mati in respect of the PPDS Technology other than net sales revenues. For the years ended December 31, 2017 and 2016, the Company received no proceeds related to the collection of this contingent consideration.

#### **19. Subsequent Events**

On March 15, 2018, Aegerion entered into a new loan and security agreement (the "New Loan Agreement") with affiliates of Broadfin Capital, LLC ("Broadfin Capital") and Sarissa Capital Management LP ("Sarissa Capital" and, together with Broadfin Capital, the "Lenders"), pursuant to which the Lenders provided a single-draw term loan to Aegerion in an aggregate amount of \$20.0 million (the "New Loan"), and secured by substantially all of Aegerion's assets, subordinated to the intercompany loan entered between QLT and Aegerion on June 14, 2016 (which intercompany loan was amended and restated in connection with Aegerion's entry into the New Loan Agreement). Interest on the New Loan accrues at 9.00% per annum and the New Loan matures on the earliest of (i) August 1, 2019, (ii) 30 days prior to the maturity date of the Convertible Notes, (iii) the date that any restructuring

or recapitalization of all or substantially all of the Convertible Notes, including any exchange offer or similar transaction, is substantially consummated, and (iv) upon acceleration of the obligations under the New Loan Agreement. Following an event of default and so long as an event of default is continuing the interest rate would increase by 3% per annum. Interest will accrue and compound quarterly in arrears and is not be payable in cash until the New Loan Maturity Date or any earlier time that interest and principal become due and payable under the New Loan. The New Loan may be prepaid, in whole or in part, by Aegerion at any time without premium or penalty. The Lenders or their affiliates are also investors in the Company's common shares, and two members of our Board of Directors are affiliates of the Lenders.

In connection with the New Loan Agreement, the Lenders were issued warrants to purchase approximately 1.8 million Novelion common shares. The warrants have an exercise price equal to \$4.40 per share, representing the volume weighted average price of Novelion common shares for the 20 trading days ending March 14, 2018, and have a term of four years.

## 20. Selected Quarterly Financial Data (unaudited)

The following table contains quarterly financial information for the years ended 2017 and 2016.

	Quarter Ended			
	December 31, 2017	September 31, 2017	June 30, 2017	March 31, 2017
	(in thousands, except per share amounts)			
Net revenues	\$ 38,908	\$ 28,669	\$ 40,877	\$ 29,984
Cost of product sales	16,993	29,505	14,277	16,445
Loss from operations	(13,964)	(39,399)	(11,772)	(21,663)
Net loss	(24,568)	(49,744)	(21,436)	(30,962)
Net loss per common share - basic and diluted	(1.32)	(2.67)	(1.15)	(1.67)

	Quarter Ended			
	December 31, 2016	September 31, 2016	June 30, 2016	March 31, 2016
	(in thousands, except per share amounts)			
Net revenues	\$ 13,574	\$ —	\$ —	\$ —
Cost of product sales	5,971	—	—	—
Loss from operations	(14,360)	(6,017)	(7,403)	(8,926)
Net loss	(19,920)	(5,936)	(5,120)	(21,894)
Net loss per common share - basic and diluted	(1.48)	(0.55)	(0.50)	(2.05)

### Item 9. Changes in and Disagreements With Accountants on Accounting and Financial Disclosure.

None.

### Item 9A. Controls and Procedures.

#### Disclosure Controls and Procedures

As initially described in Management's Report on Internal Control over Financial Reporting for the year ended December 31, 2016, we identified and concluded that there was a material weakness in our internal controls over the financial reporting process as we did not design and maintain sufficiently precise or effective review and approval controls over the forecasts used to develop management estimates, including those related to balances acquired in the Aegerion business combination that occurred during fiscal year 2016.

As required by Rules 13a-15(e) and 15d-15(e) under the Securities Exchange Act of 1934, as amended (the "Exchange Act"), management, with the participation of our principal executive officer and our principal financial officer, evaluated, as of the end of the period covered by this Annual Report, the effectiveness of our disclosure controls and procedures. Based on that evaluation, our principal executive officer and principal financial officer concluded that our disclosure controls and procedures as of such date were not effective due to the material weakness in our internal control over financial reporting described above. Notwithstanding the material weakness in our internal control over financial reporting, the consolidated financial statements

included in this Annual Report on Form 10-K fairly present, in all material respects, our financial position, results of operations and cash flows for the periods presented in conformity with accounting principles generally accepted in the United States of America.

### **Management's Report on Internal Control over Financial Reporting**

Our management is responsible for establishing and maintaining adequate internal control over financial reporting (as defined in Rule 13a-15(f) under the Exchange Act). Our internal control over financial reporting is a process designed under the supervision of our principal executive officer and principal financial officer to provide reasonable assurance regarding the reliability of financial reporting and the preparation of our financial statements for external purposes in accordance with accounting principles generally accepted in the United States. Management evaluated the effectiveness of our internal control over financial reporting using the criteria set forth by the Committee of Sponsoring Organizations of the Treadway Commission (COSO) in Internal Control-Integrated Framework (2013).

Management, under the supervision and with the participation of the principal executive officer and principal financial officer, assessed the effectiveness of our internal control over financial reporting. In the course of completing its assessment of internal control over financial reporting as of December 31, 2016, management determined that there was a material weakness in our controls over the financial reporting process as we did not design and maintain sufficiently precise or effective review and approval controls over the forecasts used to develop management estimates, including those related to balances acquired in the Aegerion business combination that occurred during fiscal year 2016.

While the Company has implemented a number of remediation efforts over the course of 2017 with respect to the material weakness, the Company has not remediated the material weakness in connection with the Company's assessment of internal control over financial reporting as of December 31, 2017 because there has been a lack of instances to test the remediated controls as of December 31, 2017 to conclude that the controls are operating effectively. As a result, management concluded that we did not maintain effective internal control over financial reporting as of December 31, 2017.

Our independent registered public accounting firm, Deloitte & Touche LLP, has issued an adverse opinion on the effectiveness of our internal control over financial reporting as of December 31, 2017. Their report appears below.

### **Remediation Plan**

The Company is committed to remediate the control deficiencies that constituted the above material weakness by implementing changes to its internal control over financial reporting. Management is responsible for implementing changes and improvements in the internal control over financial reporting and for remediating the control deficiencies that gave rise to the material weakness. To remediate the material weakness described above, the Company has enhanced and implemented controls and processes to properly document the qualitative and quantitative assumptions to be used in forecasting, as well as, properly documenting management's review and approval of forecasts.

### **Changes to Internal Controls over Financial Reporting**

Except for the continued remediation efforts of the previously identified material weakness, there were no changes in our internal control over financial reporting that occurred during the year ended December 31, 2017 that materially affected, or were reasonably likely to materially affect, our internal control over financial reporting.

### **Item 9B. Other Information.**

None.

## Report of Independent Registered Public Accounting Firm

To the Board of Directors and Shareholders of  
Novelion Therapeutics Inc.

### Opinion on Internal Control over Financial Reporting

We have audited the internal control over financial reporting of Novelion Therapeutics Inc. and subsidiaries (the "Company") as of December 31, 2017, based on criteria established in *Internal Control - Integrated Framework (2013)* issued by the Committee of Sponsoring Organizations of the Treadway Commission (COSO). In our opinion, because of the effect of the material weakness identified below on the achievement of the objectives of the control criteria, the Company has not maintained effective internal control over financial reporting as of December 31, 2017, based on criteria established in *Internal Control - Integrated Framework (2013)* issued by COSO.

We have also audited, in accordance with the standards of the Public Company Accounting Oversight Board (United States) (PCAOB), the consolidated financial statements as of and for the year ended December 31, 2017, of the Company and our report dated March 16, 2018, expressed an unqualified opinion on those financial statements.

### Basis for Opinion

The Company's management is responsible for maintaining effective internal control over financial reporting and for its assessment of the effectiveness of internal control over financial reporting, included in the accompanying Management's Report on Internal Control over Financial Reporting. Our responsibility is to express an opinion on the Company's internal control over financial reporting based on our audit. We are a public accounting firm registered with the PCAOB and are required to be independent with respect to the Company in accordance with the U.S. federal securities laws and the applicable rules and regulations of the Securities and Exchange Commission and the PCAOB.

We conducted our audit in accordance with the standards of the PCAOB. Those standards require that we plan and perform the audit to obtain reasonable assurance about whether effective internal control over financial reporting was maintained in all material respects. Our audit included obtaining an understanding of internal control over financial reporting, assessing the risk that a material weakness exists, testing and evaluating the design and operating effectiveness of internal control based on the assessed risk, and performing such other procedures as we considered necessary in the circumstances. We believe that our audit provides a reasonable basis for our opinion.

### Definition and Limitations of Internal Control over Financial Reporting

A company's internal control over financial reporting is a process designed to provide reasonable assurance regarding the reliability of financial reporting and the preparation of financial statements for external purposes in accordance with generally accepted accounting principles. A company's internal control over financial reporting includes those policies and procedures that (1) pertain to the maintenance of records that, in reasonable detail, accurately and fairly reflect the transactions and dispositions of the assets of the company; (2) provide reasonable assurance that transactions are recorded as necessary to permit preparation of financial statements in accordance with generally accepted accounting principles, and that receipts and expenditures of the company are being made only in accordance with authorizations of management and directors of the company; and (3) provide reasonable assurance regarding prevention or timely detection of unauthorized acquisition, use, or disposition of the company's assets that could have a material effect on the financial statements.

Because of its inherent limitations, internal control over financial reporting may not prevent or detect misstatements. Also, projections of any evaluation of effectiveness to future periods are subject to the risk that controls may become inadequate because of changes in conditions, or that the degree of compliance with the policies or procedures may deteriorate.

### Material Weakness

A material weakness is a deficiency, or a combination of deficiencies, in internal control over financial reporting, such that there is a reasonable possibility that a material misstatement of the company's annual or interim financial statements will not be prevented or detected on a timely basis. The following material weakness has been identified and included in management's assessment: the Company did not design and maintain sufficiently precise or effective review and approval controls over the forecasts used to develop management estimates, including those related to balances acquired in the Aegerion business combination that occurred during fiscal year 2016. This material weakness was considered in determining the nature, timing, and extent of audit tests applied in our audit of the consolidated financial statements as of and for the year ended December 31, 2017, of the Company, and this report does not affect our report on such financial statements.

/s/ Deloitte & Touche LLP

Boston, Massachusetts  
March 16, 2018

### PART III

**Item 10. Directors, Executive Officers and Corporate Governance.**

The information required by this item will be contained in our definitive proxy statement, which we plan to file with the SEC in connection with our 2018 Annual Meeting of Shareholders within 120 days of the end of the fiscal year ended December 31, 2017 (the "Proxy Statement"). Such information is incorporated herein by reference.

**Code of Ethics**

Our Board of Directors has adopted a code of conduct (the "Code") that applies to our directors, officers and employees. The Code is available on the corporate governance section of our website (which is a subsection of the "Investors" section of our website) at the following address: [www.novelion.com](http://www.novelion.com). We intend to disclose on our website any amendments or waivers to the Code that are required to be disclosed by SEC rules. You may also request a printed copy of the Code, without charge, by writing to us at Novelion Therapeutics Inc., 1800-510 West Georgia Street, Vancouver, B.C., Canada V6B 0M3 Attn: Investor Relations.

**Item 11. Executive Compensation.**

The information required by this item will be contained in our Proxy Statement. Such information is incorporated herein by reference.

**Item 12. Security Ownership of Certain Beneficial Owners and Management and Related Shareholder Matters.**

The information required by this item will be contained in our Proxy Statement. Such information is incorporated herein by reference.

**Item 13. Certain Relationships and Related Transactions, and Director Independence.**

The information required by this item will be contained in our Proxy Statement. Such information is incorporated herein by reference.

**Item 14. Principal Accounting Fees and Services.**

The information required by this item will be contained in our Proxy Statement. Such information is incorporated herein by reference.



**PART IV**

**Item 15. Exhibits, Financial Statement Schedules.**

(a) The following documents are filed as part of this Report:

1. Financial statements (see Item 8).
2. All information is included in the financial statements or notes thereto.
3. Exhibits:

See Exhibit Index.

**Item 16. Form 10-K Summary.**

Not applicable.

## EXHIBIT INDEX

The exhibits listed below are filed as part of this Annual Report. References under the caption “Location” to exhibits or other filings indicate that the exhibit or other filing has been filed, that the indexed exhibit and the exhibit or other filing referred to are the same and that the exhibit or other filing referred to is incorporated by reference.

### **Table of Contents**

<b>Exhibit</b>	<b>Description of Document</b>	<b>Location</b>
<a href="#"><u>2.1</u></a>	# Asset Purchase Agreement, dated September 21, 2012, by and between the Company and Valeant.	Exhibit 10.65 to the Company’s Current Report on Form 8-K, filed with the SEC on September 27, 2012.
<a href="#"><u>2.2</u></a>	# Asset Purchase Agreement, dated November 5, 2014, by and among Aegerion Pharmaceuticals, Inc., Amylin Pharmaceuticals, LLC and, solely for purposes of Sections 2.1.1, 2.2.1 and 2.3.2, AstraZeneca Pharmaceuticals LP.	Exhibit 10.29 to Aegerion Pharmaceuticals, Inc.’s Amendment No. 1 to the Annual Report on Form 10-K, filed with the SEC on July 7, 2015.
<a href="#"><u>2.3</u></a>	First Amendment to Asset Purchase Agreement dated January 9, 2015, by and among Aegerion Pharmaceuticals, Inc., Amylin Pharmaceuticals, LLC and, solely for purposes of Sections 2.1.1, 2.2.1 and 2.3.2, AstraZeneca Pharmaceuticals LP.	Exhibit 10.30 to Aegerion Pharmaceuticals, Inc.’s Annual Report on Form 10-K, filed with the SEC on March 2, 2015.
<a href="#"><u>2.4</u></a>	Share Purchase Agreement, dated June 8, 2015, by and among the Company, Broadfin Healthcare Master Fund, Ltd., JW Partners, LP, JW Opportunities Fund, LLC, EcoR1 Capital Fund Qualified, L.P. and EcoR1 Capital Fund, L.P.	Exhibit 2.3 to the Company’s Current Report on Form 8-K, filed with the SEC on June 12, 2015.
<a href="#"><u>2.5</u></a>	Share Purchase and Registration Rights Agreement, dated June 8, 2015, by and among the Company, Broadfin Healthcare Master Fund, Ltd., JW Partners, LP, JW Opportunities Fund, LLC, EcoR1 Capital Fund Qualified, L.P. and EcoR1 Capital Fund, L.P.	Exhibit 2.4 to the Company’s Current Report on Form 8-K, filed with the SEC on June 12, 2015.
<a href="#"><u>2.6</u></a>	Letter agreement, dated June 8, 2015, by and among the Company, Broadfin Healthcare Master Fund, Ltd., JW Partners, LP, JW Opportunities Fund, LLC, EcoR1 Capital Fund Qualified, L.P. and EcoR1 Capital Fund, L.P.	Exhibit 2.5 to the Company’s Current Report on Form 8-K, filed with the SEC on June 12, 2015.
<a href="#"><u>2.7</u></a>	Amended and Restated Share Subscription Agreement, dated December 7, 2015, among the Company, Tribute Pharmaceuticals Canada Inc., POZEN Inc., Aralez Pharmaceuticals, Inc., Aralez Pharmaceuticals Plc, Deerfield Private Design Fund III, L.P., Deerfield International Master Fund, L.P., Deerfield Partners, L.P., Broadfin Healthcare Master Fund, Ltd., JW Partners, LP, JW Opportunities Fund, LLC, and J.W. Opportunities Master Fund, Ltd.	Exhibit 10.1 to the Company’s Current Report on Form 8-K, filed with the SEC on December 11, 2015.
<a href="#"><u>2.8</u></a>	Unit Subscription Agreement, dated June 14, 2016, by and among the Company, Deerfield International Master Fund, L.P., Deerfield Partners, L.P., Broadfin Healthcare Master Fund, Ltd., JW Partners LP, JW Opportunities Master Fund, Ltd., The K2 Principal Fund L.P., Healthcare Value Partners, L.P., Tiger Legatus Capital Management, LLC, Sarissa Capital Domestic Fund LP, Sarissa Capital Offshore Master Fund LP, Armistice Capital Master Fund, Ltd., Levcap Alternative Fund, L.P., Ulysses Partners, L.P., Ulysses Offshore Fund, Ltd. and Jason Aryeh, as amended as applied to Broadfin Healthcare Master Fund, Ltd. on September 9, 2016.	Exhibit 10.1 to the Company’s Current Report on Form 8-K, filed with the SEC on December 5, 2016.
<a href="#"><u>2.9</u></a>	Agreement and Plan of Merger, dated June 14, 2016, and Amendment No. 1 thereto, by and among Aegerion Pharmaceuticals, Inc., the Company and Isotope Acquisition Corp.	Annex A to the Company’s Amendment No. 1 to the Registration Statement on Form S-4, filed with the SEC on September 12, 2016.
<a href="#"><u>3.1</u></a>	Articles of the Company, dated May 25, 2005.	Exhibit 3.2 to the Company’s Current Report on Form 8-K, filed with the SEC on June 1, 2005.
<a href="#"><u>3.2</u></a>	Notice of Articles of the Company, dated November 29, 2016.	Exhibit 3.1 to the Company’s Current Report on Form 8-K, filed with the SEC on December 5, 2016.

<b>Exhibit</b>	<b>Description of Document</b>	<b>Location</b>
<a href="#">4.1</a>	Specimen Common Share Certificate of the Company.	Exhibit 4.1 to the Company's Current Report on Form 8-K, filed with the SEC on December 16, 2016.
<a href="#">4.2</a>	Indenture, dated August 15, 2014, by and between Aegerion Pharmaceuticals, Inc. and The Bank of New York Mellon Trust Company, N.A., as Trustee, relating to the 2.00% Convertible Senior Notes Due 2019.	Exhibit 4.1 to Aegerion Pharmaceuticals, Inc.'s Current Report on Form 8-K, filed with the SEC on August 15, 2014.
<a href="#">4.3</a>	Supplemental Indenture, dated November 29, 2016, by and between Aegerion Pharmaceuticals, Inc. and The Bank of New York Mellon Trust Company, N.A., as Trustee, relating to the 2.00% Convertible Senior Notes Due 2019.	Exhibit 4.1 to Aegerion Pharmaceuticals, Inc.'s Current Report on Form 8-K, filed with the SEC on November 29, 2016.
<a href="#">4.4</a>	Amended and Restated Supplemental Indenture, by and among Aegerion Pharmaceuticals, Inc, the Company, and The Bank of New York Mellon Trust Company, N.A., dated as of May 8, 2017.	Exhibit 4.1 to the Company's Quarterly Report on Form 10-Q, filed with the SEC on May 9, 2017.
<a href="#">10.1</a>	# License Agreement, dated February 7, 2006, by and between Amylin Pharmaceuticals, LLC and Amgen Inc.	Exhibit 10.32 to Aegerion Pharmaceuticals, Inc.'s Annual Report on Form 10-K, filed with the SEC on March 2, 2015.
<a href="#">10.2</a>	# Patent License Agreement, dated May 19, 2006, as amended September 27, 2006, by and between Aegerion Pharmaceuticals, Inc. and University of Pennsylvania.	Exhibit 10.6 to Aegerion Pharmaceuticals, Inc.'s Registration Statement on Form S-1, as amended, filed with the SEC on August 10, 2010.
<a href="#">10.3</a>	# License Agreement, dated July 8, 2009, by and between Amylin Pharmaceuticals, LLC and Shionogi & Co., Ltd.	Exhibit 10.31 to Aegerion Pharmaceuticals, Inc.'s Annual Report on Form 10-K, filed with the SEC on March 2, 2015.
<a href="#">10.4</a>	Lease, dated January 1, 2011, by and between Aegerion Pharmaceuticals, Inc. and RREEF America REIT II CORP. PPP.	Exhibit 10.1 to Aegerion Pharmaceuticals, Inc.'s Current Report on Form 8-K, filed with the SEC on January 6, 2011.
<a href="#">10.5</a>	First Amendment to Lease, dated November 7, 2011, by and between Aegerion Pharmaceuticals, Inc. and RREEF America REIT II CORP. PPP.	Exhibit 10.22 to Aegerion Pharmaceuticals, Inc.'s Annual Report on Form 10-K, filed with the SEC on March 15, 2012.
<a href="#">10.6</a>	Second Amendment to Lease, dated September 4, 2012, by and between Aegerion Pharmaceuticals, Inc. and RREEF America REIT II Corp. PPP.	Exhibit 10.1 to Aegerion Pharmaceuticals, Inc.'s Quarterly Report on Form 10-Q, filed with the SEC on November 9, 2012.
<a href="#">10.7</a>	Third Amendment to Lease, dated June 19, 2013, by and between Aegerion Pharmaceuticals, Inc. and RREEF America REIT II Corp. PPP.	Exhibit 10.1 to Aegerion Pharmaceuticals, Inc.'s Quarterly Report on Form 10-Q, filed with the SEC on August 9, 2013.
<a href="#">10.8</a>	Fourth Amendment to Lease, dated January 1, 2014, by and between Aegerion Pharmaceuticals, Inc. and RREEF America REIT II Corp. PPP.	Exhibit 10.25 to Aegerion Pharmaceuticals, Inc.'s Annual Report on Form 10-K, filed with the SEC on March 3, 2014.
<a href="#">10.9</a>	Fifth Amendment to Lease, dated July 19, 2016, by and between Aegerion Pharmaceuticals, Inc. and RREEF America REIT II Corp. PPP.	Exhibit 10.3 to Aegerion Pharmaceuticals, Inc.'s Quarterly Report on Form 10-Q, filed with the SEC on November 4, 2016.
<a href="#">10.10</a>	Loan and Security Agreement, dated June 14, 2016, by and between the Company and Aegerion Pharmaceuticals, Inc.	Exhibit 10.2 to Aegerion Pharmaceuticals, Inc.'s Current Report on Form 8-K, filed with the SEC on June 15, 2016.
<a href="#">10.11</a>	Letter agreement, dated December 7, 2015, by and among the Company, Broadfin Healthcare Master Fund, Ltd., JW Partners, LP, JW Opportunities Fund, LLC, and J.W. Opportunities Master Fund, Ltd.	Exhibit 10.2 to the Company's Current Report on Form 8-K, filed with the SEC on December 11, 2015.
<a href="#">10.12</a>	* Deferred Share Unit Plan For Non-Employee Directors of the Company.	Exhibit 10.32 to the Company's Quarterly Report on Form 10-Q, filed with the SEC on August 9, 2005.
<a href="#">10.13</a>	* Aegerion Pharmaceuticals, Inc. 2010 Stock Option and Incentive Plan.	Exhibit 10.2 to Aegerion Pharmaceuticals, Inc.'s Registration Statement on Form S-1, as amended, filed with the SEC on October 7, 2010.

<b>Exhibit</b>	<b>Description of Document</b>	<b>Location</b>
<a href="#">10.14</a>	* Form of Indemnity Agreement (Directors).	Exhibit 10.27 to the Company's Annual Report on Form 10-K, filed with the SEC on March 30, 2017.
<a href="#">10.15</a>	* Form of Indemnity Agreement (Officers).	Exhibit 10.28 to the Company's Annual Report on Form 10-K, filed with the SEC on March 30, 2017.
<a href="#">10.16</a>	* Employment Agreement, dated January 7, 2016, by and between Aegerion Pharmaceuticals, Inc. and Mary T. Szela.	Exhibit 10.1 to Aegerion Pharmaceuticals, Inc.'s Form 8-K, filed with the SEC on January 11, 2016.
<a href="#">10.17</a>	* Employment Offer Letter, dated November 28, 2016, between Novelion Services USA, Inc. and Gregory D. Perry.	Exhibit 10.37 to the Company's Annual Report on Form 10-K, filed with the SEC on March 30, 2017.
<a href="#">10.18</a>	* Employment Offer Letter, dated November 28, 2016, between Novelion Services USA, Inc. and Benjamin Harshbarger.	Exhibit 10.46 to the Company's Annual Report on Form 10-K, filed with the SEC on March 30, 2017.
<a href="#">10.19</a>	* Employment Offer Letter, dated November 28, 2016, between Novelion Services USA, Inc. and Roger Louis.	Exhibit 10.47 to the Company's Annual Report on Form 10-K, filed with the SEC on March 30, 2017.
<a href="#">10.20</a>	* Employment Offer Letter, dated November 28, 2016, between Novelion Services USA, Inc. and Remi Menes.	Exhibit 10.48 to the Company's Annual Report on Form 10-K, filed with the SEC on March 30, 2017.
<a href="#">10.21</a>	* Amended and Restated Employment Agreement, by and between Mary Szela and the Company, dated as of May 8, 2017.	Exhibit 10.1 to the Company's Quarterly Report on Form 10-Q, filed with the SEC on May 9, 2017.
<a href="#">10.22</a>	* Form of Amendment to Employment Agreement (Executives).	Exhibit 10.2 to the Company's Quarterly Report on Form 10-Q, filed with the SEC on May 9, 2017.
<a href="#">10.23</a>	* Amended and Restated Novelion 2017 Equity Incentive Plan.	Exhibit 99.1 to the Company's Registration Statement on Form S-8, filed with the SEC on July 31, 2017.
<a href="#">10.24</a>	* 2017 Employee Stock Purchase Plan.	Exhibit 99.1 to the Company's Registration Statement on Form S-8, filed with the SEC on July 31, 2017.
<a href="#">10.25</a>	Plea Agreement, dated September 22, 2017.	Exhibit 10.1 to the Company's Current Report on Form 8-K, filed with the SEC on September 22, 2017.
<a href="#">10.26</a>	Civil Settlement Agreement, dated September 22, 2017.	Exhibit 10.2 to the Company's Current Report on Form 8-K, filed with the SEC on September 22, 2017.
<a href="#">10.27</a>	Proposed Final Judgment, dated September 22, 2017.	Exhibit 10.3 to the Company's Current Report on Form 8-K, filed with the SEC on September 22, 2017.
<a href="#">10.28</a>	Deferred Prosecution Agreement, dated September 22, 2017.	Exhibit 10.4 to the Company's Current Report on Form 8-K, filed with the SEC on September 22, 2017.
<a href="#">10.29</a>	Corporate Integrity Agreement, dated September 22, 2017.	Exhibit 10.5 to the Company's Current Report on Form 8-K, filed with the SEC on September 22, 2017.
<a href="#">10.30</a>	Consent Decree of Final Injunction, dated September 22, 2017.	Exhibit 10.6 to the Company's Current Report on Form 8-K, filed with the SEC on September 22, 2017.
<a href="#">10.31</a>	* Separation Letter Agreement, by and between Remi Menes and Novelion Services USA, Inc., dated as of August 31, 2017.	Exhibit 10.1 to the Company's Quarterly Report on Form 10-Q, filed with the SEC on November 9, 2017.
<a href="#">10.32</a>	* Amendment No. 4 to Employment Agreement, by and between Gregory Perry and Novelion Services USA, Inc., dated as of July 6, 2017.	Exhibit 10.2 to the Company's Quarterly Report on Form 10-Q, filed with the SEC on November 9, 2017.

<b>Exhibit</b>	<b>Description of Document</b>	<b>Location</b>
<a href="#">10.33</a>	* Form of Stock Option Award Grant Notice and Stock Option Award Agreement (Directors) under the Amended and Restated Novelion 2017 Equity Incentive Plan.	Filed herewith.
<a href="#">10.34</a>	* Form of Stock Option Award Grant Notice and Stock Option Award Agreement (Employees) under the Amended and Restated Novelion 2017 Equity Incentive Plan.	Filed herewith.
<a href="#">10.35</a>	* Form of Stock Option Award Grant Notice and Stock Option Award Agreement (Executives) under the Amended and Restated Novelion 2017 Equity Incentive Plan.	Filed herewith.
<a href="#">10.36</a>	* Form of Restricted Stock Unit Award Grant Notice and Restricted Stock Unit Award Agreement (Employees) under the Amended and Restated Novelion 2017 Equity Incentive Plan.	Filed herewith.
<a href="#">10.37</a>	* Form of Restricted Stock Unit Award Grant Notice and Restricted Stock Unit Award Agreement (Executives) under the Amended and Restated Novelion 2017 Equity Incentive Plan.	Filed herewith.
<a href="#">10.38</a>	* Employment Agreement, dated as of October 31, 2017, between Novelion Services USA, Inc. and Jeffrey Hackman.	Filed herewith.
<a href="#">10.39</a>	* Separation Letter Agreement, by and between Mary T. Szela and Novelion Services USA, Inc., dated as of November 8, 2017.	Filed herewith.
<a href="#">10.40</a>	* Employment Agreement, dated as of November 27, 2017, between Novelion Services USA, Inc. and Michael D. Price.	Filed herewith.
<a href="#">10.41</a>	* Separation Letter Agreement, by and between Gregory D. Perry and Novelion Services USA, Inc., dated as of November 30, 2017.	Filed herewith.
<a href="#">21.1</a>	Subsidiaries of the Company.	Filed herewith.
<a href="#">23.1</a>	Consent of Independent Registered Public Accounting Firm- Deloitte & Touche LLP.	Filed herewith.
<a href="#">23.2</a>	Consent of Independent Registered Public Accounting Firm- Deloitte LLP.	Filed herewith.
24.1	Power of Attorney.	Contained on signature page hereto.
<a href="#">31.1</a>	Certification pursuant to Rule 13a-14(a)/15(d)-14(a) of the Exchange Act, as adopted pursuant to Section 302 of the Sarbanes-Oxley Act of 2002: Principal Executive Officer.	Filed herewith.
<a href="#">31.2</a>	Certification pursuant to Rule 13a-14(a)/15(d)-14(a) of the Exchange Act, as adopted pursuant to Section 302 of the Sarbanes-Oxley Act of 2002: Principal Financial Officer.	Filed herewith.
<a href="#">32.1</a>	Certification pursuant to 18 U.S.C. Section 1350, as adopted pursuant to Section 906 of the Sarbanes-Oxley Act of 2002: Principal Executive Officer.	Furnished herewith.
<a href="#">32.2</a>	Certification pursuant to 18 U.S.C. Section 1350, as adopted pursuant to Section 906 of the Sarbanes-Oxley Act of 2002: Principal Financial Officer.	Furnished herewith.
101.1	The following financial statements from the Company's Annual Report on Form 10-K for the year ended December 31, 2017, formatted in Extensible Business Reporting Language:  Consolidated Balance Sheets; Consolidated Statements of Operations; Consolidated Statements of Comprehensive Loss; Consolidated Statements of Shareholders' Equity; Consolidated Statements of Cash Flows; and Notes to Consolidated Financial Statements.	Filed herewith.

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**Notes:**

\* Denotes executive compensation plans or arrangements.

# Confidential treatment has been received for certain provisions of this Exhibit. Confidential portions have been omitted and filed separately with the SEC.

## SIGNATURES

Pursuant to the requirements of the Securities Exchange Act of 1934, the registrant has duly caused this report to be signed on its behalf by the undersigned, thereunto duly authorized.

NOVELION THERAPEUTICS INC.

(Registrant)

Date: March 16, 2018

By: /s/ Jeffrey Hackman  
Jeffrey Hackman  
Principal Executive Officer

Date: March 16, 2018

By: /s/ Michael D. Price  
Michael D. Price  
Principal Financial Officer

## POWER OF ATTORNEY

KNOW ALL PERSONS BY THESE PRESENTS, that each person whose signature appears below appoints severally, Jeffrey Hackman, Michael D. Price and Benjamin Harshbarger, and each one of them, his or her attorneys-in-fact, each with the power of substitution for him in any and all capacities, to sign any and all amendments to this Annual Report on Form 10-K and to file the same, with exhibits thereto and other documents in connection therewith, with the SEC, hereby ratifying and confirming all that each attorneys-in-fact, or his substitute or substitutes, may do or cause to be done by virtue hereof.

Pursuant to the requirements of the Securities Exchange Act of 1934, this report has been signed below by the following persons on behalf of the registrant and in the capacities and on the dates indicated.

<b>Signature</b>	<b>Capacity</b>	<b>Date</b>
<u>/s/ Jeffrey Hackman</u> Jeffrey Hackman	<u>Chief Operating Officer (principal executive officer)</u>	<u>March 16, 2018</u>
<u>/s/ Michael D. Price</u> Michael D. Price	<u>Chief Financial Officer (principal financial officer)</u>	<u>March 16, 2018</u>
<u>/s/ Barbara Chan</u> Barbara Chan	<u>President and Chief Accounting Officer, Aegerion Pharmaceuticals, Inc. (principal accounting officer)</u>	<u>March 16, 2018</u>
<u>/s/ Jason Aryeh</u> Jason Aryeh	<u>Chairman of the Board of Directors</u>	<u>March 16, 2018</u>
<u>/s/ Suzanne Bruhn</u> Suzanne Bruhn	<u>Director</u>	<u>March 16, 2018</u>
<u>/s/ Mark Corrigan</u> Mark Corrigan	<u>Director</u>	<u>March 16, 2018</u>
<u>/s/ Mark DiPaolo</u> Mark DiPaolo	<u>Director</u>	<u>March 16, 2018</u>
<u>/s/ Kevin Kotler</u> Kevin Kotler	<u>Director</u>	<u>March 16, 2018</u>
<u>/s/ John Orloff</u> John Orloff	<u>Director</u>	<u>March 16, 2018</u>
<u>/s/ Stephen Sabba</u> Stephen Sabba	<u>Director</u>	<u>March 16, 2018</u>
<u>/s/ Donald K. Stern</u> Donald K. Stern	<u>Director</u>	<u>March 16, 2018</u>
<u>/s/ John C. Thomas, Jr.</u> John C. Thomas, Jr.	<u>Director</u>	<u>March 16, 2018</u>



**NOVELION THERAPEUTICS INC.**  
**AMENDED AND RESTATED**  
**NOVELION 2017 EQUITY INCENTIVE PLAN**  
**STOCK OPTION AWARD GRANT NOTICE AND**  
**STOCK OPTION AWARD AGREEMENT**

**(Directors)**

Novelion Therapeutics Inc. (the “Company”), pursuant to its Amended and Restated 2017 Equity Incentive Plan, as amended from time to time (the “Plan”), hereby grants to the individual listed below (“Grantee”), an award (“Award”) of an option (“Option”) to purchase a number of Common Shares, as set forth below. The Option is subject to the conditions and limitations set forth in this Stock Option Award Grant Notice (the “Grant Notice”), the Stock Option Award Agreement attached hereto as Exhibit A (the “Award Agreement”) and the Plan. Unless otherwise defined in this Grant Notice or the Award Agreement, defined terms shall have the meaning set forth in the Plan.

**Grantee’s Name:**

**Grant Date:**

**Number of Common Shares Subject to Option:**

**Option Exercise Price:**

**Expiry Date:**

**Vesting Commencement Date:**

**Vesting Schedule:**

*[Remainder of this page intentionally left blank]*

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By accepting the Award, Grantee agrees to be bound by the terms and conditions of the Plan, the Award Agreement and this Grant Notice. Grantee has reviewed the Award Agreement, the Plan and this Grant Notice in their entirety and fully understands all provisions of the Award Agreement, the Plan and this Grant Notice. Additionally, by accepting the Award, Grantee agrees that he or she has read, fully understands and agrees to abide by the terms of the Company's Insider Trading Policy and has read and fully understands the Plan Prospectus, copies of which have been made available to Grantee. Grantee hereby agrees to accept as binding, conclusive and final all decisions or interpretations of the Committee regarding any questions arising under the Plan or relating to the Option.

**Novelion therapeutics inc.**

By: \_\_\_\_\_

Print Name: Linda Buono  
Senior Vice President, Human Resources, Novelion

Title: Therapeutics, Inc.

The foregoing Agreement is hereby accepted and the terms and conditions thereof hereby agreed to by the undersigned.

By: \_\_\_\_\_

Print Name: \_\_\_\_\_

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## EXHIBIT A

### STOCK OPTION AWARD AGREEMENT (DIRECTORS)

1. **General** . Pursuant to the Grant Notice (the “ **Grant Notice** ”) to which this Stock Option Award Agreement (the “ **Award Agreement** ”) is attached, Novelion Therapeutics Inc. (the “ **Company** ”) has granted to Grantee an award of an Option under the Company’s Amended and Restated 2017 Equity Incentive Plan, as amended from time to time (the “ **Plan** ”).
  2. **Defined Terms** . All capitalized terms which are not defined in the Grant Notice or below have the meaning given to them in the Plan.
  3. **Term** . Subject to the terms and conditions of the Plan and this Award Agreement, the Option will terminate on the earlier of:
    - (a) The date on which the Option is exercised with respect to all Common Shares subject to the Option; and
    - (b) 5:00 p.m. (Vancouver time) on the Expiry Date.
  4. **Vesting** . The vesting provisions applicable to the Option shall be as set forth in the Grant Notice.
  5. **Exercise of Options** .
    - (a) Exercise Notice . No portion of the Option may be exercised until such portion vests. Grantee may exercise some or all of the vested portion of the Option by giving written notice of exercise (the “ **Exercise Notice** ”) signed and dated by Grantee (and not postdated), stating that Grantee elects to exercise his or her rights to purchase Common Shares subject to the Option and specifying the number of Common Shares in respect of which the Option is being exercised and specifying the Option Exercise Price to be paid therefor.
    - (b) Delivery and Payment . Grantee shall deliver the Exercise Notice to the Company at its principal office at 887 Great Northern Way, Suite 101, Vancouver, British Columbia, Canada, V5T 4T5 (or at such other address as the principal office of the Company may be located at the time of exercise) addressed to the attention of the Secretary or assistant secretary (if any) of the Company (or a designee notified in writing from time to time by the Company) and such Exercise Notice shall be accompanied by full payment (payable at par in Vancouver, British Columbia) in any combination of the following (subject to all applicable laws):
      - (i) cash, bank draft or certified cheque;
      - (ii) if and so long as the Common Shares are listed on an Exchange, delivery of a properly executed Exercise Notice, together with irrevocable instructions, to
        - (A) a brokerage firm designated by the Company to deliver promptly to the Company the aggregate amount of sale proceeds to pay the Option Exercise Price and any withholding tax obligations that may arise in connection with the exercise, and
        - (B) the Company to deliver the certificates for such purchased shares directly to such brokerage firm,all in accordance with the regulations of any relevant regulatory authorities; and
      - (iii) with prior written consent of the Company and subject to Section 13.3 of the Plan, written instructions from Grantee to the Company to effect a net settlement of Common Shares subject to the Option having a value equal to the Option Exercise Price of any Option and/or the withholding taxes due with respect to the exercise of the Option; and
    - (c) Certificate . As soon as practicable after any exercise of the Option, a certificate or certificates representing the Common Shares into which the Option is exercised will be delivered by the Company to Grantee or to Grantee’s designated brokered firm, as applicable.
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6. **Rules Upon Termination of Service** . The Option will terminate on the earlier of the expiry of the Option under Section 3 above and the 90th day (effective following the close of trading on the Exchange, if such day is a trading day) after the date of Grantee's Termination of Service as a director of the Company or its Affiliates, provided that, upon Grantee's Termination of Service as a result of:
- (a) ceasing to meet the qualifications set forth in subsection 124(2) of the Business Corporations Act (British Columbia), as amended, or such other qualifications required by the corporate laws in any other jurisdiction under which the Company is continued or amalgamated,
  - (b) a special resolution having been passed by the shareholders of the Company pursuant to subsection 128(3) of the Business Corporations Act (British Columbia), as amended, or an equivalent enactment pursuant to the corporate laws in any other jurisdiction under which the Company is continued or amalgamated, or
  - (c) by order of a securities commission, the TSX, NASDAQ or any other regulatory body having jurisdiction to so order, unless otherwise determined by the Committee and approved by the Exchange (if applicable), the Option (whether vested or unvested) will expire automatically on the date of Grantee's Termination of Service.

For the avoidance of doubt, the Option will cease to vest after the date of Grantee's Termination of Service as a director of the Company. Further, the option and performance stock unit awards granted to Grantee during his employment with the Company terminated at the time of his termination of employment with the Company on June 2, 2017.

7. **Other** .

- (a) **Sale Event** . In the event that Grantee is party to an effective employment or similar individual agreement with the Company or its Affiliates that provides for the treatment of an equity award in connection with "Sale Event" (as defined in such agreement), such provision shall only apply in connection with a "Sale Event" that occurs on or after the Grant Date (and shall not, for the avoidance of doubt, apply in connection with a "Sale Event" that occurred prior to the Grant Date).
- (b) **Section 4985** . If any amount payable or paid by the Company or any of its affiliates pursuant to this Agreement or otherwise to or for the benefit of Grantee becomes subject to the excise tax imposed by Section 4985 of the Code (including any interest, penalties or additions to tax relating thereto) (the "**4985 Excise Tax**") by reason of the consummation of the transactions contemplated by the Agreement and Plan of Merger, dated as of June 14, 2016 (as amended), among QLT, Inc., Aegerion Pharmaceuticals, Inc. and certain other parties thereto, as reasonably determined by the Company, then the Company shall pay to Grantee (1) an amount equal to the 4985 Excise Tax, and (2) an amount (the "**4985 Gross-up Payment**") equal to the amount necessary to put Grantee in the same net after-tax position (taking into account any and all applicable Federal, state, local and foreign income, employment, excise and other taxes) that Grantee would have been in if Grantee had not incurred any liability for taxes under Section 4985 of the Code. Any determination regarding the amount of any payment or payments hereunder shall be made in writing by the Company's independent accountants or other accounting or consulting firm selected by the Company, whose determination shall be conclusive and binding upon Grantee and the Company for all purposes.

8. **Conditions to Exercise** . Notwithstanding any of the provisions of the Award Agreement, the Company's obligation to issue Common Shares to Grantee upon exercise of the Option is subject to the following:

- (a) **Qualification** . Completion of registration or other qualification of the Common Shares or obtaining approval of such governmental authority as the Company determines is necessary or advisable in connection with the authorization, issuance or sale of the Common Shares;
- (b) **Listing** . The admission of the Common Shares to listing or quotation on the Exchange; and
- (c) **Undertakings** . The receipt by the Company from Grantee of such representations, agreements and undertakings, including as to future dealings in the Common Shares, as the Company or its counsel determines are necessary or advisable in order to safeguard against the violation of securities laws of any jurisdiction.

9. **Tax** . Grantee is solely responsible for the payment of any applicable taxes arising from the grant, vesting, settlement or exercise of the Option and any payment is to be in a manner satisfactory to the Company. Notwithstanding the foregoing, the Company will have the right to withhold from any amount payable to Grantee, either under the Plan or otherwise, such amount as may be necessary to enable the Company to comply with the applicable requirements of any federal,

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provincial, state, local or foreign law, or any administrative policy of any applicable tax authority, relating to the withholding of tax or any other required deductions with respect to the Option (the “**Withholding Obligations**”). The Company may require Grantee, as a condition to the exercise or settlement of the Option, to make such arrangements as the Company may require so that the Company can satisfy applicable Withholding Obligations, including, without limitation, requiring Grantee to (i) remit the amount of any such Withholding Obligations to the Company in advance; (ii) reimburse the Company for any such Withholding Obligations; (iii) deliver written instructions contemplated in Section 5(b)(iii) hereof, to effect a net settlement of Common Shares subject to the Option in an amount required to satisfy any such Withholding Obligations; or (iv) pursuant to Section 5(b)(ii) hereof, cause such broker to withhold from the proceeds realized from such transaction the amount required to satisfy any such Withholding Obligations and to remit such amount directly to the Company.

10. **Black Out Periods** . Grantee acknowledges and agrees that the Award Agreement and the grant of the Option to Grantee is subject to Grantee’s agreement to at all times comply with the Company’s policies with respect to black out periods, as more particularly set out in the Company’s Trading Policy, as amended from time to time.
11. **No Rights as Shareholder** . Grantee will not have any rights as a Shareholder with respect to any of the Common Shares subject to the Option until such time as Grantee becomes the record owner of such Common Shares.
12. **No Effect on Service** . Nothing in the Award Agreement will:
  - (a) Continue Service . Confer upon Grantee any right to continue in the service of the Company or any Affiliate or affect in any way the right of the Company or any Affiliate to terminate his or her service at any time.
  - (b) Extend Service . Be construed to constitute an agreement, or an expression of intent, on the part of the Company or any Affiliate to extend the service of Grantee beyond the time that he or she would normally be retired pursuant to the provisions of any present or future retirement plan or policy of the Company or any Affiliate, or beyond the time at which he or she would otherwise be retired pursuant to the provisions of any contract of service with the Company or any Affiliate.
13. **Enurement** . The Award Agreement shall enure to the benefit of and be binding upon the parties to the Award Agreement and upon the successors or assigns of the Company and upon the executors, administrators and legal personal representatives of Grantee.
14. **Further Assurances** . Each of the parties to the Award Agreement will do such further acts and execute such further documents as may be required to give effect to and carry out the intent of the Award Agreement.
15. **Non-Assignable** . The Option is personal to Grantee and may not be assigned or transferred in whole or in part, except by will or by the operation of the laws of devolution or distribution and descent.
16. **Amendments** . Any amendments to the Award Agreement must be in writing duly executed by the parties and will (if required) be subject to the approval of the applicable regulatory authorities.
17. **Time of the Essence** . Time is of the essence of the Award Agreement.
18. **Governing Law** . The Award Agreement shall be governed, construed and enforced according to the laws of the Province of British Columbia and is subject to the exclusive jurisdiction of the courts of the Province of British Columbia.
19. **Interpretation of the Award Agreement and the Plan** . If any question or dispute arises as to the interpretation of the Award Agreement, the question or dispute will be determined by the Committee and such determination will be final, conclusive and binding for all purposes on both the Company and Grantee.
20. **Conflict Between Award Agreement and the Plan** . If there is any conflict between this Award Agreement and the Plan, the Plan, as amended from time to time, will govern.

NOVELION THERAPEUTICS INC.

AMENDED AND RESTATED NOVELION 2017 EQUITY INCENTIVE PLAN

STOCK OPTION AWARD GRANT NOTICE AND  
STOCK OPTION AWARD AGREEMENT

(Employees)

Novelion Therapeutics Inc. (the “Company”), pursuant to its Amended and Restated 2017 Equity Incentive Plan, as amended from time to time (the “Plan”), hereby grants to the individual listed below (“Grantee”), an award (“Award”) of an option (“Option”) to purchase a number of Common Shares, as set forth below. The Option is subject to the conditions and limitations set forth in this Stock Option Award Grant Notice (the “Grant Notice”), the Stock Option Award Agreement attached hereto as Exhibit A (the “Award Agreement”) and the Plan. Unless otherwise defined in this Grant Notice or the Award Agreement, defined terms shall have the meaning set forth in the Plan.

**Grantee’s Name:**

**Grant Date:**

**Number of Common Shares Subject to Option:**

**Option Exercise Price:**

**Expiry Date:**

**Vesting Commencement Date:**

**Vesting Schedule:**

*[Remainder of this page intentionally left blank]*

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By accepting the Award, Grantee agrees to be bound by the terms and conditions of the Plan, the Award Agreement and this Grant Notice. Grantee has reviewed the Award Agreement, the Plan and this Grant Notice in their entirety and fully understands all provisions of the Award Agreement, the Plan and this Grant Notice. Additionally, by accepting the Award, Grantee agrees that he or she has read, fully understands and agrees to abide by the terms of the Company's Insider Trading Policy and has read and fully understands the Plan Prospectus, copies of which have been made available to Grantee. Grantee hereby agrees to accept as binding, conclusive and final all decisions or interpretations of the Committee regarding any questions arising under the Plan or relating to the Option.

**NOVELION THERAPEUTICS Inc.**

By: \_\_\_\_\_

Print Name: Linda Buono

Senior Vice President, Human Resources, Novelion

Title: Therapeutics, Inc.

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## EXHIBIT A

### STOCK OPTION AWARD AGREEMENT (EMPLOYEES)

1. **General** . Pursuant to the Grant Notice (the “ **Grant Notice** ”) to which this Stock Option Award Agreement (the “ **Award Agreement** ”) is attached, Novelion Therapeutics Inc. (the “ **Company** ”) has granted to Grantee an award of an Option under the Company’s Amended and Restated 2017 Equity Incentive Plan, as amended from time to time (the “ **Plan** ”).
  2. **Defined Terms** . All capitalized terms which are not defined in the Grant Notice or below have the meaning given to them in the Plan.
  3. **Term** . Subject to the terms and conditions of the Plan and this Award Agreement, the Option will terminate on the earlier of:
    - (a) The date on which the Option is exercised with respect to all Common Shares subject to the Option; and
    - (b) 5:00 p.m. (Vancouver time) on the Expiry Date.
  4. **Vesting** . The vesting provisions applicable to the Option shall be as set forth in the Grant Notice.
  5. **Exercise of Options** .
    - (a) **Exercise Notice** . No portion of the Option may be exercised until such portion vests. Grantee may exercise some or all of the vested portion of the Option by giving written notice of exercise (the “ **Exercise Notice** ”) signed and dated by Grantee (and not postdated), stating that Grantee elects to exercise his or her rights to purchase Common Shares subject to the Option and specifying the number of Common Shares in respect of which the Option is being exercised and specifying the Option Exercise Price to be paid therefor.
    - (b) **Delivery and Payment** . Grantee shall deliver the Exercise Notice to the Company at its principal office at 887 Great Northern Way, Suite 101, Vancouver, British Columbia, Canada, V5T 4T5 (or at such other address as the principal office of the Company may be located at the time of exercise) addressed to the attention of the Secretary or assistant secretary (if any) of the Company (or a designee notified in writing from time to time by the Company) and such Exercise Notice shall be accompanied by full payment (payable at par in Vancouver, British Columbia) in any combination of the following (subject to all applicable laws):
      - (i) cash, bank draft or certified cheque;
      - (ii) if and so long as the Common Shares are listed on an Exchange, delivery of a properly executed Exercise Notice, together with irrevocable instructions, to
        - (A) a brokerage firm designated by the Company to deliver promptly to the Company the aggregate amount of sale proceeds to pay the Option Exercise Price and any withholding tax obligations that may arise in connection with the exercise, and
        - (B) the Company to deliver the certificates for such purchased shares directly to such brokerage firm,all in accordance with the regulations of any relevant regulatory authorities; and
      - (iii) with prior written consent of the Company and subject to Section 13.3 of the Plan, written instructions from Grantee to the Company to effect a net settlement of Common Shares subject to the Option having a value equal to the Option Exercise Price of any Option and/or the withholding taxes due with respect to the exercise of the Option; and
    - (c) **Certificate** . As soon as practicable after any exercise of the Option, a certificate or certificates representing the Common Shares into which the Option is exercised will be delivered by the Company to Grantee or to Grantee’s designated brokered firm, as applicable.
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6. **Rules Upon Termination of Service** . The Option will terminate on the earlier of the expiry of the Option under Section 3 above and the 90th day (effective following the close of trading on the Exchange, if such day is a trading day) after the date of Grantee's Termination of Service, provided that upon Grantee's Termination of Service by the Company or any Affiliate for Cause (as defined below) (as determined by the Company in its sole discretion), unless otherwise determined by the Committee and approved by the Exchange (if applicable), the Option (whether vested or unvested) will expire automatically on the date of Grantee's Termination of Service.

For purposes of this Agreement, " **Cause** " shall have the meaning set forth in Grantee's employment agreement with the Company for so long as such agreement remains in effect or, if there is no such agreement between Grantee and the Company, shall mean: (i) Grantee's failure (except where due to complete disability), neglect, or refusal to perform in any material respect Grantee's duties and responsibilities, (ii) any act of Grantee that has, or could reasonably be expected to have, the effect of injuring the business of the Company or its affiliates in any material respect, (iii) Grantee's conviction of, or plea of guilty or no contest to: (A) a felony or (B) any other criminal charge that has, or could be reasonably expected to have, an adverse impact on the performance of Grantee's duties to the Company or otherwise result in material injury to the reputation or business of the Company, (iv) the commission by Grantee of an act of fraud or embezzlement against the Company, or any other act that creates or reasonably could create negative or adverse publicity for the Company; (v) any violation by Grantee of the policies of the Company, including but not limited to those relating to sexual harassment or business conduct, and those otherwise set forth in the manuals or statements of policy of the Company, (vi) Grantee's violation of federal or state securities laws, or (vii) Grantee's breach of any agreement between the Company or its affiliates and Grantee, including Grantee's breach of any non-competition, non-solicitation, confidentiality or other restrictive covenant agreement with the Company.

For the avoidance of doubt, the Option will cease to vest after the date of Grantee's Termination of Service.

7. **Sale Event** . In the event that Grantee is party to an effective employment or similar individual agreement with the Company or its Affiliates that provides for the treatment of an equity award in connection with "Sale Event" (as defined in such agreement), such provision shall only apply in connection with a "Sale Event" that occurs on or after the Grant Date (and shall not, for the avoidance of doubt, apply in connection with a "Sale Event" that occurred prior to the Grant Date).
8. **Conditions to Exercise** . Notwithstanding any of the provisions of the Award Agreement, the Company's obligation to issue Common Shares to Grantee upon exercise of the Option is subject to the following:
- (a) **Qualification** . Completion of registration or other qualification of the Common Shares or obtaining approval of such governmental authority as the Company determines is necessary or advisable in connection with the authorization, issuance or sale of the Common Shares;
  - (b) **Listing** . The admission of the Common Shares to listing or quotation on the Exchange; and
  - (c) **Undertakings** . The receipt by the Company from Grantee of such representations, agreements and undertakings, including as to future dealings in the Common Shares, as the Company or its counsel determines are necessary or advisable in order to safeguard against the violation of securities laws of any jurisdiction.
9. **Tax** . Grantee is solely responsible for the payment of any applicable taxes arising from the grant, vesting, settlement or exercise of the Option and any payment is to be in a manner satisfactory to the Company. Notwithstanding the foregoing, the Company will have the right to withhold from any amount payable to Grantee, either under the Plan or otherwise, such amount as may be necessary to enable the Company to comply with the applicable requirements of any federal, provincial, state, local or foreign law, or any administrative policy of any applicable tax authority, relating to the withholding of tax or any other required deductions with respect to the Option (the " **Withholding Obligations** "). The Company may require Grantee, as a condition to the exercise or settlement of the Option, to make such arrangements as the Company may require so that the Company can satisfy applicable Withholding Obligations, including, without limitation, requiring Grantee to (i) remit the amount of any such Withholding Obligations to the Company in advance; (ii) reimburse the Company for any such Withholding Obligations; (iii) deliver written instructions contemplated in Section 5(b)(iii) hereof, to effect a net settlement of Common Shares subject to the Option in an amount required to satisfy any such Withholding Obligations; or (iv) pursuant to Section 5(b)(ii) hereof, cause such broker to withhold from the proceeds realized from such transaction the amount required to satisfy any such Withholding Obligations and to remit such amount directly to the Company.
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10. **Black Out Periods** . Grantee acknowledges and agrees that the Award Agreement and the grant of the Option to Grantee is subject to Grantee's agreement to at all times comply with the Company's policies with respect to black out periods, as more particularly set out in the Company's Trading Policy, as amended from time to time.
11. **No Rights as Shareholder** . Grantee will not have any rights as a Shareholder with respect to any of the Common Shares subject to the Option until such time as Grantee becomes the record owner of such Common Shares.
12. **No Effect on Employment** . Nothing in the Award Agreement will:
  - (a) Continue Employment . Confer upon Grantee any right to continue in the employ of or under contract with the Company or any Affiliate or affect in any way the right of the Company or any Affiliate to terminate his or her employment or service at any time.
  - (b) Extend Employment . Be construed to constitute an agreement, or an expression of intent, on the part of the Company or any Affiliate to extend the employment or service of Grantee beyond the time that he or she would normally be retired pursuant to the provisions of any present or future retirement plan or policy of the Company or any Affiliate, or beyond the time at which he or she would otherwise be retired pursuant to the provisions of any contract of employment with the Company or any Affiliate.
13. **Clawback** . The Option (whether or not vested) is subject to forfeiture, termination and rescission, and Grantee will be obligated to return to the Company the value received with respect to the Option (including any gain realized on a subsequent sale or disposition of Common Shares) in accordance with any clawback or similar policy maintained by the Company, as such policy may be amended and in effect from time to time, or as otherwise required by law or applicable stock exchange listing standards, including, without limitation, Section 10D of the Securities Exchange Act of 1934, as amended.
14. **Enurement** . The Award Agreement shall enure to the benefit of and be binding upon the parties to the Award Agreement and upon the successors or assigns of the Company and upon the executors, administrators and legal personal representatives of Grantee.
15. **Further Assurances** . Each of the parties to the Award Agreement will do such further acts and execute such further documents as may be required to give effect to and carry out the intent of the Award Agreement.
16. **Non-Assignable** . The Option is personal to Grantee and may not be assigned or transferred in whole or in part, except by will or by the operation of the laws of devolution or distribution and descent.
17. **Amendments** . Any amendments to the Award Agreement must be in writing duly executed by the parties and will (if required) be subject to the approval of the applicable regulatory authorities.
18. **Time of the Essence** . Time is of the essence of the Award Agreement.
19. **Governing Law** . The Award Agreement shall be governed, construed and enforced according to the laws of the Province of British Columbia and is subject to the exclusive jurisdiction of the courts of the Province of British Columbia.
20. **Interpretation of the Award Agreement and the Plan** . If any question or dispute arises as to the interpretation of the Award Agreement, the question or dispute will be determined by the Committee and such determination will be final, conclusive and binding for all purposes on both the Company and Grantee.
21. **Conflict Between Award Agreement and the Plan** . If there is any conflict between this Award Agreement and the Plan, the Plan, as amended from time to time, will govern.

**NOVELION THERAPEUTICS INC.**  
**AMENDED AND RESTATED**  
**NOVELION 2017 EQUITY INCENTIVE PLAN**  
**STOCK OPTION AWARD GRANT NOTICE AND**  
**STOCK OPTION AWARD AGREEMENT**

**(Executives)**

Novelion Therapeutics Inc. (the “Company”), pursuant to its Amended and Restated 2017 Equity Incentive Plan, as amended from time to time (the “Plan”), hereby grants to the individual listed below (“Grantee”), an award (“Award”) of an option (“Option”) to purchase a number of Common Shares, as set forth below. The Option is subject to the conditions and limitations set forth in this Stock Option Award Grant Notice (the “Grant Notice”), the Stock Option Award Agreement attached hereto as Exhibit A (the “Award Agreement”) and the Plan. Unless otherwise defined in this Grant Notice or the Award Agreement, defined terms shall have the meaning set forth in the Plan.

**Grantee’s Name:**

**Grant Date:**

**Number of Common Shares Subject to Option:**

**Option Exercise Price:**

**Expiry Date:**

**Vesting Commencement Date:**

**Vesting Schedule:**

*[Remainder of this page intentionally left blank.]*

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By accepting the Award, Grantee agrees to be bound by the terms and conditions of the Plan, the Award Agreement and this Grant Notice. Grantee has reviewed the Award Agreement, the Plan and this Grant Notice in their entirety and fully understands all provisions of the Award Agreement, the Plan and this Grant Notice. Additionally, by accepting the Award, Grantee agrees that he or she has read, fully understands and agrees to abide by the terms of the Company's Insider Trading Policy and has read and fully understands the Plan Prospectus, copies of which have been made available to Grantee. Grantee hereby agrees to accept as binding, conclusive and final all decisions or interpretations of the Committee regarding any questions arising under the Plan or relating to the Option.

**NOVELION THERAPEUTICS Inc.**

By: \_\_\_\_\_

Print Name: Linda Buono

Title: Senior Vice President, Human Resources

The Grant Notice and Award Agreement are hereby accepted and the terms and conditions thereof hereby agreed to by the undersigned.

By: \_\_\_\_\_

Name:

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**EXHIBIT A**

**STOCK OPTION AWARD AGREEMENT**

**(Executives)**

1. **General** . Pursuant to the Grant Notice (the “ **Grant Notice** ”) to which this Stock Option Award Agreement (the “ **Award Agreement** ”) is attached, Novelion Therapeutics Inc. (the “ **Company** ”) has granted to Grantee an award of an Option under the Company’s Amended and Restated 2017 Equity Incentive Plan, as amended from time to time (the “ **Plan** ”).
  2. **Defined Terms** . All capitalized terms which are not defined in the Grant Notice or below have the meaning given to them in the Plan.
  3. **Term** . Subject to the terms and conditions of the Plan and this Award Agreement, the Option will terminate on the earlier of:
    - (a) The date on which the Option is exercised with respect to all Common Shares subject to the Option; and
    - (b) 5:00 p.m. (Vancouver time) on the Expiry Date.
  4. **Vesting** . The vesting provisions applicable to the Option shall be as set forth in the Grant Notice.
  5. **Exercise of Options** .
    - (a) Exercise Notice . No portion of the Option may be exercised until such portion vests. Grantee may exercise some or all of the vested portion of the Option by giving written notice of exercise (the “ **Exercise Notice** ”) signed and dated by Grantee (and not postdated), stating that Grantee elects to exercise his or her rights to purchase Common Shares subject to the Option and specifying the number of Common Shares in respect of which the Option is being exercised and specifying the Option Exercise Price to be paid therefor.
    - (b) Delivery and Payment . Grantee shall deliver the Exercise Notice to the Company at its principal office at 887 Great Northern Way, Suite 101, Vancouver, British Columbia, Canada, V5T 4T5 (or at such other address as the principal office of the Company may be located at the time of exercise) addressed to the attention of the Secretary or assistant secretary (if any) of the Company (or a designee notified in writing from time to time by the Company) and such Exercise Notice shall be accompanied by full payment (payable at par in Vancouver, British Columbia) in any combination of the following (subject to all applicable laws):
      - (i) cash, bank draft or certified cheque;
      - (ii) if and so long as the Common Shares are listed on an Exchange, delivery of a properly executed Exercise Notice, together with irrevocable instructions, to
        - (A) a brokerage firm designated by the Company to deliver promptly to the Company the aggregate amount of sale proceeds to pay the Option Exercise Price and any withholding tax obligations that may arise in connection with the exercise, and
        - (B) the Company to deliver the certificates for such purchased shares directly to such brokerage firm,all in accordance with the regulations of any relevant regulatory authorities; and
      - (iii) with prior written consent of the Company and subject to Section 13.3 of the Plan, written instructions from Grantee to the Company to effect a net settlement of Common Shares subject to the Option having a value equal to the Option Exercise Price of any Option and/or the withholding taxes due with respect to the exercise of the Option; and
    - (c) Certificate . As soon as practicable after any exercise of the Option, a certificate or certificates representing the Common Shares into which the Option is exercised will be delivered by the Company to Grantee or to Grantee’s designated brokered firm, as applicable.
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6. **Rules Upon Termination of Service** . The Option will terminate on the earlier of the expiry of the Option under Section 3 above and the 90th day (effective following the close of trading on the Exchange, if such day is a trading day) after the date of Grantee's Termination of Service, provided that upon Grantee's Termination of Service by the Company or any Affiliate for Cause (as defined below) (as determined by the Company in its sole discretion), unless otherwise determined by the Committee and approved by the Exchange (if applicable), the Option (whether vested or unvested) will expire automatically on the date of Grantee's Termination of Service.

For purposes of this Agreement, "Cause" shall have the meaning set forth in Grantee's employment agreement with the Company for so long as such agreement remains in effect or, if there is no such agreement between Grantee and the Company, shall mean: (i) Grantee's failure (except where due to complete disability), neglect, or refusal to perform in any material respect Grantee's duties and responsibilities, (ii) any act of Grantee that has, or could reasonably be expected to have, the effect of injuring the business of the Company or its affiliates in any material respect, (iii) Grantee's conviction of, or plea of guilty or no contest to: (A) a felony or (B) any other criminal charge that has, or could be reasonably expected to have, an adverse impact on the performance of Grantee's duties to the Company or otherwise result in material injury to the reputation or business of the Company, (iv) the commission by Grantee of an act of fraud or embezzlement against the Company, or any other act that creates or reasonably could create negative or adverse publicity for the Company; (v) any violation by Grantee of the policies of the Company, including but not limited to those relating to sexual harassment or business conduct, and those otherwise set forth in the manuals or statements of policy of the Company, (vi) Grantee's violation of federal or state securities laws, or (vii) Grantee's breach of any agreement between the Company or its affiliates and Grantee, including Grantee's breach of any non-competition, non-solicitation, confidentiality or other restrictive covenant agreement with the Company.

For the avoidance of doubt, the Option will cease to vest after the date of Grantee's Termination of Service.

7. **Other** .

- (a) **Sale Event** . In the event that Grantee is party to an effective employment or similar individual agreement with the Company or its Affiliates that provides for the treatment of an equity award in connection with "Sale Event" (as defined in such agreement), such provision shall only apply in connection with a "Sale Event" that occurs on or after the Grant Date (and shall not, for the avoidance of doubt, apply in connection with a "Sale Event" that occurred prior to the Grant Date).
- (b) **Section 4985** . If any amount payable or paid by the Company or any of its affiliates pursuant to this Agreement or otherwise to or for the benefit of Grantee becomes subject to the excise tax imposed by Section 4985 of the Code (including any interest, penalties or additions to tax relating thereto) (the "4985 Excise Tax") by reason of the consummation of the transactions contemplated by the Agreement and Plan of Merger, dated as of June 14, 2016 (as amended), among QLT, Inc., Aegerion Pharmaceuticals, Inc. and certain other parties thereto, as reasonably determined by the Company, then the Company shall pay to Grantee (1) an amount equal to the 4985 Excise Tax, and (2) an amount (the "4985 Gross-up Payment") equal to the amount necessary to put Grantee in the same net after-tax position (taking into account any and all applicable Federal, state, local and foreign income, employment, excise and other taxes) that Grantee would have been in if Grantee had not incurred any liability for taxes under Section 4985 of the Code. Any determination regarding the amount of any payment or payments hereunder shall be made in writing by the Company's independent accountants or other accounting or consulting firm selected by the Company, whose determination shall be conclusive and binding upon Grantee and the Company for all purposes.

8. **Conditions to Exercise** . Notwithstanding any of the provisions of the Award Agreement, the Company's obligation to issue Common Shares to Grantee upon exercise of the Option is subject to the following:

- (a) **Qualification** . Completion of registration or other qualification of the Common Shares or obtaining approval of such governmental authority as the Company determines is necessary or advisable in connection with the authorization, issuance or sale of the Common Shares;
- (b) **Listing** . The admission of the Common Shares to listing or quotation on the Exchange; and
- (c) **Undertakings** . The receipt by the Company from Grantee of such representations, agreements and undertakings, including as to future dealings in the Common Shares, as the Company or its counsel determines are necessary or advisable in order to safeguard against the violation of securities laws of any jurisdiction.
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9. **Tax** . Grantee is solely responsible for the payment of any applicable taxes arising from the grant, vesting, settlement or exercise of the Option and any payment is to be in a manner satisfactory to the Company. Notwithstanding the foregoing, the Company will have the right to withhold from any amount payable to Grantee, either under the Plan or otherwise, such amount as may be necessary to enable the Company to comply with the applicable requirements of any federal, provincial, state, local or foreign law, or any administrative policy of any applicable tax authority, relating to the withholding of tax or any other required deductions with respect to the Option (the “**Withholding Obligations**”). The Company may require Grantee, as a condition to the exercise or settlement of the Option, to make such arrangements as the Company may require so that the Company can satisfy applicable Withholding Obligations, including, without limitation, requiring Grantee to (i) remit the amount of any such Withholding Obligations to the Company in advance; (ii) reimburse the Company for any such Withholding Obligations; (iii) deliver written instructions contemplated in Section 5(b)(iii) hereof, to effect a net settlement of Common Shares subject to the Option in an amount required to satisfy any such Withholding Obligations; or (iv) pursuant to Section 5(b)(ii) hereof, cause such broker to withhold from the proceeds realized from such transaction the amount required to satisfy any such Withholding Obligations and to remit such amount directly to the Company.
  10. **Black Out Periods** . Grantee acknowledges and agrees that the Award Agreement and the grant of the Option to Grantee is subject to Grantee’s agreement to at all times comply with the Company’s policies with respect to black out periods, as more particularly set out in the Company’s Trading Policy, as amended from time to time.
  11. **No Rights as Shareholder** . Grantee will not have any rights as a Shareholder with respect to any of the Common Shares subject to the Option until such time as Grantee becomes the record owner of such Common Shares.
  12. **No Effect on Employment** . Nothing in the Award Agreement will:
    - (a) **Continue Employment** . Confer upon Grantee any right to continue in the employ of or under contract with the Company or any Affiliate or affect in any way the right of the Company or any Affiliate to terminate his or her employment or service at any time.
    - (b) **Extend Employment** . Be construed to constitute an agreement, or an expression of intent, on the part of the Company or any Affiliate to extend the employment or service of Grantee beyond the time that he or she would normally be retired pursuant to the provisions of any present or future retirement plan or policy of the Company or any Affiliate, or beyond the time at which he or she would otherwise be retired pursuant to the provisions of any contract of employment with the Company or any Affiliate.
  13. **Clawback** . The Option (whether or not vested) is subject to forfeiture, termination and rescission, and Grantee will be obligated to return to the Company the value received with respect to the Option (including any gain realized on a subsequent sale or disposition of Common Shares) in accordance with any clawback or similar policy maintained by the Company or any Affiliate, as such policy may be amended and in effect from time to time, including, without limitation, Aegerion Pharmaceuticals, Inc.’s Policy on Executive Financial Recoupment Program, which provides for forfeiture and recoupment of an amount equivalent to up to three years of incentive-based compensation upon the occurrence of certain triggering events, or as otherwise required by law or applicable stock exchange listing standards, including, without limitation, Section 10D of the Securities Exchange Act of 1934, as amended.
  14. **Enurement** . The Award Agreement shall enure to the benefit of and be binding upon the parties to the Award Agreement and upon the successors or assigns of the Company and upon the executors, administrators and legal personal representatives of Grantee.
  15. **Further Assurances** . Each of the parties to the Award Agreement will do such further acts and execute such further documents as may required to give effect to and carry out the intent of the Award Agreement.
  16. **Non-Assignable** . The Option is personal to Grantee and may not be assigned or transferred in whole or in part, except by will or by the operation of the laws of devolution or distribution and descent.
  17. **Amendments** . Any amendments to the Award Agreement must be in writing duly executed by the parties and will (if required) be subject to the approval of the applicable regulatory authorities.
  18. **Time of the Essence** . Time is of the essence of the Award Agreement.
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19. **Governing Law** . The Award Agreement shall be governed, construed and enforced according to the laws of the Province of British Columbia and is subject to the exclusive jurisdiction of the courts of the Province of British Columbia.
20. **Interpretation of the Award Agreement and the Plan** . If any question or dispute arises as to the interpretation of the Award Agreement, the question or dispute will be determined by the Committee and such determination will be final, conclusive and binding for all purposes on both the Company and Grantee.
21. **Conflict Between Award Agreement and the Plan** . If there is any conflict between this Award Agreement and the Plan, the Plan, as amended from time to time, will govern.



NOVELION THERAPEUTICS INC.

AMENDED AND RESTATED NOVELION 2017 EQUITY INCENTIVE PLAN

RESTRICTED STOCK UNIT AWARD GRANT NOTICE AND  
RESTRICTED STOCK UNIT AWARD AGREEMENT

Novelion Therapeutics Inc. (the “Company”), pursuant to its Amended and Restated 2017 Equity Incentive Plan, as amended from time to time (the “Plan”), hereby grants to the individual listed below (“Grantee”), an award (“Award”) consisting of the number of restricted stock units (“Restricted Stock Units” or “RSUs”) set forth below. Each RSU represents the conditional right to receive, without payment but subject to the conditions and limitations set forth in this Restricted Stock Unit Award Grant Notice (the “Grant Notice”), the Restricted Stock Unit Award Agreement attached hereto as Exhibit A (the “Award Agreement”) and the Plan, one Common Share, subject to adjustment pursuant to Section 16 of the Plan in respect of transactions occurring on or after the date hereof. Unless otherwise defined in this Grant Notice or the Award Agreement, defined terms shall have the meaning set forth in the Plan.

**Grantee’s Name:**

**Grant Date:**

**Number of RSUs:**

**Vesting Commencement Date:**

**Vesting Schedule:**

*[Remainder of this page intentionally left blank.]*

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By accepting the Award, Grantee agrees to be bound by the terms and conditions of the Plan, the Award Agreement and this Grant Notice. Grantee has reviewed the Award Agreement, the Plan and this Grant Notice in their entirety and fully understands all provisions of the Award Agreement, the Plan and this Grant Notice. Additionally, by accepting the Award, Grantee agrees that he or she has read, fully understands and agrees to abide by the terms of the Company's Insider Trading Policy and has read and fully understands the Plan Prospectus, copies of which have been made available to Grantee. Grantee hereby agrees to accept as binding, conclusive and final all decisions or interpretations of the Committee regarding any questions arising under the Plan or relating to the RSUs.

**NOVELION THERAPEUTICS Inc.**

By: \_\_\_\_\_

Print Name: Linda Buono

Senior Vice President, Human Resources, Novelion  
Therapeutics, Inc.

Title:



## EXHIBIT A

### RESTRICTED STOCK UNIT AWARD AGREEMENT

Pursuant to the Restricted Stock Unit Award Grant Notice (the “Grant Notice”) to which this Restricted Stock Unit Award Agreement (the “Award Agreement”) is attached, Novelion Therapeutics Inc. (the “Company”) has granted to Grantee award of restricted stock units ( “Restricted Stock Units ” or “RSUs ” ) under the Company’s Amended and Restated 2017 Equity Incentive Plan, as amended from time to time (the “Plan”).

#### ARTICLE I.

##### GENERAL

1.1 Defined Terms. Capitalized terms not specifically defined herein shall have the meanings specified in the Plan and the Grant Notice.

1.2 Incorporation of Terms of Plan. RSUs are subject to the terms and conditions of the Plan which are incorporated herein by reference. In the event of any inconsistency between the Plan and this Award Agreement, the terms of the Plan shall control.

#### ARTICLE II.

##### GRANT OF RESTRICTED STOCK UNITS

2.1 Grant of RSUs. In consideration of Grantee’s service as an officer, employee or Consultant of the Company or its Affiliates and for other good and valuable consideration, effective as of the Grant Date set forth in the Grant Notice (the “Grant Date”), the Company grants to Grantee the number of RSUs as set forth in the Grant Notice (the “Award”). Each RSU represents the conditional right to receive, without payment but subject to the conditions and limitations set forth in the Grant Notice, this Award Agreement and the Plan, one Common Share, subject to adjustment pursuant to Section 16 of the Plan in respect of transactions occurring after the date hereof.

2.2 Company’s Obligation. Unless and until the RSUs have vested in a manner set forth in Article II hereof, Grantee will have no right to receive any Common Shares in respect of any such RSUs.

2.3 Vesting Schedule. No portion of the Award is vested as of the date hereof. Subject to Section 2.4 below, the RSUs will vest and become nonforfeitable according to the vesting schedule set forth on the Grant Notice to which this Award Agreement is attached (the “Vesting Schedule”).

2.4 Forfeiture, Termination and Cancellation upon Termination of Services.

(a) Termination of Service. Except to the extent contemplated in the Vesting Schedule, if applicable, or as otherwise set forth in subsection (b) below, upon Grantee’s Termination of Service for any reason, all unvested RSUs will be automatically forfeited, terminated and cancelled as of the applicable termination date without payment of any consideration by the Company, and Grantee, or Grantee’s beneficiary or personal representative, as the case may be, shall have no further rights hereunder.

(b) Sale Event. In the event that Grantee is party to an effective employment or similar individual agreement with the Company or its Affiliates that provides for the treatment of an equity award in connection with a “Sale Event” (as defined in such agreement), such provision shall only apply in connection with a “Sale Event” that occurs on or after the Grant Date (and shall not, for the avoidance of doubt, apply in connection with a “Sale Event” that occurred prior to the Grant Date).

2.5 Payment after Vesting.

(a) As soon as administratively practicable following the vesting of any RSUs (but in no event later than March 15 of the year following the year in which the RSUs become vested), the Company shall deliver to Grantee (or, in the event of Grantee’s death or complete disability, to the person to whom the Award has passed by will or the laws of descent and distribution or to Grantee’s legal guardian or representative, as applicable) a number of Common Shares equal to the number of RSUs that vested on the applicable vesting date. Notwithstanding the foregoing, in the event Common Shares cannot be issued pursuant to Section 2.7(a) or (b) hereof, then the Common Shares shall be issued pursuant to the preceding sentence as soon as administratively

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practicable after the Committee determines that Common Shares can again be issued in accordance with Sections 2.7(a) or (b) hereof.

(b) Grantee will be solely responsible for paying any applicable withholding taxes arising from the grant, vesting or settlement of any RSUs and any payment is to be in a manner satisfactory to the Company. Notwithstanding the foregoing, the Company will have the right to withhold from any amount payable to Grantee, either under the Plan or otherwise, such amount as may be necessary to enable the Company to comply with the applicable requirements of any federal, provincial, state, local or foreign law, or any administrative policy of any applicable tax authority, relating to the withholding of tax or any other required deductions (the “Withholding Obligations”). The Company may require Grantee, as a condition to the vesting or settlement of an RSU, to make such arrangements as the Company may require so that the Company can satisfy applicable Withholding Obligations, including, without limitation, requiring Grantee to (i) remit the amount of any such Withholding Obligations to the Company in advance; (ii) reimburse the Company for any such Withholding Obligations; (iii) deliver written instructions contemplated in Section 13.1(c) of the Plan, to effect a net settlement of Common Shares under an RSU in an amount required to satisfy any such Withholding Obligations; or (iv) pursuant to a transaction as contemplated in Section 13.1(b) of the Plan, cause such broker to withhold from the proceeds realized from such transaction the amount required to satisfy any such Withholding Obligations and to remit such amount directly to the Company.

Notwithstanding any other provision of this Award Agreement or the Plan to the contrary, if Grantee is an “executive officer” of the Company within the meaning of Section 13(k) of the U.S. Exchange Act, Grantee shall not be permitted to make payment with respect to any RSUs, or continue any extension of credit with respect to such payment, with a loan from the Company or a loan arranged by the Company in violation of Section 13(k) of the U.S. Exchange Act.

The Company shall not be obligated to deliver any new certificate representing Common Shares to Grantee or Grantee’s legal representative or enter such Common Shares in book entry form unless and until Grantee or Grantee’s legal representative shall have paid or otherwise satisfied in full the amount of all federal, state, provincial and local taxes applicable to the taxable income of Grantee resulting from the vesting and settlement of RSUs into Common Shares.

2.6 Rights as Shareholder. Unless otherwise determined by the Committee, Grantee shall possess no incidents of ownership with respect to the Common Shares underlying the RSUs and deliverable hereunder unless and until such Common Shares are transferred to Grantee pursuant to the terms of the Plan and this Award Agreement.

2.7 Conditions to Delivery of Common Shares. Subject to Section 13.5 of the Plan, the Common Shares deliverable hereunder, or any portion thereof, may be either previously authorized but unissued Common Shares or issued Common Shares which have then been reacquired by the Company. Such Common Shares shall be fully paid and nonassessable.

(a) The Company shall not be required to issue or deliver any Common Shares deliverable hereunder or portion thereof prior to fulfillment of all of the following conditions:

i. The completion of such registration or other qualification of such Common Shares or obtaining approval of such governmental authority as the Company will determine to be necessary or advisable in connection with the authorization, issuance or sale thereof;

ii. The admission of such Common Shares to listing or quotation on the Exchange;

iii. The obtaining of any approval or other clearance from any state, provincial or federal governmental agency which the Committee shall, in its absolute discretion, determine to be necessary or advisable;

iv. the receipt from Grantee of such representations, agreements and undertakings, including as to future dealings in such Common Shares, as the Company or its counsel determines to be necessary or advisable in order to safeguard against the violation of the securities laws of any jurisdiction;

(b) No fractional Common Shares shall be issued under this Award Agreement and any such fractional shares shall be eliminated by rounding down.

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**ARTICLE III.**  
**OTHER PROVISIONS**

3.1 Administration. The Committee shall have the power to interpret the Plan and this Award Agreement and to adopt such rules for the administration, interpretation and application of the Plan as are consistent therewith and to interpret, amend or revoke any such rules. All actions taken and all interpretations and determinations made by the Committee in good faith shall be final and binding upon Grantee, the Company and all other interested persons. No member of the Committee or the Board shall be personally liable for any action, determination or interpretation made in good faith with respect to the Plan, this Award Agreement or the RSUs.

3.2 Adjustments upon Specified Events. Upon the occurrence of certain events relating to the Common Shares contemplated by Section 16.1 of the Plan (including, without limitation, an extraordinary cash dividend on such Common Shares), the Committee shall make such adjustments as the Committee deems appropriate in the number of RSUs then outstanding and the number and kind of securities that may be issued in respect of the RSUs. Grantee acknowledges that the RSUs are subject to modification and termination in certain events as provided in this Award Agreement and Sections 15 and 16 of the Plan.

3.3 Grant is not Transferable. During the lifetime of Grantee, this grant and the rights and privileges conferred hereby will not be transferred, assigned, pledged or hypothecated in any way (whether by operation of law or otherwise) and will not be subject to sale under execution, attachment or similar process. Upon any attempt to transfer, assign, pledge, hypothecate or otherwise dispose of the RSUs, or any right or privilege conferred hereby, or upon any attempted sale under any execution, attachment or similar process, the RSUs and the rights and privileges conferred hereby immediately will become null and void. Notwithstanding anything herein to the contrary, this Section 3.3 shall not prevent transfers by will or by operation of the laws of devolution or distribution and descent or pursuant to a qualified domestic relations order, as defined by the U.S. Code.

3.4 Binding Agreement. Subject to the limitation on the transferability of the RSUs contained herein, this Award Agreement will be binding upon and inure to the benefit of the heirs, legatees, legal representatives, successors and assigns of the parties hereto.

3.5 Notices. Any notice to be given under the terms of this Award Agreement to the Company shall be addressed to the Company in care of the Secretary of the Company at the Company's principal office in Vancouver, B.C., and any notice to be given to Grantee shall be addressed to Grantee at Grantee's last address reflected on the records of the Company or its Affiliate. By a notice given pursuant to this Section 3.5, either party may hereafter designate a different address for notices to be given to that party. Any notice shall be deemed duly given when sent via email or when sent by certified mail (return receipt requested) and deposited (with postage prepaid) in a post office or branch post office regularly maintained by the United States Postal Service or Canada Post, as applicable.

3.6 Titles. The division of this Award Agreement into Sections and Articles and the insertion of headings are for convenience of reference only and will not affect the construction or interpretation of this Award Agreement or the Plan.

3.7 Governing Law; Severability. The laws of the Province of British Columbia shall govern the interpretation, validity, administration, enforcement and performance of the terms of this Award Agreement regardless of the law that might be applied under principles of conflicts of laws.

3.8 Conformity to Securities Laws. Grantee acknowledges that the Plan and this Award Agreement are intended to conform to the extent necessary with all applicable provisions of the U.S. Securities Act and the U.S. Securities Exchange Act and any and all regulations and rules promulgated by the U.S. Securities and Exchange Commission thereunder, and applicable state and Canadian securities laws and regulations. This Award Agreement, the Plan, the granting and vesting of the RSUs under the Plan and this Award Agreement, and the settlement and delivery of Common Shares hereunder are subject to compliance with all applicable federal, state, provincial, local and foreign laws, rules and regulations (including but not limited to state, provincial, federal and foreign securities law and margin requirements) and to such approvals by any stock exchange, regulatory or governmental authority as may, in the opinion of counsel for the Company, be necessary or advisable in connection therewith. Any securities delivered under this Award Agreement or the Plan shall be subject to such restrictions, and the person acquiring such securities shall, if requested by the Company, provide such assurances and representations to the Company as the Company may deem necessary or desirable to assure compliance with all applicable legal requirements. To the extent permitted by applicable law, this Award Agreement, the Plan and the RSUs granted hereunder shall be deemed amended to the extent necessary to conform to such laws, rules and regulations.

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3.9 Suspension, Amendment or Termination. To the extent permitted by the Plan, this Award Agreement may be wholly or partially amended or otherwise modified, suspended or terminated at any time or from time to time by the Committee, *provided*, that, the Committee will not have the right, without the consent of Grantee, to affect in a manner that is adverse or prejudicial to, or that impairs, the benefits and/or rights of Grantee under this Award Agreement (subject to any necessary adjustment pursuant to Article 16 of the Plan).

3.10 Successors and Assigns. The Company may assign any of its rights under this Award Agreement to single or multiple assignees, and this Award Agreement shall inure to the benefit of the successors and assigns of the Company. Subject to the restrictions on transfer herein set forth in Section 3.3 hereof, this Agreement shall be binding upon Grantee and his or her heirs, executors, administrators, successors and assigns.

3.11 Limitations Applicable to Section 16 Persons. Notwithstanding any other provision of the Plan or this Award Agreement, if Grantee is subject to Section 16 of the U.S. Exchange Act, the Plan, the RSUs and this Award Agreement shall be subject to any additional limitations set forth in any applicable exemptive rule under Section 16 of the U.S. Exchange Act (including any amendment to Rule 16b-3 of the U.S. Exchange Act) that are requirements for the application of such exemptive rule. To the extent permitted by applicable law, this Award Agreement shall be deemed amended to the extent necessary to conform to such applicable exemptive rule.

3.12 Not a Contract of Employment. Nothing in this Award Agreement or the Plan will confer upon Grantee any right to continue in the employ or service of or under contract with the Company or any Affiliate or affect in any way the right of the Company or any such Affiliate to terminate his or her employment or service at any time; nor will anything in this Award Agreement or the Plan be deemed or construed to constitute an agreement, or an expression of intent, on the part of the Company or any such Affiliate to extend the employment or the service of Grantee beyond the time that he or she would normally be retired pursuant to the provisions of any present or future retirement plan of the Company or any Affiliate or any present or future retirement policy of the Company or any Affiliate, or beyond the time at which he or she would otherwise be retired pursuant to the provisions of any contract of employment with the Company or any Affiliate.

3.13 Entire Agreement. The Plan, the Grant Notice and this Award Agreement constitute the entire agreement of the parties and supersede in their entirety all prior undertakings and agreements of the Company and Grantee with respect to the subject matter hereof, except for provisions of an employment agreement that cover the subject matter hereof.

3.14 Section 409A. The RSUs are not intended to constitute “nonqualified deferred compensation” within the meaning of Section 409A of the U.S. Code (together with any Department of Treasury regulations and other interpretive guidance issued thereunder, including without limitation any such regulations or other guidance that may be issued after the date hereof, “Section 409A”). However, notwithstanding any other provision of the Plan, the Grant Notice or this Award Agreement, if at any time the Committee determines that the RSUs (or any portion thereof) may be subject to Section 409A, the Committee shall have the right in its sole discretion (without any obligation to do so or to indemnify Grantee or any other person for failure to do so) to adopt such amendments to the Plan, this Award Agreement or the Grant Notice or adopt other policies and procedures (including amendments, policies and procedures with retroactive effect), or take any other actions, as the Committee determines are necessary or appropriate either for the RSUs to be exempt from the application of Section 409A or to comply with the requirements of Section 409A. Neither the Company, nor any subsidiary, nor the Committee or Board, nor any person acting on behalf of the Company, any subsidiary, or the Committee or Board, shall be liable to Grantee or to the estate or beneficiary of Grantee by reason of any acceleration of income, or any additional tax, asserted by reason of the failure of the Grant Notice, this Award Agreement or any payment hereunder to satisfy the requirements of Section 409A.

3.15 Limitation on Grantee’s Rights. Participation in the Plan confers no rights or interests other than as herein provided. This Award Agreement creates only a contractual obligation on the part of the Company as to amounts payable and shall not be construed as creating a trust. Neither the Plan nor any underlying program, in and of itself, has any assets. Grantee shall have only the rights of a general unsecured creditor of the Company with respect to amounts credited and benefits payable, if any, with respect to the RSUs, and rights no greater than the right to receive Common Shares as a general unsecured creditor with respect to RSUs, as and when payable hereunder.

3.16 Election Under Section 83(b). Grantee expressly acknowledges that because this Award consists of an unfunded and unsecured promise by the Company to deliver Common Shares in the future, subject to the terms hereof, it is not possible to make a so-called “83(b) election” with respect to the Award.

NOVELION THERAPEUTICS INC.

AMENDED AND RESTATED  
NOVELION 2017 EQUITY INCENTIVE PLAN

RESTRICTED STOCK UNIT AWARD GRANT NOTICE AND  
RESTRICTED STOCK UNIT AWARD AGREEMENT

(Executives)

Novelion Therapeutics Inc. (the “Company”), pursuant to its Amended and Restated 2017 Equity Incentive Plan, as amended from time to time (the “Plan”), hereby grants to the individual listed below (“Grantee”), an award (“Award”) consisting of the number of restricted stock units (“Restricted Stock Units” or “RSUs”) set forth below. Each RSU represents the conditional right to receive, without payment but subject to the conditions and limitations set forth in this Restricted Stock Unit Award Grant Notice (the “Grant Notice”), the Restricted Stock Unit Award Agreement attached hereto as Exhibit A (the “Award Agreement”) and the Plan, one Common Share, subject to adjustment pursuant to Section 16 of the Plan in respect of transactions occurring on or after the date hereof. Unless otherwise defined in this Grant Notice or the Award Agreement, defined terms shall have the meaning set forth in the Plan.

**Grantee’s Name:**

**Grant Date:**

**Number of RSUs:**

**Vesting Commencement Date:**

**Vesting Schedule:**

*[Remainder of this page intentionally left blank.]*

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By accepting the Award, Grantee agrees to be bound by the terms and conditions of the Plan, the Award Agreement and this Grant Notice. Grantee has reviewed the Award Agreement, the Plan and this Grant Notice in their entirety and fully understands all provisions of the Award Agreement, the Plan and this Grant Notice. Additionally, by accepting the Award, Grantee agrees that he or she has read, fully understands and agrees to abide by the terms of the Company's Insider Trading Policy and has read and fully understands the Plan Prospectus, copies of which have been made available to Grantee. Grantee hereby agrees to accept as binding, conclusive and final all decisions or interpretations of the Committee regarding any questions arising under the Plan or relating to the RSUs.

**NOVELION THERAPEUTICS Inc.**

By: \_\_\_\_\_

Print Name: Linda Buono

Title: Senior Vice President, Human Resources

The Grant Notice and Award Agreement are hereby accepted and the terms and conditions thereof hereby agreed to by the undersigned.

By: \_\_\_\_\_

Name:

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## EXHIBIT A

### RESTRICTED STOCK UNIT AWARD AGREEMENT

Pursuant to the Restricted Stock Unit Award Grant Notice (the “Grant Notice”) to which this Restricted Stock Unit Award Agreement (the “Award Agreement”) is attached, Novelion Therapeutics Inc. (the “Company”) has granted to Grantee award of restricted stock units ( “Restricted Stock Units ” or “RSUs ” ) under the Company’s Amended and Restated 2017 Equity Incentive Plan, as amended from time to time (the “Plan”).

#### ARTICLE I.

##### GENERAL

1.1 Defined Terms. Capitalized terms not specifically defined herein shall have the meanings specified in the Plan and the Grant Notice.

1.2 Incorporation of Terms of Plan. RSUs are subject to the terms and conditions of the Plan which are incorporated herein by reference. In the event of any inconsistency between the Plan and this Award Agreement, the terms of the Plan shall control.

#### ARTICLE II.

##### GRANT OF RESTRICTED STOCK UNITS

2.1 Grant of RSUs. In consideration of Grantee’s service as an officer, employee or Consultant of the Company or its Affiliates and for other good and valuable consideration, effective as of the Grant Date set forth in the Grant Notice (the “Grant Date”), the Company grants to Grantee the number of RSUs as set forth in the Grant Notice (the “Award”). Each RSU represents the conditional right to receive, without payment but subject to the conditions and limitations set forth in the Grant Notice, this Award Agreement and the Plan, one Common Share, subject to adjustment pursuant to Section 16 of the Plan in respect of transactions occurring after the date hereof.

2.2 Company’s Obligation. Unless and until the RSUs have vested in a manner set forth in Article II hereof, Grantee will have no right to receive any Common Shares in respect of any such RSUs.

2.3 Vesting Schedule. No portion of the Award is vested as of the date hereof. Subject to Section 2.4 below, the RSUs will vest and become nonforfeitable according to the vesting schedule set forth on the Grant Notice to which this Award Agreement is attached (the “Vesting Schedule”).

2.4 Forfeiture, Termination and Cancellation upon Termination of Services.

(a) Termination of Service. Except to the extent contemplated in the Vesting Schedule, if applicable, or as otherwise set forth in subsection (b) below, upon Grantee’s Termination of Service for any reason, all unvested RSUs will be automatically forfeited, terminated and cancelled as of the applicable termination date without payment of any consideration by the Company, and Grantee, or Grantee’s beneficiary or personal representative, as the case may be, shall have no further rights hereunder.

(b) Sale Event. In the event that Grantee is party to an effective employment or similar individual agreement with the Company or its Affiliates that provides for the treatment of an equity award in connection with a “Sale Event” (as defined in such agreement), such provision shall only apply in connection with a “Sale Event” that occurs on or after the Grant Date (and shall not, for the avoidance of doubt, apply in connection with a “Sale Event” that occurred prior to the Grant Date).

2.5 Payment after Vesting.

(a) As soon as administratively practicable following the vesting of any RSUs (but in no event later than March 15 of the year following the year in which the RSUs become vested), the Company shall deliver to Grantee (or, in the event of Grantee’s death or complete disability, to the person to whom the Award has passed by will or the laws of descent and distribution or to Grantee’s legal guardian or representative, as applicable) a number of Common Shares equal to the number of RSUs that vested on the applicable vesting date. Notwithstanding the foregoing, in the event Common Shares cannot be issued pursuant to Section 2.7(a) or (b) hereof, then the Common Shares shall be issued pursuant to the preceding sentence as soon as administratively

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practicable after the Committee determines that Common Shares can again be issued in accordance with Sections 2.7(a) or (b) hereof.

(b) Grantee will be solely responsible for paying any applicable withholding taxes arising from the grant, vesting or settlement of any RSUs and any payment is to be in a manner satisfactory to the Company. Notwithstanding the foregoing, the Company will have the right to withhold from any amount payable to Grantee, either under the Plan or otherwise, such amount as may be necessary to enable the Company to comply with the applicable requirements of any federal, provincial, state, local or foreign law, or any administrative policy of any applicable tax authority, relating to the withholding of tax or any other required deductions (the “Withholding Obligations”). The Company may require Grantee, as a condition to the vesting or settlement of an RSU, to make such arrangements as the Company may require so that the Company can satisfy applicable Withholding Obligations, including, without limitation, requiring Grantee to (i) remit the amount of any such Withholding Obligations to the Company in advance; (ii) reimburse the Company for any such Withholding Obligations; (iii) deliver written instructions contemplated in Section 13.1(c) of the Plan, to effect a net settlement of Common Shares under an RSU in an amount required to satisfy any such Withholding Obligations; or (iv) pursuant to a transaction as contemplated in Section 13.1(b) of the Plan, cause such broker to withhold from the proceeds realized from such transaction the amount required to satisfy any such Withholding Obligations and to remit such amount directly to the Company.

Notwithstanding any other provision of this Award Agreement or the Plan to the contrary, if Grantee is an “executive officer” of the Company within the meaning of Section 13(k) of the U.S. Exchange Act, Grantee shall not be permitted to make payment with respect to any RSUs, or continue any extension of credit with respect to such payment, with a loan from the Company or a loan arranged by the Company in violation of Section 13(k) of the U.S. Exchange Act.

The Company shall not be obligated to deliver any new certificate representing Common Shares to Grantee or Grantee’s legal representative or enter such Common Shares in book entry form unless and until Grantee or Grantee’s legal representative shall have paid or otherwise satisfied in full the amount of all federal, state, provincial and local taxes applicable to the taxable income of Grantee resulting from the vesting and settlement of RSUs into Common Shares.

2.6 Rights as Shareholder. Unless otherwise determined by the Committee, Grantee shall possess no incidents of ownership with respect to the Common Shares underlying the RSUs and deliverable hereunder unless and until such Common Shares are transferred to Grantee pursuant to the terms of the Plan and this Award Agreement.

2.7 Conditions to Delivery of Common Shares. Subject to Section 13.5 of the Plan, the Common Shares deliverable hereunder, or any portion thereof, may be either previously authorized but unissued Common Shares or issued Common Shares which have then been reacquired by the Company. Such Common Shares shall be fully paid and nonassessable.

(a) The Company shall not be required to issue or deliver any Common Shares deliverable hereunder or portion thereof prior to fulfillment of all of the following conditions:

i. The completion of such registration or other qualification of such Common Shares or obtaining approval of such governmental authority as the Company will determine to be necessary or advisable in connection with the authorization, issuance or sale thereof;

ii. The admission of such Common Shares to listing or quotation on the Exchange;

iii. The obtaining of any approval or other clearance from any state, provincial or federal governmental agency which the Committee shall, in its absolute discretion, determine to be necessary or advisable;

iv. the receipt from Grantee of such representations, agreements and undertakings, including as to future dealings in such Common Shares, as the Company or its counsel determines to be necessary or advisable in order to safeguard against the violation of the securities laws of any jurisdiction;

(b) No fractional Common Shares shall be issued under this Award Agreement and any such fractional shares shall be eliminated by rounding down.

### ARTICLE III.

#### OTHER PROVISIONS

3.1 Administration. The Committee shall have the power to interpret the Plan and this Award Agreement and to adopt such rules for the administration, interpretation and application of the Plan as are consistent therewith and to interpret, amend

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or revoke any such rules. All actions taken and all interpretations and determinations made by the Committee in good faith shall be final and binding upon Grantee, the Company and all other interested persons. No member of the Committee or the Board shall be personally liable for any action, determination or interpretation made in good faith with respect to the Plan, this Award Agreement or the RSUs.

3.2 Adjustments upon Specified Events. Upon the occurrence of certain events relating to the Common Shares contemplated by Section 16.1 of the Plan (including, without limitation, an extraordinary cash dividend on such Common Shares), the Committee shall make such adjustments as the Committee deems appropriate in the number of RSUs then outstanding and the number and kind of securities that may be issued in respect of the RSUs. Grantee acknowledges that the RSUs are subject to modification and termination in certain events as provided in this Award Agreement and Sections 15 and 16 of the Plan.

3.3 Grant is not Transferable. During the lifetime of Grantee, this grant and the rights and privileges conferred hereby will not be transferred, assigned, pledged or hypothecated in any way (whether by operation of law or otherwise) and will not be subject to sale under execution, attachment or similar process. Upon any attempt to transfer, assign, pledge, hypothecate or otherwise dispose of the RSUs, or any right or privilege conferred hereby, or upon any attempted sale under any execution, attachment or similar process, the RSUs and the rights and privileges conferred hereby immediately will become null and void. Notwithstanding anything herein to the contrary, this Section 3.3 shall not prevent transfers by will or by operation of the laws of devolution or distribution and descent or pursuant to a qualified domestic relations order, as defined by the U.S. Code.

3.4 Binding Agreement. Subject to the limitation on the transferability of the RSUs contained herein, this Award Agreement will be binding upon and inure to the benefit of the heirs, legatees, legal representatives, successors and assigns of the parties hereto.

3.5 Notices. Any notice to be given under the terms of this Award Agreement to the Company shall be addressed to the Company in care of the Secretary of the Company at the Company's principal office in Vancouver, B.C., and any notice to be given to Grantee shall be addressed to Grantee at Grantee's last address reflected on the records of the Company or its Affiliate. By a notice given pursuant to this Section 3.5, either party may hereafter designate a different address for notices to be given to that party. Any notice shall be deemed duly given when sent via email or when sent by certified mail (return receipt requested) and deposited (with postage prepaid) in a post office or branch post office regularly maintained by the United States Postal Service or Canada Post, as applicable.

3.6 Titles. The division of this Award Agreement into Sections and Articles and the insertion of headings are for convenience of reference only and will not affect the construction or interpretation of this Award Agreement or the Plan.

3.7 Governing Law; Severability. The laws of the Province of British Columbia shall govern the interpretation, validity, administration, enforcement and performance of the terms of this Award Agreement regardless of the law that might be applied under principles of conflicts of laws.

3.8 Conformity to Securities Laws. Grantee acknowledges that the Plan and this Award Agreement are intended to conform to the extent necessary with all applicable provisions of the U.S. Securities Act and the U.S. Securities Exchange Act and any and all regulations and rules promulgated by the U.S. Securities and Exchange Commission thereunder, and applicable state and Canadian securities laws and regulations. This Award Agreement, the Plan, the granting and vesting of the RSUs under the Plan and this Award Agreement, and the settlement and delivery of Common Shares hereunder are subject to compliance with all applicable federal, state, provincial, local and foreign laws, rules and regulations (including but not limited to state, provincial, federal and foreign securities law and margin requirements) and to such approvals by any stock exchange, regulatory or governmental authority as may, in the opinion of counsel for the Company, be necessary or advisable in connection therewith. Any securities delivered under this Award Agreement or the Plan shall be subject to such restrictions, and the person acquiring such securities shall, if requested by the Company, provide such assurances and representations to the Company as the Company may deem necessary or desirable to assure compliance with all applicable legal requirements. To the extent permitted by applicable law, this Award Agreement, the Plan and the RSUs granted hereunder shall be deemed amended to the extent necessary to conform to such laws, rules and regulations.

3.9 Suspension, Amendment or Termination. To the extent permitted by the Plan, this Award Agreement may be wholly or partially amended or otherwise modified, suspended or terminated at any time or from time to time by the Committee, *provided*, that, the Committee will not have the right, without the consent of Grantee, to affect in a manner that is adverse or prejudicial to, or that impairs, the benefits and/or rights of Grantee under this Award Agreement (subject to any necessary adjustment pursuant to Article 16 of the Plan).

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3.10 Successors and Assigns. The Company may assign any of its rights under this Award Agreement to single or multiple assignees, and this Award Agreement shall inure to the benefit of the successors and assigns of the Company. Subject to the restrictions on transfer herein set forth in Section 3.3 hereof, this Agreement shall be binding upon Grantee and his or her heirs, executors, administrators, successors and assigns.

3.11 Limitations Applicable to Section 16 Persons. Notwithstanding any other provision of the Plan or this Award Agreement, if Grantee is subject to Section 16 of the U.S. Exchange Act, the Plan, the RSUs and this Award Agreement shall be subject to any additional limitations set forth in any applicable exemptive rule under Section 16 of the U.S. Exchange Act (including any amendment to Rule 16b-3 of the U.S. Exchange Act) that are requirements for the application of such exemptive rule. To the extent permitted by applicable law, this Award Agreement shall be deemed amended to the extent necessary to conform to such applicable exemptive rule.

3.12 Not a Contract of Employment. Nothing in this Award Agreement or the Plan will confer upon Grantee any right to continue in the employ or service of or under contract with the Company or any Affiliate or affect in any way the right of the Company or any such Affiliate to terminate his or her employment or service at any time; nor will anything in this Award Agreement or the Plan be deemed or construed to constitute an agreement, or an expression of intent, on the part of the Company or any such Affiliate to extend the employment or the service of Grantee beyond the time that he or she would normally be retired pursuant to the provisions of any present or future retirement plan of the Company or any Affiliate or any present or future retirement policy of the Company or any Affiliate, or beyond the time at which he or she would otherwise be retired pursuant to the provisions of any contract of employment with the Company or any Affiliate.

3.13 Entire Agreement. The Plan, the Grant Notice and this Award Agreement constitute the entire agreement of the parties and supersede in their entirety all prior undertakings and agreements of the Company and Grantee with respect to the subject matter hereof, except for provisions of an employment agreement that cover the subject matter hereof.

3.14 Section 409A. The RSUs are not intended to constitute “nonqualified deferred compensation” within the meaning of Section 409A of the U.S. Code (together with any Department of Treasury regulations and other interpretive guidance issued thereunder, including without limitation any such regulations or other guidance that may be issued after the date hereof, “Section 409A”). However, notwithstanding any other provision of the Plan, the Grant Notice or this Award Agreement, if at any time the Committee determines that the RSUs (or any portion thereof) may be subject to Section 409A, the Committee shall have the right in its sole discretion (without any obligation to do so or to indemnify Grantee or any other person for failure to do so) to adopt such amendments to the Plan, this Award Agreement or the Grant Notice or adopt other policies and procedures (including amendments, policies and procedures with retroactive effect), or take any other actions, as the Committee determines are necessary or appropriate either for the RSUs to be exempt from the application of Section 409A or to comply with the requirements of Section 409A. Neither the Company, nor any subsidiary, nor the Committee or Board, nor any person acting on behalf of the Company, any subsidiary, or the Committee or Board, shall be liable to Grantee or to the estate or beneficiary of Grantee by reason of any acceleration of income, or any additional tax, asserted by reason of the failure of the Grant Notice, this Award Agreement or any payment hereunder to satisfy the requirements of Section 409A.

3.15 Limitation on Grantee’s Rights. Participation in the Plan confers no rights or interests other than as herein provided. This Award Agreement creates only a contractual obligation on the part of the Company as to amounts payable and shall not be construed as creating a trust. Neither the Plan nor any underlying program, in and of itself, has any assets. Grantee shall have only the rights of a general unsecured creditor of the Company with respect to amounts credited and benefits payable, if any, with respect to the RSUs, and rights no greater than the right to receive Common Shares as a general unsecured creditor with respect to RSUs, as and when payable hereunder.

3.16 Election Under Section 83(b). Grantee expressly acknowledges that because this Award consists of an unfunded and unsecured promise by the Company to deliver Common Shares in the future, subject to the terms hereof, it is not possible to make a so-called “83(b) election” with respect to the Award.

3.17 Section 4985. If any amount payable or paid by the Company or any of its affiliates pursuant to this Agreement or otherwise to or for the benefit of Grantee becomes subject to the excise tax imposed by Section 4985 of the Code (including any interest, penalties or additions to tax relating thereto) (the “4985 Excise Tax”) by reason of the consummation of the transactions contemplated by the Agreement and Plan of Merger, dated as of June 14, 2016 (as amended), among QLT, Inc., Aegerion Pharmaceuticals, Inc. and certain other parties thereto, as reasonably determined by the Company, then the Company shall pay to Grantee (1) an amount equal to the 4985 Excise Tax, and (2) an amount (the “4985 Gross-up Payment”) equal to the amount necessary to put Grantee in the same net after-tax position (taking into account any and all applicable Federal, state, local and foreign income, employment, excise and other taxes) that Grantee would have been in if Grantee had not incurred any liability for taxes under Section 4985 of the Code. Any determination regarding the amount of any payment or payments hereunder shall

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be made in writing by the Company's independent accountants or other accounting or consulting firm selected by the Company, whose determination shall be conclusive and binding upon Grantee and the Company for all purposes.

3.18 Clawback. The RSUs (whether or not vested) are subject to forfeiture, termination and rescission, and Grantee will be obligated to return to the Company the value received with respect to the RSUs (including any gain realized on a subsequent sale or disposition of Common Shares) in accordance with any clawback or similar policy maintained by the Company or any Affiliate, as such policy may be amended and in effect from time to time, including, without limitation, Aegerion Pharmaceuticals, Inc.'s Policy on Executive Financial Recoupment Program, which provides for forfeiture and recoupment of an amount equivalent to up to three years of incentive-based compensation upon the occurrence of certain triggering events, or as otherwise required by law or applicable stock exchange listing standards, including, without limitation, Section 10D of the Securities Exchange Act of 1934, as amended.

## EMPLOYMENT AGREEMENT

This Employment Agreement (“Agreement”) is made as of the 31st day of October, 2017, between Novelion Services USA, Inc., a Delaware corporation (the “Company”), and Jeffrey Hackman (the “Executive”).

WHEREAS, the Company desires to employ the Executive and the Executive desires to be employed by the Company beginning on or before November 1, 2017 (the “Commencement Date”) on the terms contained herein.

NOW, THEREFORE, in consideration of the mutual covenants and agreements herein contained and other good and valuable consideration, the receipt and sufficiency of which is hereby acknowledged, the parties agree as follows:

1. Employment.

(a) Term. The Company hereby employs the Executive, and the Executive hereby accepts such employment, commencing as of the Commencement Date and continuing on an at-will basis until terminated by either party in accordance with the provisions of Section 3 (“Term”).

(b) Position and Duties. During the Term, the Executive shall serve as the Executive Vice President, Chief Operating Office (“COO”), reporting to the Company’s Chief Executive Officer (“CEO). Pursuant to the Master Service Agreement between the Company and Novelion Therapeutics, Inc. (“Novelion”) dated November 29, 2016 (the “Service Agreement”), Executive may also be required, on behalf of the Company, to perform services to Novelion and its other Affiliates, including holding an office in Novelion. As of the Effective Date, these services shall include serving as COO of Novelion, and such other duties consistent with the Service Agreement as may be assigned and/or prescribed from time to time by the CEO, the Board of Directors of the Company (the “Board”) or its designee, or by the Board of Directors of Novelion (the “Novelion Board”) pursuant to the Service Agreement, provided that such duties are consistent with the Executive’s position or other positions that he may hold from time to time. The Executive will comply with the policies of the Company and Aegerion. For certainty, at all times Executive will be an employee of the Company and not an employee of Novelion, and when Executive provides services to Novelion he will be doing so as an employee of the Company performing contracted management services as provided to Novelion under the Service Agreement. For the purpose of this Agreement, “Affiliate” with reference to the Company and Novelion, shall have the meaning given to it in the Delaware General Corporation Law as of the date of this Agreement and, for certainty includes, without limitation, Novelion and Aegerion Pharmaceuticals, Inc. (“Aegerion”) and any other current or future Affiliates of the Company.

(c) Performance. Executive shall devote his full business time, attention, skill, and best efforts to the performance of his duties under this Agreement and shall not engage in any other business or occupation during the Term, including, without limitation, any activity that (x) conflicts with the interests of the Company or any of its Affiliates, (y) interferes with the proper and efficient performance of Executive’s duties for the Company or any of its Affiliates, or (z) interferes with Executive’s exercise of judgment in the Company’s or any of its Affiliates’ best interests. Notwithstanding the foregoing, nothing herein shall preclude Executive from (i) serving, with the prior written consent of the Novelion Board, as a member of the boards of directors or advisory boards (or their equivalents in the case of a non-corporate entity) of non-competing businesses and charitable organizations, (ii) engaging in charitable activities and community affairs, and (iii) managing Executive’s personal investments and affairs; *provided, however*, that the activities set out in clauses (i), (ii), and (iii) shall be limited by Executive so as not to interfere, individually or in the aggregate, with the performance of Executive’s duties and responsibilities hereunder. Executive represents that he has provided the Company with a comprehensive list of all outside professional activities with which he is currently involved or reasonably expects to become involved. In the event that, during his employment by the Company, the Executive desires to engage in other outside professional activities, not included on such list, Executive will first seek written approval from the Novelion CEO and such approval shall not be unreasonably withheld.

2. Compensation and Related Matters.

(a) Base Salary. During the Term, the Executive’s initial annual base salary shall be \$480,000. The base salary in effect at any given time is referred to herein as “Base Salary.” The Base Salary shall be payable in a manner that is consistent with the Company’s usual payroll practices for senior executives. All payments in this Agreement are on a gross, pre-tax basis and shall be subject to all applicable federal, state and local, and if applicable Canadian federal and provincial, withholding, payroll and other taxes required by law. Executive’s Base Salary will be subject to increase by the Board following approval by the Novelion Board of an increase in the Base Salary.

(b) Target Bonus. Beginning January 1, 2018, the Executive shall be eligible to earn an annual target bonus, based upon the level of achievement of the Executive, and Novelion and the Company against his/their corporate goals and milestones, as determined by the Board in its sole discretion (“Target Bonus”). The Target Bonus, if any, shall be paid no later than March 15 of the calendar year following the year in which it is earned. The Executive’s Target Bonus shall be 45% percent of his Base Salary. To earn a Target Bonus, the Executive must be employed by the Company on the day such Target Bonus is paid.

(c) Stock Options/Equity Grants. Subject to approval by the Compensation Committee of the Novelion Board, the Company will grant Executive: (i) an option (the “Option Award”) to purchase 135,000 shares of the Company’s common shares (the “Common Shares”); (ii) an award of 50,000 restricted stock units (the “RSU Award”); and (iii) an award of 92,500 restricted stock units (the “Additional RSU Award”). Each of these awards will have a grant date of Executive’s Commencement Date, will be subject to vesting and will be issued pursuant to, and subject to, the terms of the Amended and Restated Novelion 2017 Equity Incentive Plan (or a successor plan, if any) and subject to the terms of agreements thereunder (collectively the “Equity Documents”). The Option Award shall have an exercise price equal to the fair market value of the Common Stock on the grant date. The vesting schedule for the Option Award will be the vesting schedule outlined in the Equity Documents for the Option Awards (i.e., the option to purchase 135,000 shares will vest in three (3) equal installments on the first three (3) anniversaries of the grant date. The vesting schedule for the RSU Award will be the vesting schedule outlined in the Equity Documents for the RSU Award (i.e., the RSU Award will vest in three (3) equal installments on the first three (3) anniversaries of the grant date. The vesting schedule for the Additional RSU Award will be the vesting schedule outlined in the Equity Documents for the Additional RSU Award (i.e., the Additional RSU Award will vest in full on the first anniversary of the grant date). The full terms and conditions related to these option grants shall be set forth in the Equity Documents and to the extent that there is any inconsistency between this Agreement and the Equity Documents, the Equity Documents shall control. Because the Commencement Date will be after October 1, 2017, the Executive will not be eligible to receive equity awards as part of the 2017 performance review cycle.

The Executive will be also entitled to participate in those equity incentive plans and programs provided from time to time to the Executive by Novelion on the terms and conditions for such participation as established and changed from time to time by Novelion in its sole discretion. Section 5 of this Agreement contains terms and conditions covering the acceleration of such equity awards upon the occurrence of certain events. To the extent that there is any inconsistency between the applicable terms of Section 5 of this Agreement and the Equity Documents, this Agreement shall control.

The Executive is subject to, and will abide by, Novelion’s Insider Trading Policy, as amended by the Novelion Board from time to time, and is required to file insider reports disclosing the grant or exercise of any options and restricted stock units as well as the acquisition and sale of any shares in the Company. The Executive will comply with pre-approval, notification and other internal procedures set forth in Novelion’s Insider Trading Policy or as otherwise established by Novelion and communicated to the Executive. The Executive will also abide by the share ownership guidelines of Novelion as may be established and amended by the Novelion Board from time to time.

(d) Annual Tax and Financial Planning Reimbursements. During the Term, for as long as Executive continues to provide management services on behalf of the Company in Canada, Executive will be entitled to annual reimbursement up to a maximum of USD \$5,000 for his reasonable expenses for independent tax consultation regarding the Canadian tax implications of Executive’s work on behalf of the Company in Canada and/or preparation of Executive’s Canadian tax return.

(e) Forfeiture and Recoupment of Incentive-based Compensation. The Target Bonus and any equity awards that Executive receives in accordance with Section 4(b), Section 5 and Section 2(c) of this Agreement are subject to Aegerion’s Policy on the Executive Financial Recoupment Program, as may be amended by Aegerion from time to time in its sole discretion. The Policy on Executive Financial Recoupment Program provides for forfeiture and recoupment of an amount equivalent to up to three years of incentive-based compensation upon the occurrence of certain triggering events.

(f) Executive Benefits and Vacation. During the Term, Executive shall be eligible to participate in health insurance and other benefits provided generally to similarly situated employees of the Company, subject to the terms and conditions of the applicable benefit plans (which shall govern). Executive also shall be eligible for the same number of holidays and vacation days as well as any other benefits, in each case as are generally allowed to similarly situated employees of the Company in accordance with the Company policy as in effect from time to time. Nothing contained herein shall be construed to limit the Company’s ability to amend, suspend, or terminate any employee benefit plan or policy at any time without providing Executive notice, and the right to do so is expressly reserved.

(g) Reimbursement of Business Expenses. During the Term of Employment, the Company shall pay (or promptly reimburse Executive) for documented, out-of-pocket expenses reasonably incurred by Executive in the course of performing his duties and responsibilities hereunder, which are consistent with the Company’s policies in effect from time to time

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with respect to business expenses, subject to the Company's requirements with respect to reporting of such expenses; provided that, the terms of the Expatriate Assignment Letter shall govern the payment or reimbursement of expenses incurred in connection with any Expatriate Assignment.

3. Termination. During the Term, the Executive's employment hereunder may be terminated without any breach of this Agreement under the following circumstances:

(a) Death. The Executive's employment hereunder shall terminate upon his death.

(b) Disability. The Company may terminate the Executive's employment if he is disabled and unable to perform the essential functions of the Executive's then existing position or positions under this Agreement with or without reasonable accommodation for a period of 90 consecutive days, or 180 days (which need not be consecutive) in any 12-month period. If any question shall arise as to whether during any period the Executive is disabled so as to be unable to perform the essential functions of the Executive's then existing position or positions with or without reasonable accommodation, the Executive may, and at the request of the Company shall, submit to the Company a certification in reasonable detail by a physician selected by the Company to whom the Executive or the Executive's guardian has no reasonable objection as to whether the Executive is so disabled or how long such disability is expected to continue, and such certification shall for the purposes of this Agreement be conclusive of the issue. The Executive shall cooperate with any reasonable request of the physician in connection with such certification. If such question shall arise and the Executive shall fail to submit such certification, the Company's determination of such issue shall be binding on the Executive. Nothing in this Section 3(b) shall be construed to waive the Executive's rights, if any, under existing law including, without limitation, the Family and Medical Leave Act of 1993, 29 U.S.C. §2601 *et seq.* and the Americans with Disabilities Act, 42 U.S.C. §12101 *et seq.*

(c) Termination by Company for Cause. The Company may terminate the Executive's employment hereunder for Cause. For purposes of this Agreement, "Cause" shall mean: (i) conduct by the Executive constituting a material act of misconduct in connection with the performance of his duties, including, without limitation, misappropriation of funds or property of the Company or any of its subsidiaries or affiliates other than the occasional, customary and de minimis use of Company property for personal purposes; (ii) a material and willful misrepresentation by the Executive to the CEO or the Board regarding a matter of material importance to the Company; (iii) the commission by the Executive of any felony or a misdemeanor involving moral turpitude, deceit, dishonesty or fraud, or any conduct by the Executive that would reasonably be expected to result in material injury or reputational harm to the Company or any of its subsidiaries and affiliates if he were retained in his position; (iv) continued non-performance by the Executive of his duties hereunder (other than by reason of the Executive's physical or mental illness, incapacity or disability) which has continued for more than 30 days following written notice of such non-performance from the CEO; (v) a breach by the Executive of any of the provisions contained in Section 7 of this Agreement; (vi) a material violation by the Executive of the Company's written employment policies; or (vii) failure to cooperate with a bona fide internal investigation or an investigation by regulatory or law enforcement authorities, after being instructed by the Company to cooperate, or the willful destruction or failure to preserve documents or other materials known to be relevant to such investigation or the inducement of others to fail to cooperate or to produce documents or other materials in connection with such investigation.

(d) Termination Without Cause. The Company may terminate the Executive's employment hereunder at any time without Cause. Any termination by the Company of the Executive's employment under this Agreement which does not constitute a termination for Cause under Section 3(c) and does not result from the death or disability of the Executive under Section 3(a) or (b) shall be deemed a termination without Cause.

(e) Termination by the Executive. The Executive may terminate his employment hereunder at any time for any reason, including but not limited to Good Reason. For purposes of this Agreement, "Good Reason" shall mean that the Executive has complied with the "Good Reason Process" (hereinafter defined) following the occurrence of any of the following events: (i) a material diminution in the Executive's responsibilities, authority or duties; (ii) a material diminution in the Executive's Base Salary except for across-the-board salary reductions based on the Company's financial performance similarly affecting all or substantially all senior management employees of the Company; (iii) a material change in the geographic location at which the Executive provides services to the Company; or (iv) the material breach of this Agreement by the Company. "Good Reason Process" shall mean that (i) the Executive reasonably determines in good faith that a "Good Reason" condition has occurred; (ii) the Executive notifies the Company in writing of the first occurrence of the Good Reason condition within 60 days of the first occurrence of such condition; (iii) the Executive cooperates in good faith with the Company's efforts, for a period not less than 30 days following such notice (the "Cure Period"), to remedy the condition; (iv) notwithstanding such efforts, the Good Reason condition continues to exist; and (v) the Executive terminates his employment within 60 days after the end of the Cure Period. If the Company cures the Good Reason condition during the Cure Period, Good Reason shall be deemed not to have occurred.

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(f) Notice of Termination. Except for termination as specified in Section 3(a), any termination of the Executive's employment by the Company or any such termination by the Executive shall be communicated by written Notice of Termination to the other party hereto. For purposes of this Agreement, a "Notice of Termination" shall mean a notice which shall indicate the specific termination provision in this Agreement relied upon.

(g) Date of Termination. "Date of Termination" shall mean: (i) if the Executive's employment is terminated by his death, the date of his death; (ii) if the Executive's employment is terminated on account of disability under Section 3(b) or by the Company for Cause under Section 3(c), the date on which Notice of Termination is given; (iii) if the Executive's employment is terminated by the Company under Section 3(d), the date on which a Notice of Termination is given; (iv) if the Executive's employment is terminated by the Executive under Section 3(e) without Good Reason, 30 days after the date on which a Notice of Termination is given, and (v) if the Executive's employment is terminated by the Executive under Section 3(e) with Good Reason, the date on which a Notice of Termination is given after the end of the Cure Period. Notwithstanding the foregoing, in the event that the Executive gives a Notice of Termination to the Company, the Company may unilaterally accelerate the Date of Termination and such acceleration shall not result in a termination by the Company for purposes of this Agreement.

#### 4. Compensation Upon Termination.

(a) Termination Generally. If the Executive's employment with the Company is terminated for any reason, the Company shall pay or provide to the Executive (or to his authorized representative or estate) (i) any Base Salary earned through the Date of Termination, unpaid expense reimbursements (subject to, and in accordance with, Section 2(d) of this Agreement) and unused vacation that accrued through the Date of Termination on or before the time required by law but in no event more than 30 days after the Executive's Date of Termination; and (ii) any vested benefits the Executive may have under any employee benefit plan of the Company through the Date of Termination, which vested benefits shall be paid and/or provided in accordance with the terms of such employee benefit plans (collectively, the "Accrued Benefit").

(b) Termination by the Company Without Cause or by the Executive with Good Reason. During the Term, if the Executive's employment is terminated by the Company without Cause as provided in Section 3(d), or the Executive terminates his employment for Good Reason as provided in Section 3(e), then the Company shall pay the Executive his Accrued Benefit. In addition, subject to the Executive signing a separation agreement containing, among other provisions, a general release of claims in favor of the Company and related persons and entities, confidentiality, return of property and non-disparagement, in a form and manner satisfactory to the Company (the "Separation Agreement and Release") and the Separation Agreement and Release becoming irrevocable, all within 60 days after the Date of Termination, the Company shall make the following payments to the Executive ("Severance Pay"):

(i) the Company shall continue the Executive's Base Salary for 12 months following the Date of Termination ("Severance Pay Period"). In addition, if the Board determines that the Executive is eligible for a Target Bonus for the year during which his employment terminates, the Executive shall be paid his Target Bonus for the year of termination, prorated for the portion of the year during which the Executive was employed; and

(ii) if the Executive was participating in the Company's group health plan immediately prior to the Date of Termination and elects COBRA health continuation, then the Company shall continue to contribute its regular share of the premium for the Executive's group health coverage, during the Severance Pay Period, provided that the Executive continues to be eligible for such COBRA continuation coverage; and

(iii) the amounts payable under this Section 4(b) shall be paid out in substantially equal installments in accordance with the Company's payroll practice over 12 months commencing within 60 days after the Date of Termination; provided, however, that if the 60-day period begins in one calendar year and ends in a second calendar year, the Severance Amount shall begin to be paid in the second calendar year by the last day of such 60-day period; provided, further, that the initial payment shall include a catch-up payment to cover amounts retroactive to the day immediately following the Date of Termination. Each payment pursuant to this Agreement is intended to constitute a separate payment for purposes of Treasury Regulation Section 1.409A-2(b)(2).

5. Change in Control Payments. The provisions of this Section 5 set forth certain terms of an agreement reached between the Executive and the Company regarding the Executive's rights and obligations upon the occurrence of a Change in Control of the Company. These provisions are intended to assure and encourage in advance the Executive's continued attention and dedication to his assigned duties and his objectivity during the pendency and after the occurrence of any such event. These provisions shall apply in lieu of, and expressly supersede, the provisions of Section 4(b) regarding severance pay and benefits upon a termination of employment, if such termination of employment occurs within 18 months after the occurrence of the first

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event constituting a Change in Control. These provisions shall terminate and be of no further force or effect beginning 18 months after the occurrence of a Change in Control.

(a) Change in Control. During the Term, if within 18 months after a Change in Control, the Executive's employment is terminated by the Company without Cause as provided in Section 3(d) or the Executive terminates his employment for Good Reason as provided in Section 3(e), then, subject to the signing of the Separation Agreement and Release by the Executive and the Separation Agreement and Release becoming irrevocable, all within 60 days after the Date of Termination,

(i) the Company shall continue the Executive's current Base Salary (or the Executive's Base Salary in effect immediately prior to the Change in Control, if higher, for 12 months following the Date of Termination and pay Executive his Target Bonus for the year in which the Date of Termination occurs, pro-rated based on the number of days Executive is employed by the Company in the year of the Date of Termination); and

(ii) notwithstanding anything to the contrary in any applicable option agreement or stock-based award agreement, all stock options and other stock-based awards held by the Executive shall immediately accelerate and become fully exercisable or nonforfeitable as of the Date of Termination; and

(iii) if the Executive was participating in the Company's group health plan immediately prior to the Date of Termination and elects COBRA health continuation, then the Company shall pay to the Executive a monthly cash payment for 12 months or the Executive's COBRA health continuation period, whichever ends earlier, in an amount equal to the monthly employer contribution that the Company would have made to provide health insurance to the Executive if the Executive had remained employed by the Company; and

(iv) The amounts payable under this Section 5(a) shall be paid or commence to be paid within 60 days after the Date of Termination; provided, however, that if the 60-day period begins in one calendar year and ends in a second calendar year, such payment shall be paid or commence to be paid in the second calendar year by the last day of such 60-day period.

(b) Modified Economic Cutback Following a Sale Event. Notwithstanding any other provision in this Agreement, if it is determined that any payments made to the Executive pursuant to this Agreement in relation to a Change in Control, including without limitation a payment made pursuant to Section 5(a) of this Agreement (the "Change in Control Payments"), are or will be subject to excise tax under Section 4999 of the Code and the deduction of the excise tax payable will result in less net income to the Executive after regular tax withholdings are deducted than the net income the Executive would have received if the Change in Control Payments were the highest amount that could be paid to the Executive without incurring such excise tax, then, at the sole discretion of the Executive, the Change in Control Payments made pursuant to this Agreement may be reduced to the amount that would not cause any excise taxes to be payable by Executive.

(c) Compliance with Post-Employment Obligations. Notwithstanding anything in this Agreement to the contrary, the Executive's eligibility for the Severance Pay pursuant to Section 4(b) and the Change in Control Payments pursuant to Section 5 shall cease if the Executive engages in any conduct in violation of Section 7(a) of this Agreement.

(d) Definitions. For purposes of this Section 5, the following terms shall have the following meanings:

"Change in Control" shall mean any of the following:

(i) any "person," as such term is used in Sections 13(d) and 14(d) of the Securities Exchange Act of 1934, as amended (the "Act") (other than the Company, any of its subsidiaries, or any trustee, fiduciary or other person or entity holding securities under any employee benefit plan or trust of the Company or any of its subsidiaries), together with all "affiliates" and "associates" (as such terms are defined in Rule 12b-2 under the Act) of such person, shall become the "beneficial owner" (as such term is defined in Rule 13d-3 under the Act), directly or indirectly, of securities of the Company representing 50 percent or more of the combined voting power of the Company's then outstanding securities having the right to vote in an election of the Board ("Voting Securities") (in such case other than as a result of an acquisition of securities directly from the Company); or

(ii) the date a majority of the members of the Board is replaced during any 12-month period by directors whose appointment or election is not endorsed by a majority of the members of the Board before the date of the appointment or election; or

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(iii) the consummation of (A) any consolidation or merger of the Company where the stockholders of the Company, immediately prior to the consolidation or merger, would not, immediately after the consolidation or merger, beneficially own (as such term is defined in Rule 13d-3 under the Act), directly or indirectly, shares representing in the aggregate more than 50 percent of the voting shares of the Company issuing cash or securities in the consolidation or merger (or of its ultimate parent corporation, if any), or (B) any sale or other transfer (in one transaction or a series of transactions contemplated or arranged by any party as a single plan) of all or substantially all of the assets of the Company.

Notwithstanding the foregoing, a "Change in Control" shall not be deemed to have occurred for purposes of the foregoing clause (i) solely as the result of an acquisition of securities by the Company which, by reducing the number of shares of Voting Securities outstanding, increases the proportionate number of Voting Securities beneficially owned by any person to 50 percent or more of the combined voting power of all of the then outstanding Voting Securities; provided, however, that if any person referred to in this sentence shall thereafter become the beneficial owner of any additional shares of Voting Securities (other than pursuant to a stock split, stock dividend, or similar transaction or as a result of an acquisition of securities directly from the Company) and immediately thereafter beneficially owns 50 percent or more of the combined voting power of all of the then outstanding Voting Securities, then a "Change in Control" shall be deemed to have occurred for purposes of the foregoing clause (i).

6. Section 409A.

(a) Anything in this Agreement to the contrary notwithstanding, if at the time of the Executive's separation from service within the meaning of Section 409A of the Code, the Company determines that the Executive is a "specified employee" within the meaning of Section 409A(a)(2)(B)(i) of the Code, then to the extent any payment or benefit that the Executive becomes entitled to under this Agreement on account of the Executive's separation from service would be considered deferred compensation otherwise subject to the 20 percent additional tax imposed pursuant to Section 409A(a) of the Code as a result of the application of Section 409A(a)(2)(B)(i) of the Code, such payment shall not be payable and such benefit shall not be provided until the date that is the earlier of (A) six months and one day after the Executive's separation from service, or (B) the Executive's death. If any such delayed cash payment is otherwise payable on an installment basis, the first payment shall include a catch-up payment covering amounts that would otherwise have been paid during the six-month period but for the application of this provision, and the balance of the installments shall be payable in accordance with their original schedule.

(b) All in-kind benefits provided and expenses eligible for reimbursement under this Agreement shall be provided by the Company or incurred by the Executive during the time periods set forth in this Agreement. All reimbursements shall be paid as soon as administratively practicable, but in no event shall any reimbursement be paid after the last day of the taxable year following the taxable year in which the expense was incurred. The amount of in-kind benefits provided or reimbursable expenses incurred in one taxable year shall not affect the in-kind benefits to be provided or the expenses eligible for reimbursement in any other taxable year (except for any lifetime or other aggregate limitation applicable to medical expenses). Such right to reimbursement or in-kind benefits is not subject to liquidation or exchange for another benefit.

(c) To the extent that any payment or benefit described in this Agreement constitutes "non-qualified deferred compensation" under Section 409A of the Code, and to the extent that such payment or benefit is payable upon the Executive's termination of employment, then such payments or benefits shall be payable only upon the Executive's "separation from service." The determination of whether and when a separation from service has occurred shall be made in accordance with the presumptions set forth in Treasury Regulation Section 1.409A-1(h).

(d) The parties intend that this Agreement will be administered in accordance with Section 409A of the Code. To the extent that any provision of this Agreement is ambiguous as to its compliance with Section 409A of the Code, the provision shall be read in such a manner so that all payments hereunder comply with Section 409A of the Code. Each payment pursuant to this Agreement is intended to constitute a separate payment for purposes of Treasury Regulation Section 1.409A-2(b)(2). The parties agree that this Agreement may be amended, as reasonably requested by either party, and as may be necessary to fully comply with Section 409A of the Code and all related rules and regulations in order to preserve the payments and benefits provided hereunder without additional cost to either party.

(e) The Company makes no representation or warranty and shall have no liability to the Executive or any other person if any provisions of this Agreement are determined to constitute deferred compensation subject to Section 409A of the Code but do not satisfy an exemption from, or the conditions of, such Section.

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7. Miscellaneous Provisions.

(a) Confidentiality, Assignment of Intellectual Property and Non-Competition Agreement. The Executive acknowledges and agrees that his employment with the Company is subject to his execution of, and compliance with, a Confidentiality, Assignment of Intellectual Property and Non-Competition Agreement, which the Executive shall execute contemporaneously with this Agreement.

(b) Third-Party Agreements and Rights. The Executive hereby confirms that the Executive is not bound by the terms of any agreement with any previous employer or other party which restricts in any way the Executive's use or disclosure of information or the Executive's engagement in any business. The Executive represents to the Company that the Executive's execution of this Agreement, the Executive's employment with the Company and the performance of the Executive's proposed duties for the Company will not violate any obligations the Executive may have to any such previous employer or other party. In the Executive's work for the Company, the Executive will not disclose or make use of any information in violation of any agreements with or rights of any such previous employer or other party, and the Executive will not bring to the premises of the Company any copies or other tangible embodiments of non-public information belonging to or obtained from any such previous employment or other party.

(c) Litigation and Regulatory Cooperation. During and after the Executive's employment, the Executive shall cooperate fully with the Company in the defense or prosecution of any claims or actions now in existence or which may be brought in the future against or on behalf of the Company which relate to events or occurrences that transpired while the Executive was employed by the Company. The Executive's full cooperation in connection with such claims or actions shall include, but not be limited to, being available to meet with counsel to prepare for discovery or trial and to act as a witness on behalf of the Company at mutually convenient times. During and after the Executive's employment, the Executive also shall cooperate fully with the Company in connection with any investigation or review of any federal, state or local regulatory authority as any such investigation or review relates to events or occurrences that transpired while the Executive was employed by the Company. The Company shall reimburse the Executive for any reasonable out-of-pocket expenses incurred in connection with the Executive's performance of obligations pursuant to this Section 7(f).

(d) Taxes. The Company may withhold from any payments made under this Agreement all applicable taxes, including but not limited to income, employment, and social insurance taxes, as shall be required by law in any applicable jurisdiction. Executive acknowledges and represents that the Company has not provided any tax advice to him in connection with this Agreement and that Executive has been advised by the Company to seek tax advice from Executive's own tax advisors regarding this Agreement and payments that may be made to him or her pursuant to this Agreement, including specifically, the application of the provisions of Section 409A of the Code to such payments. The Company shall have no liability to Executive or to any other person if any of the provisions of this Agreement are determined to constitute deferred compensation subject to Section 409A but that do not satisfy an exemption from, or the conditions of, that section.

8. Arbitration of Disputes. Any controversy or claim arising out of or relating to this Agreement or the breach thereof or otherwise arising out of the Executive's employment or the termination of that employment (including, without limitation, any claims of unlawful employment discrimination whether based on age or otherwise) shall, to the fullest extent permitted by law, be settled by arbitration in any forum and form agreed upon by the parties or, in the absence of such an agreement, under the auspices of the American Arbitration Association ("AAA") in Boston, Massachusetts in accordance with the Employment Dispute Resolution Rules of the AAA, including, but not limited to, the rules and procedures applicable to the selection of arbitrators. In the event that any person or entity other than the Executive or the Company may be a party with regard to any such controversy or claim, such controversy or claim shall be submitted to arbitration subject to such other person or entity's agreement. Judgment upon the award rendered by the arbitrator may be entered in any court having jurisdiction thereof. This Section 8 shall be specifically enforceable. Notwithstanding the foregoing, this Section 8 shall not preclude either party from pursuing a court action for the sole purpose of obtaining a temporary restraining order or a preliminary injunction in circumstances in which such relief is appropriate; provided that any other relief shall be pursued through an arbitration proceeding pursuant to this Section 8.

9. Working in Canada.

(a) Right to Work in Canada. Executive shall cooperate with the Company to seek, obtain, and maintain the right to work in Canada to provide services on behalf of the Company to Novelion and its Canadian Affiliates. The Company shall pay the reasonable costs associated with Executive obtaining a permit to work in Canada.

(b) Travel to Canada. Executive acknowledges that travel will be required in connection with his employment, including travel on a regular basis to such locations in Canada that is required or desirable for the Company to provide

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its management services to Novelion and its Canadian Affiliates, including making visits to Canada as are necessary to make decisions related to the Company's business and to manage the Company's business, including attending at Board meetings.

(c) Canadian Employment Standards. This provision applies only if and to the extent that the employment laws of Canada apply to Executive's employment. If the minimum standards in the British Columbia *Employment Standards Act* or Ontario *Employment Standards Act, 2000*, or any other applicable employment standards legislation, as they exist from time to time, are more favorable to Executive in any respect than provided for in this Agreement, including but not limited to the provisions in respect of termination, the provisions of the applicable Employment Standards Act or legislation shall apply.

(d) Tax Equalization.

(i) As Executive will be subject to income tax and social security obligations arising from his services performed in Canada on behalf of the Company, the Company is prepared to address the overall tax and social security burden that Executive experiences with the intention that Executive's total tax and social security burden while working in both the United States and Canada will be equal to what his tax and social security burden would have been had he remained working solely in Massachusetts. The Company will provide Executive with tax equalization in connection with all income tax and social security liabilities arising from the performance of his employment duties within Canada. The Company intends that the income taxes and social security levies payable by Executive on all taxable employment income and related benefits, as prescribed by the applicable tax and social security laws, should be no better or worse than the personal taxes and social security levies Executive would have been required to pay on such amounts if his employment duties had been performed solely in the state of Massachusetts. Where Executive's annual tax and social security obligation yields a higher total obligation than if his employment duties were solely performed in the state of Massachusetts, the Company will reimburse him or her for the difference. Where Executive's annual tax and social security obligations yields a lower total tax and social security impact than if his employment duties were solely performed in the state of Massachusetts, Executive will reimburse the Company for the difference.

(ii) Executive shall provide all information necessary for the preparation of a tax equalization calculation.

(iii) The Company shall pay all reasonable costs and professional fees related to calculating this equalization payment, and reserves the discretion to establish the process and criteria for determining the tax equalization calculation. For clarity, the tax equalization payments described in this Section 9(d) will not take into consideration or apply to any taxable income from sources other than Executive's employment with the Company, and Executive will remain responsible for all income taxes arising from his personal income.

(iv) If Executive establishes his primary residence in Canada, the Company's obligations under this Section 9(d) shall cease, provided that there shall be a pro-rated adjustment for any partial year.

(v) If Executive's employment is terminated for any of the reasons described under Section 4 hereof, then between January 1 and July 31 of the calendar year following the calendar year in which such termination occurs, the Company shall pay Executive any remaining tax equalization payments owed in accordance with this Section 9(d) or, in the event that the reconciliation results in Executive owing money to the Company, Executive shall make such payment to the Company.

10. Consent to Jurisdiction. To the extent that any court action is permitted consistent with or to enforce Section 8 of this Agreement, the parties hereby consent to the jurisdiction of the Superior Court of the Commonwealth of Massachusetts and the United States District Court for the District of Massachusetts. Accordingly, with respect to any such court action, the Executive (a) submits to the personal jurisdiction of such courts; (b) consents to service of process; and (c) waives any other requirement (whether imposed by statute, rule of court, or otherwise) with respect to personal jurisdiction or service of process.

11. Integration. This Agreement constitutes the entire agreement between the parties with respect to the subject matter hereof and supersedes all prior agreements between the parties concerning such subject matter.

12. Withholding. All payments made by the Company to the Executive under this Agreement shall be net of any tax or other amounts required to be withheld by the Company under applicable law.

13. Successor to the Executive. This Agreement shall inure to the benefit of and be enforceable by the Executive's personal representatives, executors, administrators, heirs, distributees, devisees and legatees. In the event of the Executive's death after his termination of employment but prior to the completion by the Company of all payments due him under this Agreement, the Company shall continue such payments to the Executive's beneficiary designated in writing to the Company prior to his death (or to his estate, if the Executive fails to make such designation).

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14. Enforceability. If any portion or provision of this Agreement (including, without limitation, any portion or provision of any section of this Agreement) shall to any extent be declared illegal or unenforceable by a court of competent jurisdiction, then the remainder of this Agreement, or the application of such portion or provision in circumstances other than those as to which it is so declared illegal or unenforceable, shall not be affected thereby, and each portion and provision of this Agreement shall be valid and enforceable to the fullest extent permitted by law.

15. Survival. The provisions of this Agreement shall survive the termination of this Agreement and/or the termination of the Executive's employment to the extent necessary to effectuate the terms contained herein.

16. Waiver. No waiver of any provision hereof shall be effective unless made in writing and signed by the waiving party. The failure of any party to require the performance of any term or obligation of this Agreement, or the waiver by any party of any breach of this Agreement, shall not prevent any subsequent enforcement of such term or obligation or be deemed a waiver of any subsequent breach.

17. Notices. Any notices, requests, demands and other communications provided for by this Agreement shall be sufficient if in writing and delivered in person or sent by a nationally recognized overnight courier service or by registered or certified mail, postage prepaid, return receipt requested, to the Executive at the last address the Executive has filed in writing with the Company or, in the case of the Company, at its main offices, attention of the Board.

18. Amendment. This Agreement may be amended or modified only by a written instrument signed by the Executive and by a duly authorized representative of the Company.

19. Governing Law. This is a Massachusetts contract and shall be construed under and be governed in all respects by the laws of the Commonwealth of Massachusetts, without giving effect to the conflict of laws principles of such Commonwealth. With respect to any disputes concerning federal law, such disputes shall be determined in accordance with the law as it would be interpreted and applied by the United States Court of Appeals for the First Circuit.

20. Counterparts. This Agreement may be executed in any number of counterparts, each of which when so executed and delivered shall be taken to be an original; but such counterparts shall together constitute one and the same document.

21. Successor to Company. The Company shall require any successor (whether direct or indirect, by purchase, merger, consolidation or otherwise) to all or substantially all of the business or assets of the Company expressly to assume and agree to perform this Agreement to the same extent that the Company would be required to perform it if no succession had taken place. Failure of the Company to obtain an assumption of this Agreement at or prior to the effectiveness of any succession shall be a material breach of this Agreement.

22. Gender Neutral. Wherever used herein, a pronoun in the masculine gender shall be considered as including the feminine gender unless the context clearly indicates otherwise.

IN WITNESS WHEREOF, the parties have executed this Agreement effective on the date and year first above written ("Effective Date").

**NOVELION SERVICES USA, INC.**

By: /s/ Linda Buono  
Its: *Senior Vice President, Human Resources*

**JEFFREY HACKMAN**

/s/ Jeffrey Hackman

November 8, 2017

**PERSONAL AND CONFIDENTIAL**

Mary Szela  
6 Bannockburn Ct  
Bannockburn, IL 60015

**Re: Resignation Agreement**

Dear Mary:

This letter confirms your separation from employment with Novelion Services, USA, Inc. (the “Company”) and proposes an agreement (the “Agreement”) between you and the Company. The purpose of this Agreement is to establish an amicable arrangement for ending your employment relationship, including releasing the Company and related persons or entities from any claims and permitting you to receive separation pay and related benefits.

You acknowledge that you are entering into this Agreement knowingly and voluntarily. By proposing and entering into this Agreement, the Company is not admitting in any way that it violated any legal obligation that it owed to you.

With those understandings, you and the Company agree as follows:

1. **Resignation from Employment**

This confirms that you have submitted your written resignation from employment with the Company effective on November 7, 2017 (the “Resignation Date”). You further confirm that you have submitted your written resignation from any and all other positions that you hold with the Company as an officer, director or otherwise, or with any affiliate or subsidiary of the Company, effective on the Resignation Date. You acknowledge that as of the Company’s most recent payroll payment of salary to you, you were fully paid for all salary then due to you. The Company agrees to pay you your salary, and all accrued but unused vacation through the Resignation Date. The Company shall reimburse you for any business expenses incurred through the Resignation Date, in accordance with the Company’s expense reimbursement policy. You also agreed to remove your personal furniture from the condominium provided to you by the Company by November 20, 2017, and will leave the keys to such condominium with the building concierge no later than that date.

2. **Severance Benefits**

(a) **Severance Pay**. The Company shall pay you severance pay (“Severance Pay”) consisting of salary continuation at your final base salary rate of \$689,550 per year effective for the period from the date immediately following the Resignation Date to and including November 6, 2018 (the “Severance Pay Period”). The Company shall pay you Severance Pay on its regular payroll dates; *provided* that the Company shall not be obligated to include you on the payroll before this Agreement becomes effective. If the Company does not make one or more payments of Severance Pay on a regular payroll date because this Agreement has not yet become effective, the Company shall make all such delayed payments by the first payroll date when it is practicable to do so after the Agreement becomes effective. In addition, on the final date on which Severance Pay is paid at the conclusion of the Severance Pay Period, the Company shall also pay you, in a lump sum, the gross amount of \$413,000.

(b) **Tax Treatment**. The Company shall make deductions, withholdings and tax reports with respect to payments and benefits under this Agreement that it reasonably determines to be required. Payments under this Agreement shall be in amounts net of any such deductions or withholdings. Nothing in this Agreement shall be construed to require the Company to make any payments to compensate you for any adverse tax effect associated with any payments or benefits or for any deduction or withholding from any payment or benefit.

3. **Equity**

You and the Company acknowledge that 6,632 restricted stock units (RSU) from a grant dated May 9, 2016 vested effective May 9, 2017. You and the Company also acknowledge that none of the options or other forms of equity that you hold to

purchase, or that entitle you to receive, shares of the Company's common stock pursuant to the Novelion 2016 Equity Incentive Plan or any predecessor plan are vested as of the Resignation Date and shall lapse on that date and will not be exercisable. You agree that other than the RSUs discussed above in this Section, you have no other rights to stock awards, performance awards, or stock options of the Company.

#### 4. **Continuing Obligations**

You acknowledge that your obligations under the Employee Confidentiality, Assignment of Intellectual Property and Non-Competition Agreement ("Confidentiality and Non-Competition Agreement") shall continue in effect, including without limitation your obligations to maintain the confidentiality of Proprietary Information as defined in the Confidentiality and Non-Competition Agreement, to return documents and other property of the Company and to not compete or solicit unfairly. A copy of the Confidentiality and Non-Competition Agreement is enclosed as Exhibit A. You also agree not to communicate with any of the Company's debt or equity holders regarding the Company including, without limitation, its business, products, services or financial status, your employment or termination from employment, or the status of the Company's debt or equity, unless such communication is in connection with future litigation to which you are a party.

#### 5. **Return of Property**

You confirm that, to the best of your knowledge, within 48 hours following your execution of this Agreement, you will have returned to the Company all Company property, including, without limitation, computer equipment, software, keys and access cards, credit cards, files and any documents (including computerized data and any copies made of any computerized data or software) containing information concerning the Company, its business or its business relationships. In the event that you discover that you continue to retain any such property, you shall return it to the Company promptly. You also commit to deleting and finally purging any duplicates of files or documents that may contain Company information from any computer or other device that remains your property after the Resignation Date. In the event that you discover that you continue to retain any such duplicates of files or documents, you shall delete them promptly. Notwithstanding the above, you may retain your Company Mac laptop until you remove all of your personal data residing on such laptop and on the Company's cloud (under your Apple I.D.), according to the process agreed to by counsel for each of the parties to this Agreement. You shall accomplish this removal of your personal data and return the laptop to the Company no later than November 17, 2017, provided that if you need additional time to accomplish this, you will notify Company counsel and a reasonable extension will be provided.

#### 6. **Mutual Release of Claims**

(a) In consideration for, among other terms, the Severance Pay, to which you acknowledge you would otherwise not be entitled, you voluntarily release and forever discharge the Company, together with Novelion Therapeutics, Inc., its and their subsidiaries, and affiliated and related entities, its and their respective predecessors, successors and assigns (collectively, the "Affiliates"), its and their respective employee benefit plans, equity incentive plans, and fiduciaries of such plans, and the current and former officers, directors, shareholders, employees, attorneys, accountants and agents of each of the foregoing in their official and personal capacities (collectively referred to as the "Company Releasees") generally from all claims, demands, debts, damages and liabilities of every name and nature, known or unknown ("Claims") that, as of the date when you sign this Agreement, you have, ever had, now claim to have or ever claimed to have had against any or all of the Company Releasees relating to or arising from your employment with the Company or termination or your employment with the Company. This release includes, without limitation, all Claims:

- relating to your employment by and termination of employment with the Company;
  - arising from your Employment Agreement dated May 8, 2017;
  - of wrongful discharge or violation of public policy;
  - of breach of contract;
  - of defamation or other torts;
  - of retaliation or discrimination under federal, state or local law (including, without limitation, Claims of discrimination or retaliation under the Employee Retirement Income Security Act, the Age Discrimination in Employment Act, the Americans with Disabilities Act, and Title VII of the Civil Rights Act of 1964);
  - under any other federal or state statute (including, without limitation, Claims under the Fair Labor Standards Act);
  - under the Ontario Employment Standards Act, 2000 and the British Columbia Employment Standards Act, the Ontario Human Rights Code and the British Columbia Human Rights Code, and any other applicable Canadian or provincial law, regulation or other requirement (each as amended from time to time);
  - for wages, bonuses, incentive compensation, commissions, stock, stock options, vacation pay or any other compensation or benefits, either under the Massachusetts Wage Act, M.G.L. c. 149, §§148-150C, or otherwise; and
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- for damages or other remedies of any sort, including, without limitation, compensatory damages, punitive damages, injunctive relief and attorney's fees;

*provided*, however, that this release shall not affect (a) Claims that arise after the Effective Date of this Agreement, (b) your rights under this Agreement, (c) your vested rights under the Company's Section 401(k) plan, (d) your rights to the 6,632 RSUs discussed above in Section 3, (e) your rights to continued health insurance coverage pursuant to COBRA, (f) your rights to indemnification arising under the Company's or any Affiliate's bylaws, contracts/agreements or otherwise by law, (g) your right to coverage under the Company's or any Affiliate's D&O insurance policy; (h) your right to contribution in the event that a judgment is entered against you for which you and the Company or any of the other Company Releasees are found to be jointly liable, or (i) Claims that cannot be lawfully waived or released.

You agree not to accept damages of any nature, other equitable or legal remedies for your own benefit or attorney's fees or costs from any of the Company Releasees with respect to any Claim released by this Agreement. As a material inducement to the Company to enter into this Agreement, you represent that you have not assigned any Claim to any third party.

(b) In consideration for your execution of this Agreement, the Company, Novelion Therapeutics, Inc. and Aegerion Pharmaceuticals, Inc., ("Company Releasing Parties") voluntarily release and forever discharge you, your beneficiaries, heirs, estate, attorneys, accountants, and agents in their official and personal capacities (collectively referred to as "Your Releasees") generally from all Claims that, as of the date when the Company Releasing Parties sign this Agreement, the Company Releasing Parties have, ever had, now claim to have or ever claimed to have had against you or any of Your Releasees relating to or arising from your employment with the Company or termination or your employment with the Company, *provided* that the Company Releasing Parties do not release you from any civil Claim that is based on conduct that also satisfies the elements of a criminal offense ("Excepted Claim"). Neither the undersigned Company Releasing Parties' representatives, nor the other members of the Company's Board ("Board"), nor the Company's current Chief Operating Officer, Chief Financial Officer, General Counsel, Senior Vice-President, or Executive Vice President of R&D have knowledge or reason to believe that the Company has any Excepted Claim against you.

#### **7. Confidentiality of Agreement-Related Information**

You agree, to the fullest extent permitted by law, to keep all Agreement-Related Information completely confidential. "Agreement-Related Information" means any allegations of wrongful conduct by the Company or any of its representatives, the negotiations leading to this Agreement and the existence and terms of this Agreement. Notwithstanding the foregoing, you may disclose Agreement-Related Information to your spouse, your attorney and your financial advisors, and to them only provided that they first agree for the benefit of the Company to keep Agreement-Related Information confidential. Nothing in this section shall be construed to prevent you from disclosing Agreement-Related Information to the extent required by a lawfully issued subpoena or duly issued court order; *provided* that you provide the Company with advance written notice and a reasonable opportunity to contest such subpoena or court order.

#### **8. Non-Disparagement**

You agree not to make any disparaging statements concerning the Company, Novelion Therapeutics, Inc., or any of its or their Affiliates, products, services or current or former officers, directors, shareholders, or individuals who you know are employees or agents of the Company, Novelion Therapeutics, Inc., or their Affiliates. You represent that during the period since November 4, 2017, you have not made any such disparaging statements. The Company shall direct the Board and the Company's current Chief Operating Officer, Chief Financial Officer, General Counsel and Senior Vice-President, Executive Vice President of R&D, Human Resources, and all employees whom it informs of the terms of this Agreement not to make disparaging statements about you in any communication that is not a confidential or privileged internal communication. The Company represents that during the period since November 4, 2017, neither members of the Board nor the individuals identified above have made any such disparaging statements about you in any communication that is not a confidential or privileged internal communication.

#### **9. Communications Concerning Your Separation**

If asked about the circumstances of your separation from employment with the Company, you shall state that you resigned for personal reasons and shall not make any further comment about your employment separation. The Company shall direct the Board, and all employees whom it informs of the terms of this Agreement, if asked about the circumstances of your separation, to state that you resigned for personal reasons, and not to make any further comment about your employment separation. The Company shall direct the Board, and the Company's current Chief Operating Officer, Chief Financial Officer, General Counsel and Senior Vice-President, Executive Vice President of R&D, Human Resources, and all employees whom it informs of the

terms of this Agreement to state in any communication that is not a confidential or privileged internal communication only that you have left Novelion for personal reasons and not to make any other statement or pronouncement regarding your separation from employment.

#### 10. **Future Cooperation**

During the Severance Pay Period, you agree to respond to any inquiry from the Board regarding the status of any matter that was within your responsibilities as an employee of the Company. The Board shall endeavor to make any such inquiries in a manner that does not interfere with your personal or professional time/responsibilities, including any efforts to transition to new employment. You also agree to cooperate reasonably with the Company and all of its affiliates (including its and their outside counsel) in connection with (i) the contemplation, prosecution and defense of all phases of existing, past and future litigation about which the Company believes you may have knowledge or information; and (ii) responding to requests for information from regulatory agencies or other governmental authorities (together “Cooperation Services”). You further agree to make yourself reasonably available to provide Cooperation Services at mutually convenient times during and outside of regular business hours as reasonably deemed necessary by the Company’s counsel. The Company shall not utilize this section to require you to make yourself available to an extent that would unreasonably interfere with personal or professional responsibilities that you may have. Cooperation Services include, without limitation, appearing without the necessity of a subpoena to testify truthfully in any legal proceedings in which the Company or an affiliate calls you as a witness. The Company shall reimburse you for any reasonable travel expenses that you incur due to your performance of Cooperation Services, after receipt of appropriate documentation consistent with the Company’s business expense reimbursement policy.

#### 11. **Protected Disclosures and Other Protected Actions**

Nothing contained in this Agreement limits your ability to file a charge or complaint with any federal, state or local governmental agency or commission (a “Government Agency”). In addition, nothing contained in this Agreement limits your ability to communicate with any Government Agency or otherwise participate in any investigation or proceeding that may be conducted by any Government Agency, including your ability to provide documents or other information, without notice to the Company, nor does anything contained in this Agreement apply to truthful testimony in litigation. If you file any charge or complaint with any Government Agency and if the Government Agency pursues any claim on your behalf, or if any other third party pursues any claim on your behalf, you waive any right to monetary or other individualized relief (either individually or as part of any collective or class action); *provided* that nothing in this Agreement limits any right you may have to receive a whistleblower award or bounty for information provided to the Securities and Exchange Commission. In addition, for the avoidance of doubt, pursuant to the federal Defend Trade Secrets Act of 2016, you shall not be held criminally or civilly liable under any federal or state trade secret law or under this Agreement of the Confidentiality and Non-Competition Agreement for the disclosure of a trade secret that (a) is made (i) in confidence to a federal, state, or local government official, either directly or indirectly, or to an attorney; and (ii) solely for the purpose of reporting or investigating a suspected violation of law; or (b) is made in a complaint or other document filed in a lawsuit or other proceeding, if such filing is made under seal.

#### 12. **Termination of Payments**

If you breach any of your obligations under this Agreement, including your continuing obligations under the Confidentiality and Non-Competition Agreement, in addition to any other legal or equitable remedies it may have for such breach, the Company shall have the right to terminate its payments to you or for your benefit under this Agreement, and to escrow such payments in an escrow account controlled by Goodwin Procter, LLP, pending a determination by a court of competent jurisdiction that you have breached any of such obligations under this Agreement. The termination of such payments in the event of your breach will not affect your continuing obligations under this Agreement.

#### 13. **Section 409A**

(a) Anything in this Agreement to the contrary notwithstanding, if at the time of your separation from service within the meaning of Section 409A of the Internal Revenue Code of 1986, as amended (the “Code”), the Company determines that you are a “specified employee” within the meaning of Section 409A(a)(2)(B)(i) of the Code, then to the extent any payment or benefit that you become entitled to under this Agreement on account of your separation from service would be considered deferred compensation subject to the 20 percent additional tax imposed pursuant to Section 409A(a) of the Code as a result of the application of Section 409A(a)(2)(B)(i) of the Code, such payment shall not be payable and such benefit shall not be provided until the date that is the earlier of (A) six months and one day after your separation from service, or (B) your death.

(b) All in-kind benefits provided and expenses eligible for reimbursement under this Agreement shall be provided by the Company or incurred by you during the time periods set forth in this Agreement. All reimbursements shall be paid as soon as

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administratively practicable, but in no event shall any reimbursement be paid after the last day of the taxable year following the taxable year in which the expense was incurred. The amount of in-kind benefits provided or reimbursable expenses incurred in one taxable year shall not affect the in-kind benefits to be provided or the expenses eligible for reimbursement in any other taxable year. Such right to reimbursement or in-kind benefits is not subject to liquidation or exchange for another benefit.

(c) To the extent that any payment or benefit described in this Agreement constitutes “non-qualified deferred compensation” under Section 409A of the Code, and to the extent that such payment or benefit is payable upon your termination of employment, then such payments or benefits shall be payable only upon your “separation from service.” The determination of whether and when a separation from service has occurred shall be made in accordance with the presumptions set forth in Treasury Regulation Section 1.409A-1(h).

(d) The parties intend that this Agreement will be administered in accordance with Section 409A of the Code. To the extent that any provision of this Agreement is ambiguous as to its compliance with Section 409A of the Code, the provision shall be read in such a manner so that all payments hereunder comply with Section 409A of the Code. The parties agree that this Agreement may be amended, as reasonably requested by either party, and as may be necessary to fully comply with Section 409A of the Code and all related rules and regulations in order to preserve the payments and benefits provided hereunder without additional cost to either party.

(e) The Company makes no representation or warranty and shall have no liability to you or any other person if any provisions of this Agreement are determined to constitute deferred compensation subject to Section 409A of the Code but do not satisfy an exemption from, or the conditions of, such Section.

#### 14. **Other Provisions**

(a) **Absence of Reliance.** In signing this Agreement, you are not relying upon any promises or representations made by anyone at or on behalf of the Company.

(b) **Enforceability.** If any portion or provision of this Agreement (including, without limitation, any portion or provision of any section of this Agreement) shall to any extent be declared illegal or unenforceable by a court of competent jurisdiction, then the remainder of this Agreement, or the application of such portion or provision in circumstances other than those as to which it is so declared illegal or unenforceable, shall not be affected thereby, and each portion and provision of this Agreement shall be valid and enforceable to the fullest extent permitted by law.

(c) **Waiver.** No waiver of any provision of this Agreement shall be effective unless made in writing and signed by the waiving party. The failure of a party to require the performance of any term or obligation of this Agreement, or the waiver by a party of any breach of this Agreement, shall not prevent any subsequent enforcement of such term or obligation or be deemed a waiver of any subsequent breach.

(d) **Jurisdiction.** You and the Company hereby agree that the Superior Court of the Commonwealth of Massachusetts and the United States District Court for the District of Massachusetts shall have the exclusive jurisdiction to consider any matters related to this Agreement, including without limitation any claim of a violation of this Agreement. With respect to any such court action, you submit to the jurisdiction of such courts and you acknowledge that venue in such courts is proper.

(e) **Relief.**

(i) You agree that it would be difficult to measure any harm caused to the Company that might result from any breach by you of your promises set forth in Sections 5, 7, 8 and 10 (the “**Specified Sections**”). You further agree that money damages would be an inadequate remedy for any breach of any of the Specified Sections. Accordingly, you agree that if you breach, or propose to breach, any portion of your obligations under any of the Specified Sections, the Company shall be entitled, in addition to all other remedies it may have, to an injunction or other appropriate equitable relief to restrain any such breach, without showing or proving any actual damage to the Company and without the necessity of posting a bond. If the Company prevails in any action to enforce this Agreement, then you also shall be liable to the Company for reasonable attorney’s fees and costs incurred by the Company in enforcing this Agreement.

(ii) The Company agrees that it would be difficult to measure any harm caused to you that might result from any breach by the Company of its promises set forth in Sections 8 and 9 of this Agreement. The Company further agrees that money damages would be an inadequate remedy for any breach of such sections. Accordingly, the Company agrees that if the Company or any other person or entity bound by such sections breaches, or proposes to breach, any portion of their obligations under such sections, you shall be entitled, in addition to all other remedies you may have, to an injunction or other appropriate

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equitable relief to restrain any such breach, without showing or proving any actual damage to you and without the necessity of posting a bond. If you prevail in any action to enforce this Agreement, then the Company also shall be liable to you for reasonable attorney's fees and costs incurred by you in enforcing your rights under this Agreement.

(f) Governing Law; Interpretation. This Agreement shall be interpreted and enforced under the laws of the Commonwealth of Massachusetts, without regard to conflict of law principles. In the event of any dispute, this Agreement is intended by the parties to be construed as a whole, to be interpreted in accordance with its fair meaning, and not to be construed strictly for or against either you or the Company or the "drafter" of all or any portion of this Agreement.

(g) Entire Agreement. This Agreement constitutes the entire agreement between you and the Company. This Agreement supersedes any previous agreements or understandings between you and the Company, except the Confidentiality and Non-Competition Agreement and any other obligations specifically preserved in this Agreement.

(h) Time for Consideration; Effective Date. You acknowledge that you have knowingly and voluntarily entered into this Agreement and that the Company advises you to consult with an attorney before signing this Agreement. You understand and acknowledge that you have been given the opportunity to consider this Agreement for twenty-one (21) days from your receipt of this Agreement before signing it (the "Consideration Period"). To accept this Agreement, you must return a signed original or a signed PDF copy of this Agreement so that it is received by Bradford J. Smith (BSmith@goodwinlaw.com) at or before the expiration of the Consideration Period. If you sign this Agreement before the end of the Consideration Period, you acknowledge that such decision was entirely voluntary and that you had the opportunity to consider this Agreement for the entire Consideration Period. For the period of seven (7) days from the date when you sign this Agreement, you have the right to revoke this Agreement by written notice to Mr. Smith, provided that such notice is delivered so that it is received at or before the expiration of the seven (7) day revocation period. This Agreement shall not become effective or enforceable during the revocation period. This Agreement shall become effective on the first business day following the expiration of the revocation period (the "Effective Date").

(i) Counterparts. This Agreement may be executed in separate counterparts. When both counterparts are signed, they shall be treated together as one and the same document.

(j) Authority. The individual signing this Agreement on behalf of the Company represents and warrants that he is authorized to bind the Company (and the Affiliates, as applicable).

Please indicate your agreement to the terms of this Agreement by signing and returning to Mr. Smith the original or a PDF copy of this letter within the time period set forth above.

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Sincerely,

NOVELION SERVICES USA, INC.

By: /s/ Ben Harshbarger 11/8/2017  
BEN HARSHBARGER                      Date  
Member of the Board of  
Novelion Services USA, Inc.

NOVELION THERAPEUTICS, INC.

By: /s/ Ben Harshbarger 11/8/2017  
BEN HARSHBARGER                      Date  
Member of the Board of  
Novelion Therapeutics, Inc.

AEGERION PHARMACEUTICALS, INC.

By: /s/ Barbara Chan 11/8/2017  
BARBARA CHAN                      Date  
President  
Aegerion Pharmaceuticals, Inc.

Enclosure (Exhibit A) Confidentiality and Non-Competition Agreement

You are advised to consult with an attorney before signing this Agreement. This is a legal document. Your signature will commit you to its terms. By signing below, you acknowledge that you have carefully read and fully understand all of the provisions of this Agreement and that you are knowingly and voluntarily entering into this Agreement.

/s/ Mary Szela 11/8/2017  
MARY SZELA                      Date

## EMPLOYMENT AGREEMENT

This Employment Agreement (“Agreement”) is made as of the 27th day of November, 2017, between Novelion Services USA, Inc., a Delaware corporation (the “Company”), and Michael Price (the “Executive”).

WHEREAS, the Company desires to employ the Executive and the Executive desires to be employed by the Company beginning on November 27, 2017 (the “Commencement Date”) on the terms contained herein.

NOW, THEREFORE, in consideration of the mutual covenants and agreements herein contained and other good and valuable consideration, the receipt and sufficiency of which is hereby acknowledged, the parties agree as follows:

1. Employment.

(a) Term. The Company hereby employs the Executive, and the Executive hereby accepts such employment, commencing as of the Commencement Date and continuing on an at-will basis until terminated by either party in accordance with the provisions of Section 3 (“Term”).

(b) Position, Relocation and Duties. During the Term, the Executive shall serve as the Senior Vice President, Finance, reporting to the interim Office of the Company’s Chief Executive Officer (“Interim CEO Office”), as established by the Board of Directors of the Company (the “Board”), pending the results of ongoing executive transition discussions. The Executive agrees that after the Board appoints a new CEO, and provided that the permanent Chief Executive Officer (“CEO”) endorses the Executive’s ongoing employment with the Company, the Executive shall relocate to the Greater Boston area. Pursuant to the Master Service Agreement between the Company and Novelion Therapeutics, Inc. (“Novelion”) dated November 29, 2016 (the “Service Agreement”), Executive may also be required, on behalf of the Company, to perform services to Novelion and its other Affiliates, including holding an office in Novelion. As of the Effective Date, these services shall include serving as the Vice President, Finance, of Novelion, and such other duties consistent with the Service Agreement as may be assigned and/or prescribed from time to time by the Interim CEO Office, CEO, the Board of Directors of the Company (the “Board”) or its designee, or by the Board of Directors of Novelion (the “Novelion Board”) pursuant to the Service Agreement, provided that such duties are consistent with the Executive’s position or other positions that he may hold from time to time. The Executive will comply with the policies of the Company and Aegerion. For certainty, at all times Executive will be an employee of the Company and not an employee of Novelion, and when Executive provides services to Novelion he will be doing so as an employee of the Company performing contracted management services as provided to Novelion under the Service Agreement. For the purpose of this Agreement, “Affiliate” with reference to the Company and Novelion, shall have the meaning given to it in the Delaware General Corporation Law as of the date of this Agreement and, for certainty includes, without limitation, Novelion and Aegerion Pharmaceuticals, Inc. (“Aegerion”) and any other current or future Affiliates of the Company.

(c) Performance. Executive shall devote his full business time, attention, skill, and best efforts to the performance of his duties under this Agreement and shall not engage in any other business or occupation during the Term, including, without limitation, any activity that (x) conflicts with the interests of the Company or any of its Affiliates, (y) interferes with the proper and efficient performance of Executive’s duties for the Company or any of its Affiliates, or (z) interferes with Executive’s exercise of judgment in the Company’s or any of its Affiliates’ best interests. Notwithstanding the foregoing, nothing herein shall preclude Executive from (i) serving, with the prior written consent of the Novelion Board, as a member of the boards of directors or advisory boards (or their equivalents in the case of a non-corporate entity) of non-competing businesses and charitable organizations, (ii) engaging in charitable activities and community affairs, and (iii) managing Executive’s personal investments and affairs; *provided, however*, that the activities set out in clauses (i), (ii), and (iii) shall be limited by Executive so as not to interfere, individually or in the aggregate, with the performance of Executive’s duties and responsibilities hereunder. Executive represents that he has provided the Company with a comprehensive list of all outside professional activities with which he is currently involved or reasonably expects to become involved. In the event that, during his employment by the Company, the Executive desires to engage in other outside professional activities, not included on such list, Executive will first seek written approval from the Novelion CEO and such approval shall not be unreasonably withheld.

2. Compensation and Related Matters.

(a) Base Salary. During the Term, the Executive’s initial annual base salary shall be \$415,000. The base salary in effect at any given time is referred to herein as “Base Salary.” The Base Salary shall be payable in a manner that

is consistent with the Company's usual payroll practices for senior executives. All payments in this Agreement are on a gross, pre-tax basis and shall be subject to all applicable federal, state and local, and if applicable Canadian federal and provincial, withholding, payroll and other taxes required by law. Executive's Base Salary will be subject to increase by the Board following approval by the Novelion Board of an increase in the Base Salary.

(b) Target Bonus. Beginning January 1, 2018, the Executive shall be eligible to earn an annual target bonus, based upon the level of achievement of the Executive, and Novelion and the Company against his/their corporate goals and milestones, as determined by the Board in its sole discretion ("Target Bonus"). The Target Bonus, if any, shall be paid no later than March 15 of the calendar year following the year in which it is earned. The Executive's Target Bonus shall be 50% percent of his Base Salary. To earn a Target Bonus, the Executive must be employed by the Company on the day such Target Bonus is paid.

(c) Stock Options/Equity Grants. Subject to approval by the Compensation Committee of the Novelion Board, the Company will grant Executive: (i) an option (the "Option Award") to purchase 100,000 shares of the Company's common shares (the "Common Shares"); and (ii) an award of 20,000 restricted stock units (the "RSU Award"). Each of these awards will have a grant date of Executive's Commencement Date, will be subject to vesting and will be issued pursuant to, and subject to, the terms of the Amended and Restated Novelion 2017 Equity Incentive Plan (or a successor plan, if any) and subject to the terms of agreements thereunder (collectively the "Equity Documents"). The Option Award shall have an exercise price equal to the fair market value of the Common Stock on the grant date. The vesting schedule for the Option Award will be the vesting schedule outlined in the Equity Documents for the Option Awards (i.e., the option to purchase 100,000 shares will vest in three (3) equal installments on the first three (3) anniversaries of the grant date). The vesting schedule for the RSU Award will be the vesting schedule outlined in the Equity Documents for the RSU Award (i.e., the RSU Award will vest in three (3) equal installments on the first three (3) anniversaries of the grant date). The full terms and conditions related to the Option Award and the RSU Award shall be set forth in the Equity Documents and to the extent that there is any inconsistency between this Agreement and the Equity Documents, the Equity Documents shall control. Because the Commencement Date will be after October 1, 2017, the Executive will not be eligible to receive equity awards as part of the 2017 performance review cycle.

The Executive will be also entitled to participate in those equity incentive plans and programs provided from time to time to the Executive by Novelion on the terms and conditions for such participation as established and changed from time to time by Novelion in its sole discretion.

The Executive is subject to, and will abide by, Novelion's Insider Trading Policy, as amended by the Novelion Board from time to time, and is required to file insider reports disclosing the grant or exercise of any options and restricted stock units as well as the acquisition and sale of any shares in the Company. The Executive will comply with pre-approval, notification and other internal procedures set forth in Novelion's Insider Trading Policy or as otherwise established by Novelion and communicated to the Executive. The Executive will also abide by the share ownership guidelines of Novelion as may be established and amended by the Novelion Board from time to time.

(d) Annual Tax and Financial Planning Reimbursements. During the Term, for as long as Executive continues to provide management services on behalf of the Company in Canada, Executive will be entitled to annual reimbursement up to a maximum of USD \$5,000 for his reasonable expenses for independent tax consultation regarding the Canadian tax implications of Executive's work on behalf of the Company in Canada and/or preparation of Executive's Canadian tax return.

(e) Forfeiture and Recoupment of Incentive-based Compensation. The Target Bonus and any equity awards that Executive receives in accordance with Section 2(b) and Section 2(c) of this Agreement are subject to Aegerion's Policy on the Executive Financial Recoupment Program, as may be amended by Aegerion from time to time in its sole discretion. The Policy on Executive Financial Recoupment Program provides for forfeiture and recoupment of an amount equivalent to up to three years of incentive-based compensation upon the occurrence of certain triggering events.

(f) Executive Benefits and Vacation. During the Term, Executive shall be eligible to participate in health insurance and other benefits provided generally to similarly situated employees of the Company, subject to the terms and conditions of the applicable benefit plans (which shall govern). Executive also shall be eligible for the same number of holidays and vacation days as well as any other benefits, in each case as are generally allowed to similarly situated employees of the Company in accordance with the Company policy as in effect from time to time. Nothing contained herein shall be construed to limit the Company's ability to amend, suspend, or terminate any employee benefit plan or policy at any time without providing Executive notice, and the right to do so is expressly reserved.

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(g) Reimbursement of Business Expenses. During the Term of Employment, the Company shall pay (or promptly reimburse Executive) for documented, out-of-pocket expenses reasonably incurred by Executive in the course of performing his duties and responsibilities hereunder, which are consistent with the Company's policies in effect from time to time with respect to business expenses, subject to the Company's requirements with respect to reporting of such expenses; provided that, the terms of the Expatriate Assignment Letter shall govern the payment or reimbursement of expenses incurred in connection with any Expatriate Assignment.

(h) Relocation Transition Allowance. The Executive will receive a relocation transition allowance to cover the following expenses: (a) temporary housing, not to exceed \$5,500 per month; (b) weekly commuting costs to include airfare/train fare not to exceed \$1,000 per round trip, and taxi/car services to and from the airport/train station, all of which must comply with the Company's business expense policy (collectively with (a), the "Relocation Transition Allowance"); and (c) a "gross-up" payment in the amount necessary to offset the tax liability associated with the Relocation Transition Allowance outlined in (a) and (b); *provided*, that (x) the Executive must submit expense reports with supporting documentation in such form and containing such information as the Company may request to be reimbursed for all Relocation Transition Allowance expenses, and (y) if, prior to the 12-month anniversary of the Commencement Date, the Executive's employment terminates other than by the Company without cause, the Executive will be required to repay the amounts paid to the Executive under the Relocation Transition Allowance.

3. Termination. During the Term, the Executive's employment hereunder may be terminated at any time by the Company or by the Executive, with or without cause, without any breach of this Agreement, upon thirty (30) days written notice by the terminating party to the other party, provided, however, if the Executive's employment is terminated by the Company for cause, the date of termination shall be the date on which written notice is given. Notwithstanding the foregoing, in the event that a party gives thirty (30) days written notice of termination, the Company may pay the Executive his Base Salary during such notice period and accelerate the date of termination. If the Executive's employment with the Company is terminated for any reason, the Company shall pay or provide to the Executive (or to his authorized representative or estate) (i) any Base Salary earned through the date of termination, unpaid expense reimbursements (subject to, and in accordance with, Section 2(f) of this Agreement) and unused vacation that accrued through the date of termination on or before the time required by law but in no event more than 30 days after the Executive's date of termination; and (ii) any vested benefits the Executive may have under any employee benefit plan of the Company through the date of termination, which vested benefits shall be paid and/or provided in accordance with the terms of such employee benefit plans.

4. Miscellaneous Provisions.

(a) Confidentiality, Assignment of Intellectual Property and Non-Competition Agreement. The Executive acknowledges and agrees that his employment with the Company is subject to his execution of, and compliance with, a Confidentiality, Assignment of Intellectual Property and Non-Competition Agreement, which the Executive shall execute contemporaneously with this Agreement.

(b) Third-Party Agreements and Rights. The Executive hereby confirms that the Executive is not bound by the terms of any agreement with any previous employer or other party which restricts in any way the Executive's use or disclosure of information or the Executive's engagement in any business. The Executive represents to the Company that the Executive's execution of this Agreement, the Executive's employment with the Company and the performance of the Executive's proposed duties for the Company will not violate any obligations the Executive may have to any such previous employer or other party. In the Executive's work for the Company, the Executive will not disclose or make use of any information in violation of any agreements with or rights of any such previous employer or other party, and the Executive will not bring to the premises of the Company any copies or other tangible embodiments of non-public information belonging to or obtained from any such previous employment or other party.

(c) Litigation and Regulatory Cooperation. During and after the Executive's employment, the Executive shall cooperate fully with the Company in the defense or prosecution of any claims or actions now in existence or which may be brought in the future against or on behalf of the Company which relate to events or occurrences that transpired while the Executive was employed by the Company. The Executive's full cooperation in connection with such claims or actions shall include, but not be limited to, being available to meet with counsel to prepare for discovery or trial and to act as a witness on behalf of the Company at mutually convenient times. During and after the Executive's employment, the Executive also shall cooperate fully with the Company in connection with any investigation or review of any federal, state or local regulatory authority as any such investigation or review relates to events or occurrences that transpired while the Executive was employed by the Company. The Company shall reimburse the Executive for any reasonable out-of-pocket expenses incurred in connection with the Executive's performance of obligations pursuant to this Section 4(c).

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(d) Taxes. The Company may withhold from any payments made under this Agreement all applicable taxes, including but not limited to income, employment, and social insurance taxes, as shall be required by law in any applicable jurisdiction. Executive acknowledges and represents that the Company has not provided any tax advice to him in connection with this Agreement and that Executive has been advised by the Company to seek tax advice from Executive's own tax advisors regarding this Agreement and payments that may be made to him or her pursuant to this Agreement.

5. Arbitration of Disputes. Any controversy or claim arising out of or relating to this Agreement or the breach thereof or otherwise arising out of the Executive's employment or the termination of that employment (including, without limitation, any claims of unlawful employment discrimination whether based on age or otherwise) shall, to the fullest extent permitted by law, be settled by arbitration in any forum and form agreed upon by the parties or, in the absence of such an agreement, under the auspices of the American Arbitration Association ("AAA") in Boston, Massachusetts in accordance with the Employment Dispute Resolution Rules of the AAA, including, but not limited to, the rules and procedures applicable to the selection of arbitrators. In the event that any person or entity other than the Executive or the Company may be a party with regard to any such controversy or claim, such controversy or claim shall be submitted to arbitration subject to such other person or entity's agreement. Judgment upon the award rendered by the arbitrator may be entered in any court having jurisdiction thereof. This Section 5 shall be specifically enforceable. Notwithstanding the foregoing, this Section 5 shall not preclude either party from pursuing a court action for the sole purpose of obtaining a temporary restraining order or a preliminary injunction in circumstances in which such relief is appropriate; provided that any other relief shall be pursued through an arbitration proceeding pursuant to this Section 5.

6. Working in Canada.

(a) Right to Work in Canada. Executive shall cooperate with the Company to seek, obtain, and maintain the right to work in Canada to provide services on behalf of the Company to Novellion and its Canadian Affiliates. The Company shall pay the reasonable costs associated with Executive obtaining a permit to work in Canada.

(b) Travel to Canada. Executive acknowledges that travel will be required in connection with his employment, including travel on a regular basis to such locations in Canada that is required or desirable for the Company to provide its management services to Novellion and its Canadian Affiliates, including making visits to Canada as are necessary to make decisions related to the Company's business and to manage the Company's business, including attending at Board meetings.

(c) Canadian Employment Standards. This provision applies only if and to the extent that the employment laws of Canada apply to Executive's employment. If the minimum standards in the British Columbia *Employment Standards Act* or Ontario *Employment Standards Act, 2000*, or any other applicable employment standards legislation, as they exist from time to time, are more favorable to Executive in any respect than provided for in this Agreement, including but not limited to the provisions in respect of termination, the provisions of the applicable Employment Standards Act or legislation shall apply.

(d) Tax Equalization.

(i) As Executive will be subject to income tax and social security obligations arising from his services performed in Canada on behalf of the Company, the Company is prepared to address the overall tax and social security burden that Executive experiences with the intention that Executive's total tax and social security burden while working in both the United States and Canada will be equal to what his tax and social security burden would have been had he remained working solely in Massachusetts. The Company will provide Executive with tax equalization in connection with all income tax and social security liabilities arising from the performance of his employment duties within Canada. The Company intends that the income taxes and social security levies payable by Executive on all taxable employment income and related benefits, as prescribed by the applicable tax and social security laws, should be no better or worse than the personal taxes and social security levies Executive would have been required to pay on such amounts if his employment duties had been performed solely in the state of Massachusetts. Where Executive's annual tax and social security obligation yields a higher total obligation than if his employment duties were solely performed in the state of Massachusetts, the Company will reimburse him or her for the difference. Where Executive's annual tax and social security obligations yields a lower total tax and social security impact than if his employment duties were solely performed in the state of Massachusetts, Executive will reimburse the Company for the difference.

(ii) Executive shall provide all information necessary for the preparation of a tax equalization calculation.

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(iii) The Company shall pay all reasonable costs and professional fees related to calculating this equalization payment, and reserves the discretion to establish the process and criteria for determining the tax equalization calculation. For clarity, the tax equalization payments described in this Section 6(d) will not take into consideration or apply to any taxable income from sources other than Executive's employment with the Company, and Executive will remain responsible for all income taxes arising from his personal income.

(iv) If Executive establishes his primary residence in Canada, the Company's obligations under this Section 6(d) shall cease, provided that there shall be a pro-rated adjustment for any partial year.

(v) If Executive's employment is terminated for any reason, then between January 1 and July 31 of the calendar year following the calendar year in which such termination occurs, the Company shall pay Executive any remaining tax equalization payments owed in accordance with this Section 6(d) or, in the event that the reconciliation results in Executive owing money to the Company, Executive shall make such payment to the Company.

7. Consent to Jurisdiction. To the extent that any court action is permitted consistent with or to enforce Section 5 of this Agreement, the parties hereby consent to the jurisdiction of the Superior Court of the Commonwealth of Massachusetts and the United States District Court for the District of Massachusetts. Accordingly, with respect to any such court action, the Executive (a) submits to the personal jurisdiction of such courts; (b) consents to service of process; and (c) waives any other requirement (whether imposed by statute, rule of court, or otherwise) with respect to personal jurisdiction or service of process.

8. Integration. This Agreement constitutes the entire agreement between the parties with respect to the subject matter hereof and supersedes all prior agreements between the parties concerning such subject matter.

9. Successor to the Executive. This Agreement shall inure to the benefit of and be enforceable by the Executive's personal representatives, executors, administrators, heirs, distributees, devisees and legatees. In the event of the Executive's death after his termination of employment but prior to the completion by the Company of all payments due him under this Agreement, the Company shall continue such payments to the Executive's beneficiary designated in writing to the Company prior to his death (or to his estate, if the Executive fails to make such designation).

10. Enforceability. If any portion or provision of this Agreement (including, without limitation, any portion or provision of any section of this Agreement) shall to any extent be declared illegal or unenforceable by a court of competent jurisdiction, then the remainder of this Agreement, or the application of such portion or provision in circumstances other than those as to which it is so declared illegal or unenforceable, shall not be affected thereby, and each portion and provision of this Agreement shall be valid and enforceable to the fullest extent permitted by law.

11. Survival. The provisions of this Agreement shall survive the termination of this Agreement and/or the termination of the Executive's employment to the extent necessary to effectuate the terms contained herein.

12. Waiver. No waiver of any provision hereof shall be effective unless made in writing and signed by the waiving party. The failure of any party to require the performance of any term or obligation of this Agreement, or the waiver by any party of any breach of this Agreement, shall not prevent any subsequent enforcement of such term or obligation or be deemed a waiver of any subsequent breach.

13. Notices. Any notices, requests, demands and other communications provided for by this Agreement shall be sufficient if in writing and delivered in person or sent by a nationally recognized overnight courier service or by registered or certified mail, postage prepaid, return receipt requested, to the Executive at the last address the Executive has filed in writing with the Company or, in the case of the Company, at its main offices, attention of the Board.

14. Amendment. This Agreement may be amended or modified only by a written instrument signed by the Executive and by a duly authorized representative of the Company.

15. Governing Law. This is a Massachusetts contract and shall be construed under and be governed in all respects by the laws of the Commonwealth of Massachusetts, without giving effect to the conflict of laws principles of such Commonwealth. With respect to any disputes concerning federal law, such disputes shall be determined in accordance with the law as it would be interpreted and applied by the United States Court of Appeals for the First Circuit.

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16. Counterparts. This Agreement may be executed in any number of counterparts, each of which when so executed and delivered shall be taken to be an original; but such counterparts shall together constitute one and the same document.

17. Successor to Company. The Company shall require any successor (whether direct or indirect, by purchase, merger, consolidation or otherwise) to all or substantially all of the business or assets of the Company expressly to assume and agree to perform this Agreement to the same extent that the Company would be required to perform it if no succession had taken place. Failure of the Company to obtain an assumption of this Agreement at or prior to the effectiveness of any succession shall be a material breach of this Agreement.

18. Gender Neutral. Wherever used herein, a pronoun in the masculine gender shall be considered as including the feminine gender unless the context clearly indicates otherwise.

IN WITNESS WHEREOF, the parties have executed this Agreement effective on the date and year first above written ("Effective Date").

**NOVELION SERVICES USA, INC.**

By: /s/ Linda Buono  
Its: *Senior Vice President, Human Resources*

**JEFFREY HACKMAN**

/s/ Michael D. Price

November 30, 2017

**BY EMAIL**  
**PERSONAL AND CONFIDENTIAL**

Greg Perry

**Re: Resignation Agreement**

Dear Greg:

This letter confirms your resignation from employment with Novelion Services, USA, Inc. (the "Company") and proposes an agreement (the "Agreement") between you and the Company. The purpose of this Agreement is to establish an amicable arrangement for ending your employment relationship, including releasing the Company and related persons or entities from any claims and permitting you to receive separation pay and related benefits.

You acknowledge that you are entering into this Agreement knowingly and voluntarily. By proposing and entering into this Agreement, the Company is not admitting in any way that it violated any legal obligation that it owed to you.

With those understandings, you and the Company agree as follows:

**1. Resignation from Employment**

This confirms that you have submitted your written resignation from employment with the Company effective on December 31, 2017 (the "Resignation Date"). You also confirm that you have resigned from your status as Chief Financial Officer, effective December 4, 2017. You further confirm that you have submitted your written resignation from any and all other positions that you hold with the Company as an officer, director or otherwise, or with any affiliate or subsidiary of the Company, effective on the Resignation Date. You agree that until the Resignation Date, you will continue to perform your duties and responsibilities at the Company, including the transition of those duties and responsibilities to your successor. You acknowledge that as of the Company's most recent payroll payment of salary to you, you were fully paid for all salary then due to you. The Company agrees to pay you your salary, benefits, and all accrued but unused vacation through the Resignation Date. The Company shall reimburse you for any business expenses incurred through the Resignation Date, in accordance with the Company's expense reimbursement policy. The Company shall also provide you with the tax consultation expense reimbursement and the tax equalization benefit for the 2017 tax year, as described in Sections 10 and 11 of your Employment Agreement dated November 28, 2016.

**2. Severance Benefits**

(a) Severance Pay. The Company shall pay you severance pay consisting of salary continuation at your final base salary rate of \$450,000 per year effective for the period from the date immediately following the Resignation Date to and including December 31, 2018 (the "Severance Pay Period"). The Company shall pay you salary continuation on its regular payroll dates; *provided* that the Company shall not be obligated to include you on the payroll before this Agreement becomes effective. If the Company does not make one or more payments of salary continuation on a regular payroll date because this Agreement has not yet become effective, the Company shall make all such delayed payments by the first payroll date when it is practicable to do so after the Agreement becomes effective. Subject to your timely election of COBRA coverage, the Company shall also continue to pay the employer portion of your premiums for the Company's group health and dental programs until the earliest of (i) the end of the Severance Pay Period; (ii) the date you become eligible for health and/or dental insurance coverage through other employment; or (iii) the end of your eligibility under COBRA for continuation coverage for medical or dental care. In addition, no later than March 15, 2018, the Company shall pay you your 2017 bonus in the gross amount of \$168,750.00. The Company also shall pay you the gross sum of \$225,000.00, representing a 2018 bonus, on the last date on which your salary continuation is paid during the Severance Pay Period. The salary continuation and benefits payments and the two bonus payments described in this Section 2(a) shall be defined as "Severance Pay" for the purposes of this Agreement.

(b) Tax Treatment. The Company shall make deductions, withholdings and tax reports with respect to payments and benefits under this Agreement that it reasonably determines to be required. Payments under this Agreement shall be in amounts net of any such deductions or withholdings. Nothing in this Agreement shall be construed to require the Company to

make any payments to compensate you for any adverse tax effect associated with any payments or benefits or for any deduction or withholding from any payment or benefit.

3. **Equity**

You and the Company acknowledge that as of the Resignation Date, you are vested in 2,393 restricted stock units (RSU) in Novellion Therapeutics, Inc., from a grant dated May 9, 2016 and that all of the remaining RSUs (4,786) in such RSU award shall vest on the Effective Date (as defined in Section 13(g)). In addition, all of the 166,020 options to purchase common stock of Novellion Therapeutics, Inc., at a price of \$8.65 per share, that you were granted on December 22, 2016, shall vest on the Effective Date. All such RSUs and options are subject of the terms and conditions of the Novellion 2016 Equity Incentive Plan, including but not limited to, the exercise date of such options. You and the Company also acknowledge that other than the 2,393 RSUs that will be vested as of the Resignation Date, the 4,786 RSUs that will vest as of the Effective Date, and the 166,020 options that will vest as of the Effective Date, each discussed above, you have no other rights to stock awards, performance awards, or stock options, and none of the options or other forms of equity (including, without limitation, the 81,760 performance share units you were granted on December 22, 2016) that you hold to purchase, or that entitle you to receive, shares of common stock in Novellion Therapeutics, Inc. pursuant to the Novellion 2016 Equity Incentive Plan or any predecessor plan, are vested as of the Resignation Date, and shall lapse on that date and will not be exercisable.

4. **Continuing Obligations**

You acknowledge that your obligations under the Employee Confidentiality, Assignment of Intellectual Property and Non-Competition Agreement (“Confidentiality and Non-Competition Agreement”), dated November 28, 2016, shall continue in effect, including without limitation your obligations to maintain the confidentiality of Proprietary Information as defined in the Confidentiality and Non-Competition Agreement, to return documents and other property of the Company and to not compete or solicit unfairly. A copy of the Confidentiality and Non-Competition Agreement is enclosed as Exhibit A.

5. **Return of Property**

You agree to return all Company property to the Company by the Resignation Date, including, without limitation, computer equipment, software, keys and access cards, credit cards, files and any documents (including computerized data and any copies made of any computerized data or software) containing information concerning the Company, its business or its business relationships. In the event that you discover that you continue to retain any such property, you shall return it to the Company promptly. You also commit to deleting and finally purging any duplicates of files or documents that may contain Company information from any computer or other device that remains your property after the Resignation Date. In the event that you discover that you continue to retain any such duplicates of files or documents, you shall delete them promptly.

6. **Release of Claims**

In consideration for, among other terms, the Severance Pay, to which you acknowledge you would otherwise not be entitled, you voluntarily release and forever discharge the Company, together with Novellion Therapeutics, Inc., its and their subsidiaries, and affiliated and related entities, its and their respective predecessors, successors and assigns (collectively, the “Affiliates”), its and their respective employee benefit plans, equity incentive plans, and fiduciaries of such plans, and the current and former officers, directors, shareholders, employees, attorneys, accountants and agents of each of the foregoing in their official and personal capacities (collectively referred to as the “Company Releasees”) generally from all claims, demands, debts, damages and liabilities of every name and nature, known or unknown (“Claims”) that, as of the date when you sign this Agreement, you have, ever had, now claim to have or ever claimed to have had against any or all of the Company Releasees relating to or arising from your employment with the Company or termination or your employment with the Company. This release includes, without limitation, all Claims:

- relating to your employment by and termination of employment with the Company;
  - arising from your Employment Agreement dated November 28, 2016, as amended;
  - of wrongful discharge or violation of public policy;
  - of breach of contract;
  - of retaliation or discrimination under federal, state or local law (including, without limitation, Claims of discrimination or retaliation under the Employee Retirement Income Security Act and the Age Discrimination in Employment Act);
  - under any other federal or state statute (including, without limitation, Claims under the Fair Labor Standards Act);
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- under the Ontario Employment Standards Act, 2000 and the British Columbia Employment Standards Act, the Ontario Human Rights Code and the British Columbia Human Rights Code, and any other applicable Canadian or provincial law, regulation or other requirement (each as amended from time to time);
- for wages, bonuses, incentive compensation, commissions, stock, stock options, vacation pay or any other compensation or benefits, either under the Massachusetts Wage Act, M.G.L. c. 149, §§148-150C, or otherwise; and
- for damages or other remedies of any sort, including, without limitation, compensatory damages, punitive damages, injunctive relief and attorney's fees;

*provided*, however, that this release shall not affect (a) Claims that arise after the Effective Date of this Agreement, (b) your rights under this Agreement, (c) your vested rights under the Company's Section 401(k) plan, (d) your rights to the options and RSUs discussed above in Section 3, (e) your rights to continued health insurance coverage pursuant to COBRA, (f) your rights to indemnification arising under the Company's or any Affiliate's bylaws, contracts/agreements or otherwise by law, (g) your right to coverage under the Company's or any Affiliate's D&O insurance policy; (h) your right to contribution in the event that a judgment is entered against you for which you and the Company or any of the other Company Releasees are found to be jointly liable, (i) your right to the tax consultation expense reimbursement and the tax equalization benefit as described in Section 10 and 11 of your Employment Agreement dated November 28, 2016; or (j) Claims that cannot be lawfully waived or released.

You agree not to accept damages of any nature, other equitable or legal remedies for your own benefit or attorney's fees or costs from any of the Company Releasees with respect to any Claim released by this Agreement. As a material inducement to the Company to enter into this Agreement, you represent that you have not assigned any Claim to any third party.

#### **7. Confidentiality of Agreement-Related Information**

You agree, to the fullest extent permitted by law, to keep all Agreement-Related Information completely confidential. "Agreement-Related Information" means the negotiations leading to this Agreement and the existence and terms of this Agreement. Notwithstanding the foregoing, you may disclose Agreement-Related Information to your spouse, your attorney and your financial advisors, and to them only provided that they first agree for the benefit of the Company to keep Agreement-Related Information confidential. Nothing in this section shall be construed to prevent you from disclosing Agreement-Related Information to the extent required by a lawfully issued subpoena or duly issued court order; *provided* that you provide the Company with advance written notice and a reasonable opportunity to contest such subpoena or court order.

#### **8. Non-Disparagement**

You agree not to make any disparaging statements concerning the Company, Novelion Therapeutics, Inc., or any of its or their Affiliates, products, services or current or former officers, directors, shareholders, or individuals who you know are employees or agents of the Company, Novelion Therapeutics, Inc., or their Affiliates. The Company agrees to instruct its directors, senior executives, officers and employees who are aware of this Agreement not to make any disparaging statements concerning you, or any statements that are inconsistent with the press release issued by the Company on December 4, 2017.

#### **9. Future Cooperation**

During the Severance Pay Period, you agree to respond to any inquiry from senior management of the Company or the Board regarding the status of any matter that was within your responsibilities as an employee of the Company, provided that such inquiries shall not interfere with your personal or professional time/responsibilities, including any efforts to transition to new employment. You also agree to cooperate reasonably with the Company and all of its affiliates (including its and their outside counsel) in connection with (i) the contemplation, prosecution and defense of all phases of existing, past and future litigation about which the Company believes you may have knowledge or information; and (ii) responding to requests for information from regulatory agencies or other governmental authorities (together "Cooperation Services"). You further agree to make yourself reasonably available to provide Cooperation Services at mutually convenient times during and outside of regular business hours as reasonably deemed necessary by the Company's counsel. The Company shall not utilize this section to require you to make yourself available to an extent that would unreasonably interfere with personal or professional responsibilities that you may have. Cooperation Services include, without limitation, appearing without the necessity of a subpoena to testify truthfully in any legal proceedings in which the Company or an affiliate calls you as a witness. The Company shall reimburse you for any reasonable expenses that you incur due to your performance of Cooperation Services, after receipt of appropriate documentation consistent with the Company's business expense reimbursement policy. In addition, to the extent the Cooperation Services are provided after the Severance Pay Period, the Company shall pay you an hourly rate that is equivalent to your last base salary rate at the Company for your time spent providing such Cooperation Services.

## 10. Protected Disclosures and Other Protected Actions

Nothing contained in this Agreement limits your ability to file a charge or complaint with any federal, state or local governmental agency or commission (a “Government Agency”). In addition, nothing contained in this Agreement limits your ability to communicate with any Government Agency or otherwise participate in any investigation or proceeding that may be conducted by any Government Agency, including your ability to provide documents or other information, without notice to the Company, nor does anything contained in this Agreement apply to truthful testimony in litigation. If you file any charge or complaint with any Government Agency and if the Government Agency pursues any claim on your behalf, or if any other third party pursues any claim on your behalf, you waive any right to monetary or other individualized relief (either individually or as part of any collective or class action); *provided* that nothing in this Agreement limits any right you may have to receive a whistleblower award or bounty for information provided to the Securities and Exchange Commission. In addition, for the avoidance of doubt, pursuant to the federal Defend Trade Secrets Act of 2016, you shall not be held criminally or civilly liable under any federal or state trade secret law or under this Agreement of the Confidentiality and Non-Competition Agreement for the disclosure of a trade secret that (a) is made (i) in confidence to a federal, state, or local government official, either directly or indirectly, or to an attorney; and (ii) solely for the purpose of reporting or investigating a suspected violation of law; or (b) is made in a complaint or other document filed in a lawsuit or other proceeding, if such filing is made under seal.

## 11. Termination of Payments

If you breach any of your obligations under this Agreement, including your continuing obligations under the Confidentiality and Non-Competition Agreement, and the Company files suit against you in a court of competent jurisdiction for such breach, in addition to any other legal or equitable remedies it may have for such breach, the Company shall have the right to terminate its payments to you or for your benefit under this Agreement, and to escrow such payments in an escrow account controlled by Goodwin Procter, LLP, pending a determination by such court that you have breached any of such obligations under this Agreement. The termination of such payments in the event of your breach will not affect your continuing obligations under this Agreement, provided, however, that the parties’ obligations under Sections 7, 8 and 9 shall cease in such event.

## 12. Section 409A

(a) Anything in this Agreement to the contrary notwithstanding, if at the time of your separation from service within the meaning of Section 409A of the Internal Revenue Code of 1986, as amended (the “Code”), the Company determines that you are a “specified employee” within the meaning of Section 409A(a)(2)(B)(i) of the Code, then to the extent any payment or benefit that you become entitled to under this Agreement on account of your separation from service would be considered deferred compensation subject to the 20 percent additional tax imposed pursuant to Section 409A(a) of the Code as a result of the application of Section 409A(a)(2)(B)(i) of the Code, such payment shall not be payable and such benefit shall not be provided until the date that is the earlier of (A) six months and one day after your separation from service, or (B) your death.

(b) All in-kind benefits provided and expenses eligible for reimbursement under this Agreement shall be provided by the Company or incurred by you during the time periods set forth in this Agreement. All reimbursements shall be paid as soon as administratively practicable, but in no event shall any reimbursement be paid after the last day of the taxable year following the taxable year in which the expense was incurred. The amount of in-kind benefits provided or reimbursable expenses incurred in one taxable year shall not affect the in-kind benefits to be provided or the expenses eligible for reimbursement in any other taxable year. Such right to reimbursement or in-kind benefits is not subject to liquidation or exchange for another benefit.

(c) To the extent that any payment or benefit described in this Agreement constitutes “non-qualified deferred compensation” under Section 409A of the Code, and to the extent that such payment or benefit is payable upon your termination of employment, then such payments or benefits shall be payable only upon your “separation from service.” The determination of whether and when a separation from service has occurred shall be made in accordance with the presumptions set forth in Treasury Regulation Section 1.409A-1(h).

(d) The parties intend that this Agreement will be administered in accordance with Section 409A of the Code. To the extent that any provision of this Agreement is ambiguous as to its compliance with Section 409A of the Code, the provision shall be read in such a manner so that all payments hereunder comply with Section 409A of the Code. The parties agree that this Agreement may be amended, as reasonably requested by either party, and as may be necessary to fully comply with Section 409A of the Code and all related rules and regulations in order to preserve the payments and benefits provided hereunder without additional cost to either party.

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(e) The Company makes no representation or warranty and shall have no liability to you or any other person if any provisions of this Agreement are determined to constitute deferred compensation subject to Section 409A of the Code but do not satisfy an exemption from, or the conditions of, such Section.

13. **Other Provisions**

(a) **Absence of Reliance**. In signing this Agreement, you are not relying upon any promises or representations made by anyone at or on behalf of the Company.

(b) **Enforceability**. If any portion or provision of this Agreement (including, without limitation, any portion or provision of any section of this Agreement) shall to any extent be declared illegal or unenforceable by a court of competent jurisdiction, then the remainder of this Agreement, or the application of such portion or provision in circumstances other than those as to which it is so declared illegal or unenforceable, shall not be affected thereby, and each portion and provision of this Agreement shall be valid and enforceable to the fullest extent permitted by law.

(c) **Waiver**. No waiver of any provision of this Agreement shall be effective unless made in writing and signed by the waiving party. The failure of a party to require the performance of any term or obligation of this Agreement, or the waiver by a party of any breach of this Agreement, shall not prevent any subsequent enforcement of such term or obligation or be deemed a waiver of any subsequent breach.

(d) **Jurisdiction**. You and the Company hereby agree that the Superior Court of the Commonwealth of Massachusetts and the United States District Court for the District of Massachusetts shall have the exclusive jurisdiction to consider any matters related to this Agreement, including without limitation any claim of a violation of this Agreement. With respect to any such court action, you submit to the jurisdiction of such courts and you acknowledge that venue in such courts is proper.

(e) **Governing Law; Interpretation**. This Agreement shall be interpreted and enforced under the laws of the Commonwealth of Massachusetts, without regard to conflict of law principles. In the event of any dispute, this Agreement is intended by the parties to be construed as a whole, to be interpreted in accordance with its fair meaning, and not to be construed strictly for or against either you or the Company or the “drafter” of all or any portion of this Agreement.

(f) **Entire Agreement**. This Agreement constitutes the entire agreement between you and the Company. This Agreement supersedes any previous agreements or understandings between you and the Company, except the Confidentiality and Non-Competition Agreement and any other obligations specifically preserved in this Agreement.

(g) **Time for Consideration; Effective Date**. You acknowledge that you have knowingly and voluntarily entered into this Agreement and that the Company advises you to consult with an attorney before signing this Agreement. You understand and acknowledge that you have been given the opportunity to consider this Agreement for twenty-one (21) days from your receipt of this Agreement before signing it (the “Consideration Period”). To accept this Agreement, you must return a signed original or a signed PDF copy of this Agreement so that it is received by Linda Buono at the Company (Linda.Buono@Novelion.com) at or before the expiration of the Consideration Period. If you sign this Agreement before the end of the Consideration Period, you acknowledge that such decision was entirely voluntary and that you had the opportunity to consider this Agreement for the entire Consideration Period. For the period of seven (7) days from the date when you sign this Agreement, you have the right to revoke this Agreement by written notice to Ms. Buono, provided that such notice is delivered so that it is received at or before the expiration of the seven (7) day revocation period. This Agreement shall not become effective or enforceable during the revocation period. This Agreement shall become effective on the first business day following the expiration of the revocation period (the “Effective Date”).

(h) **Counterparts**. This Agreement may be executed in separate counterparts. When both counterparts are signed, they shall be treated together as one and the same document.

Please indicate your agreement to the terms of this Agreement by signing and returning to Ms. Buono the original or a PDF copy of this letter within the time period set forth above.

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Sincerely,

NOVELION SERVICES USA, INC.

By: /s/ Ben Harshbarger 12/4/2017      Date  
BEN HARSHBARGER  
Member of the Board of  
Novelion Services USA, Inc.

Enclosure (Exhibit A) Confidentiality and Non-Competition Agreement

You are advised to consult with an attorney before signing this Agreement. This is a legal document. Your signature will commit you to its terms. By signing below, you acknowledge that you have carefully read and fully understand all of the provisions of this Agreement and that you are knowingly and voluntarily entering into this Agreement.

/s/ Greg Perry 12/4/2017  
GREG PERRY      Date

## SUBSIDIARIES OF NOVELION THERAPEUTICS INC. AS OF DECEMBER 31, 2017

<b>Entity Name</b>	<b>Jurisdiction of Organization or Incorporation</b>
Aegerion Pharmaceuticals, Inc.	Delaware
Aegerion Pharmaceuticals Ltd.	Bermuda
Aegerion Pharmaceuticals (Canada) Ltd.	Canada
Aegerion Pharmaceuticals Holdings, Inc.	Delaware
Aegerion Argentina S.R.L.	Argentina
Aegerion Pharmaceuticals K.K.	Japan
Aegerion Securities Corporation	Massachusetts
Aegerion Pharmaceuticals Limited	United Kingdom
Aegerion Brasil Comercio E Importacao De Medicamentos LTDA	Brazil
Aegerion Pharmaceuticals, SAS	France
Aegerion Pharmaceuticals S.r.l.	Italy
Aegerion Pharmaceuticals GmbH	Germany
Aegerion Pharmaceuticals SARL	Switzerland
Aegerion İlaç Ticaret Limited Şirketi	Turkey
Aegerion Pharmaceuticals B.V.	Netherlands
Aegerion Colombia S.A.S.	Colombia
Aegerion International Ltd.	Bermuda
Novelion Services USA, Inc.	Delaware
QLT Ophthalmics (UK), Ltd.	United Kingdom
QLT Therapeutics, Inc.	Delaware
QLT Plug Delivery, Inc.	Delaware
QLT Ophthalmics, Inc.	Delaware

**CONSENT OF INDEPENDENT REGISTERED PUBLIC ACCOUNTING FIRM**

We consent to the incorporation by reference in Registration Statement Nos. 333-190221, 333-100070, 333-120657, 333-162465, 333-214907, 333-219587 and 333-219588 on Form S-8 and Registration Statement No. 333-214202 on Form S-3 of our reports dated March 16, 2018, relating to the financial statements of Novelion Therapeutics Inc. and subsidiaries (the “Company”) and the effectiveness of Novelion Therapeutics Inc.’s internal control over financial reporting (which report expresses an adverse opinion on the effectiveness of the Company’s internal control over financial reporting because of a material weakness), appearing in this Annual Report on Form 10-K of Novelion Therapeutics Inc. for the year ended December 31, 2017.

/s/ Deloitte & Touche LLP

Boston, Massachusetts  
March 16, 2018

**CONSENT OF INDEPENDENT REGISTERED PUBLIC ACCOUNTING FIRM**

We consent to the incorporation by reference in Registration Statement Nos. 333-190221, 333-100070, 333-120657, 333-162465, 333-214907, 333-219587, and 333-219588 on Form S-8 and 333-214202 on Form S-3 of our report dated March 30, 2017, relating to the 2016 and 2015 consolidated financial statements of Novelon Therapeutics Inc. and subsidiaries (the “Company”) appearing in the Annual Report on Form 10-K of the Company for the year ended December 31, 2017.

/s/ Deloitte LLP

Chartered Professional Accountants  
March 16, 2018

## CERTIFICATIONS

I, Jeffrey Hackman, certify that:

1. I have reviewed this annual report on Form 10-K of Novelion Therapeutics Inc.;
2. Based on my knowledge, this report does not contain any untrue statement of a material fact or omit to state a material fact necessary to make the statements made, in light of the circumstances under which such statements were made, not misleading with respect to the period covered by this report;
3. Based on my knowledge, the financial statements, and other financial information included in this report, fairly present in all material respects the financial condition, results of operations and cash flows of the registrant as of, and for, the periods presented in this report;
4. The registrant's other certifying officer(s) and I are responsible for establishing and maintaining disclosure controls and procedures (as defined in Exchange Act Rules 13a-15(e) and 15d-15(e)) and internal control over financial reporting (as defined in Exchange Act Rules 13a-15(f) and 15d-15(f)) for the registrant and have:
  - (a) Designed such disclosure controls and procedures, or caused such disclosure controls and procedures to be designed under our supervision, to ensure that material information relating to the registrant, including its consolidated subsidiaries, is made known to us by others within those entities, particularly during the period in which this report is being prepared;
  - (b) Designed such internal control over financial reporting, or caused such internal control over financial reporting to be designed under our supervision, to provide reasonable assurance regarding the reliability of financial reporting and the preparation of financial statements for external purposes in accordance with generally accepted accounting principles;
  - (c) Evaluated the effectiveness of the registrant's disclosure controls and procedures and presented in this report our conclusions about the effectiveness of the disclosure controls and procedures, as of the end of the period covered by this report based on such evaluation; and
  - (d) Disclosed in this report any change in the registrant's internal control over financial reporting that occurred during the registrant's most recent fiscal quarter (the registrant's fourth fiscal quarter in the case of an annual report) that has materially affected, or is reasonably likely to materially affect, the registrant's internal control over financial reporting; and
5. The registrant's other certifying officer(s) and I have disclosed, based on our most recent evaluation of internal control over financial reporting, to the registrant's auditors and the audit committee of the registrant's board of directors (or persons performing the equivalent functions):
  - (a) All significant deficiencies and material weaknesses in the design or operation of internal control over financial reporting which are reasonably likely to adversely affect the registrant's ability to record, process, summarize and report financial information; and
  - (b) Any fraud, whether or not material, that involves management or other employees who have a significant role in the registrant's internal control over financial reporting.

Date: March 16, 2018

/s/ Jeffrey Hackman

Name: Jeffrey Hackman

Title: Principal Executive Officer

## CERTIFICATIONS

I, Michael D. Price, certify that:

1. I have reviewed this annual report on Form 10-K of Novelion Therapeutics Inc.;
2. Based on my knowledge, this report does not contain any untrue statement of a material fact or omit to state a material fact necessary to make the statements made, in light of the circumstances under which such statements were made, not misleading with respect to the period covered by this report;
3. Based on my knowledge, the financial statements, and other financial information included in this report, fairly present in all material respects the financial condition, results of operations and cash flows of the registrant as of, and for, the periods presented in this report;
4. The registrant's other certifying officer(s) and I are responsible for establishing and maintaining disclosure controls and procedures (as defined in Exchange Act Rules 13a-15(e) and 15d-15(e)) and internal control over financial reporting (as defined in Exchange Act Rules 13a-15(f) and 15d-15(f)) for the registrant and have:
  - (a) Designed such disclosure controls and procedures, or caused such disclosure controls and procedures to be designed under our supervision, to ensure that material information relating to the registrant, including its consolidated subsidiaries, is made known to us by others within those entities, particularly during the period in which this report is being prepared;
  - (b) Designed such internal control over financial reporting, or caused such internal control over financial reporting to be designed under our supervision, to provide reasonable assurance regarding the reliability of financial reporting and the preparation of financial statements for external purposes in accordance with generally accepted accounting principles;
  - (c) Evaluated the effectiveness of the registrant's disclosure controls and procedures and presented in this report our conclusions about the effectiveness of the disclosure controls and procedures, as of the end of the period covered by this report based on such evaluation; and
  - (d) Disclosed in this report any change in the registrant's internal control over financial reporting that occurred during the registrant's most recent fiscal quarter (the registrant's fourth fiscal quarter in the case of an annual report) that has materially affected, or is reasonably likely to materially affect, the registrant's internal control over financial reporting; and
5. The registrant's other certifying officer(s) and I have disclosed, based on our most recent evaluation of internal control over financial reporting, to the registrant's auditors and the audit committee of the registrant's board of directors (or persons performing the equivalent functions):
  - (a) All significant deficiencies and material weaknesses in the design or operation of internal control over financial reporting which are reasonably likely to adversely affect the registrant's ability to record, process, summarize and report financial information; and
  - (b) Any fraud, whether or not material, that involves management or other employees who have a significant role in the registrant's internal control over financial reporting.

Date: March 16, 2018

/s/ Michael D. Price

Name: Michael D. Price

Title: Principal Financial Officer

**CERTIFICATIONS PURSUANT TO  
18 U.S.C. SECTION 1350,  
AS ADOPTED PURSUANT TO  
SECTION 906 OF THE SARBANES-OXLEY ACT OF 2002**

In connection with this Annual Report on Form 10-K of Novelion Therapeutics Inc. (the "Company") for the fiscal year ended December 31, 2017, as filed with the Securities and Exchange Commission on the date hereof (the "Report"), the undersigned officer of the Company hereby certifies, pursuant to 18 U.S.C. (section) 1350, as adopted pursuant to (section) 906 of the Sarbanes-Oxley Act of 2002, that to the best of my knowledge:

- (1) The Report fully complies with the requirements of Section 13(a) or 15(d) of the Securities Exchange Act of 1934; and
- (2) The information contained in the Report fairly presents, in all material respects, the financial condition and results of operations of the Company.

/s/ Jeffrey Hackman

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Name: Jeffrey Hackman

Title: Principal Executive Officer

Date: March 16, 2018

A signed original of this written statement required by Section 906 has been provided to the Company and will be retained by the Company and furnished to the Securities and Exchange Commission or its staff upon request.

**CERTIFICATIONS PURSUANT TO  
18 U.S.C. SECTION 1350,  
AS ADOPTED PURSUANT TO  
SECTION 906 OF THE SARBANES-OXLEY ACT OF 2002**

In connection with this Annual Report on Form 10-K of Novelion Therapeutics Inc. (the "Company") for the fiscal year ended December 31, 2017, as filed with the Securities and Exchange Commission on the date hereof (the "Report"), the undersigned officer of the Company hereby certifies, pursuant to 18 U.S.C. (section) 1350, as adopted pursuant to (section) 906 of the Sarbanes-Oxley Act of 2002, that to the best of my knowledge:

- (1) The Report fully complies with the requirements of Section 13(a) or 15(d) of the Securities Exchange Act of 1934; and
- (2) The information contained in the Report fairly presents, in all material respects, the financial condition and results of operations of the Company.

/s/ Michael D. Price

\_\_\_\_\_  
Name: Michael D. Price

Title: Principal Financial Officer

Date: March 16, 2018

A signed original of this written statement required by Section 906 has been provided to the Company and will be retained by the Company and furnished to the Securities and Exchange Commission or its staff upon request.