

UNITED STATES
SECURITIES AND EXCHANGE COMMISSION
Washington, D.C. 20549

FORM 10-K

ANNUAL REPORT PURSUANT TO SECTION 13 OR 15(d) OF THE SECURITIES EXCHANGE ACT OF 1934

For the Fiscal Year Ended December 31, 2018

Transition Report Pursuant to Section 13 or 15(d) of the Securities Exchange Act of 1934

Commission File Number: 001-36257

RETROPHIN, INC.

(Exact Name of Registrant as specified in its Charter)

Delaware
(State or other jurisdiction of incorporation or organization)

27-4842691
(I.R.S. Employer Identification No.)

3721 Valley Centre Drive, Suite 200, San Diego CA
(Address of Principal Executive Offices)

92130
(Zip code)

760-260-8600

(Registrant's telephone number, including area code)

Securities registered pursuant to Section 12(b) of the Act:

Title of each class	Name of exchange on which registered
Common Stock, par value \$0.0001 per share	The Nasdaq Global 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 Section 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 every Interactive Data File required to be submitted pursuant to Rule 405 of Regulation S-T during the preceding 12 months (or for such shorter period that the registrant was required to submit such files). Yes No

Indicate by check mark if disclosure of delinquent filers pursuant to Item 405 of Regulation S-K 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 an emerging growth company. See 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 checked="" type="checkbox"/>	Accelerated Filer	<input type="checkbox"/>
Non-Accelerated Filer	<input type="checkbox"/>	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 7(a)(2)(B) of the Securities Act.

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

State the aggregate market value of the voting and non-voting common equity held by non-affiliates computed by reference to the price at which the common equity was last sold, or the average bid and asked price of such common equity, as of the last business day of the registrant's most recently completed second fiscal quarter. \$912,685,763.

The number of shares of outstanding common stock, par value \$0.0001 per share, of the Registrant as of February 25, 2019 was 41,412,835.

DOCUMENTS INCORPORATED BY REFERENCE: Portions of the Proxy Statement for the registrant's Annual Meeting of Stockholders to be held May 8, 2019, to be filed within 120 days after the conclusion of the registrant's fiscal year ended December 31, 2018, are incorporated by reference into Part III of this Annual Report on Form 10-K.

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CAUTIONARY STATEMENT REGARDING FORWARD-LOOKING STATEMENTS

Certain information contained in this Annual Report on Form 10-K of Retrophin, Inc., a Delaware corporation (the "Company") include forward-looking statements within the meaning of the Private Securities Litigation Reform Act of 1995. The statements herein which are not historical reflect our current expectations and projections about the Company's future results, performance, liquidity, financial condition, prospects and opportunities and are based upon information currently available to the Company and management and is subject to its interpretation of what is believed to be significant factors affecting the businesses, including many assumptions regarding future events. Such forward-looking statements include statements regarding, among other things:

- our ability to produce, sustain and expand sales of our products;
- our ability to develop, acquire and/or introduce new products;
- our projected future sales, profitability and other financial metrics;
- our future financing plans;
- our anticipated needs for working capital;
- the anticipated trends in our industry;
- acquisitions of other companies or assets that we might undertake in the future;
- our operations in the United States and abroad, and the domestic and foreign regulatory, economic and political conditions; and
- competition existing today or that will likely arise in the future.

Forward-looking statements, which involve assumptions and describe our future plans, strategies and expectations, are generally identifiable by use of the words "may," "should," "expect," "anticipate," "estimate," "believe," "intend," "seek," or "project" or the negative of these words or other variations on these words or comparable terminology. Actual results, performance, liquidity, financial condition and results of operations, prospects and opportunities could differ materially from those expressed in, or implied by, these forward-looking statements as a result of various risks, uncertainties and other factors, including the ability to raise sufficient capital to continue the Company's operations. Actual events or results may differ materially from those discussed in forward-looking statements as a result of various factors, including, without limitation, the risks outlined under "Risk Factors" and matters described in this Annual Report generally. In light of these risks and uncertainties, there can be no assurance that the forward-looking statements contained in this Annual Report will in fact occur. Potential investors should not place undue reliance on any forward-looking statements. Except as expressly required by the federal securities laws, there is no undertaking to publicly update or revise any forward-looking statements, whether as a result of new information, future events, changed circumstances or any other reason.

The specific discussions in this Annual Report about the Company include financial projections and future estimates and expectations about the Company's business. The projections, estimates and expectations are presented in this Annual Report only as a guide about future possibilities and do not represent actual amounts or assured events. All the projections and estimates are based exclusively on the Company management's own assessment of the business, the industry in which it works and the economy at large and other operational factors, including capital resources and liquidity, financial condition, fulfillment of contracts and opportunities. The actual results may differ significantly from the projections.

Potential investors should not make an investment decision based solely on the Company's projections, estimates or expectations.

PART I

In this Annual Report on Form 10-K, unless the context requires otherwise, the terms “we”, “our”, “us”, “Retrophin” and the “Company” refer to Retrophin, Inc., a Delaware corporation, as well as our direct and indirect subsidiaries.

We own or have rights to various trademarks used in our business, including those referenced in the subsection of Item 1 below titled “Trademarks”. Our logos and trademarks are the property of Retrophin, Inc. All other brand names or trademarks appearing in this report are the property of their respective holders.

ITEM 1. BUSINESS

Those statements in the following discussion that are not historical in nature should be considered forward-looking statements that are inherently uncertain. Actual results and the timing of the events may differ materially from those contained in these forward looking statements due to a number of factors, including those discussed in the “Cautionary Statement Regarding Forward-Looking Statements” and “Risk Factors” set forth elsewhere in this Annual Report.

Overview

We are a biopharmaceutical company headquartered in San Diego, California, focused on identifying, developing and delivering life-changing therapies to people living with rare diseases. Our approach centers on our pipeline with multiple late clinical-stage programs targeting rare diseases with significant unmet medical needs. Our research and development efforts are supported by revenues from our commercialized products, Chenodal®, Cholbam® and Thiola®. In addition we regularly evaluate and, where appropriate, act on opportunities to expand our product pipeline through licenses and acquisitions of products in areas that will serve patients with serious or rare diseases and that we believe offer attractive growth characteristics.

We currently have the following product candidates in clinical development:

Fosmetpantotenate (RE-024)

Fosmetpantotenate, also known as RE-024, is a novel small molecule being evaluated in a Phase 3 clinical study as a potential treatment for pantothenate kinase-associated neurodegeneration (“PKAN”). PKAN is a genetic neurodegenerative disorder that is typically diagnosed in the first decade of life. Symptoms of PKAN include dystonia, dysarthria, rigidity, retinal degeneration, and severe digestive problems.

Sparsentan (RE-021)

Sparsentan, also known as RE-021, is an investigational product candidate with a dual mechanism of action, a potent angiotensin receptor blocker (“ARB”) and selective endothelin receptor antagonist (“ERA”), with in vitro selectivity toward endothelin receptor type A. We have secured a license to sparsentan from Ligand Pharmaceuticals, Inc. and Bristol-Myers Squibb Company (“BMS”) (who referred to it as DARA). Sparsentan is currently being evaluated in two pivotal Phase 3 clinical studies in the following indications:

- **Focal segmental glomerulosclerosis (“FSGS”)** is a rare kidney disease characterized by proteinuria where the glomeruli become progressively scarred. FSGS is a leading cause of end-stage renal disease.
- **Immunoglobulin A nephropathy (“IgAN”)** is an immune-complex-mediated glomerulonephritis characterized by hematuria, proteinuria, and variable rates of progressive renal failure. IgAN is the most common primary glomerular disease.

CNSA-001

We have entered into a joint development agreement with Censa Pharmaceuticals Inc. (“Censa”), a privately held biotechnology company focused on developing therapies for orphan metabolic diseases, to evaluate CNSA-001 for the treatment of phenylketonuria (PKU). CNSA-001 is an orally bioavailable proprietary form of sepiapterin, a natural precursor of tetrahydrobiopterin (BH4) that is converted by an endogenous enzymatic pathway to BH4. CNSA-001 is currently being evaluated in a Phase 2 proof-of-concept study in patients with PKU.

In addition, we also have the following programs in discovery or development:

NGLY1 Deficiency Discovery Efforts

N-glycanase deficiency, or NGLY1 deficiency, is an extremely rare genetic disorder believed to be caused by a deficiency in an enzyme called N-glycanase-1, which is encoded by the gene NGLY1. In September 2017, we entered into a three-way Cooperative Research and Development Agreement (“CRADA”) with the National Institutes of Health’s National Center for Advancing Translational Sciences (NCATS) and patient advocacy foundation NGLY1.org to collaborate on research efforts aimed at the identification of potential small molecule therapeutics for NGLY1 deficiency.

Liquid Ursodeoxycholic Acid

Liquid ursodeoxycholic acid ("L-UDCA") is a liquid formulation of ursodeoxycholic acid being developed for the treatment of a rare liver disease called primary biliary cholangitis ("PBC"). We obtained rights to L-UDCA in 2016 with the intention of making L-UDCA commercially available to the subset of PBC patients who have difficulty swallowing. There are no liquid formulations of ursodeoxycholic acid currently approved by the U.S. Food and Drug Administration (FDA).

We currently sell the following three products:

- **Chenodal® (chenodeoxycholic acid)** is approved in the United States for the treatment of patients suffering from gallstones in whom surgery poses an unacceptable health risk due to disease or advanced age. Chenodal® has also been the standard of care for cerebrotendinous xanthomatosis ("CTX") patients for more than three decades and we are currently pursuing adding this indication to the label.
- **Cholbam® (cholic acid)** is approved in the United States for the treatment of bile acid synthesis disorders due to single enzyme defects and is further indicated for adjunctive treatment of patients with certain peroxisomal disorders.
- **Thiola® (tiopronin)** is approved in the United States for the prevention of cystine (kidney) stone formation in patients with severe homozygous cystinuria.




Our Strategy

Our goal is to become a preeminent, global and fully-integrated biopharmaceutical company within the rare disease community. In order to achieve our goal, we intend to:

- **Focus on developing products to treat rare diseases characterized by severe unmet medical needs.** We believe that our research, development, and commercialization capabilities in rare disease represent distinct competitive advantages. We leverage our development capabilities in rare disease to focus on advancing therapeutic candidates with life-changing potential. Given these capabilities, the well-established regulatory model and the ability to demonstrate clinical effects in small clinical studies, we believe that we can successfully bring new therapies to patients living with severe unmet medical needs.
- **Develop a sustainable pipeline by employing disciplined decision criteria in the evaluation of potential in-licensing candidates.** We seek to build a sustainable product pipeline by employing multiple therapeutic approaches and by developing or acquiring orphan drug candidates. We seek to augment our internally developed pipeline projects by selectively and strategically acquiring pipeline assets that will add value to the portfolio. We intend to mitigate risk by employing rigorous decision criteria, favoring drug candidates that have undergone at least some clinical study. Our decision to acquire rights to a drug candidate also depends on the scientific merits of the available clinical data; the identifiable orphan patient population; the economic terms of any proposed acquisition of rights; the projected amount of capital required to develop the drug candidate; and the economic potential of the drug candidate, should it be commercialized. We believe this strategy minimizes our clinical development risk and allows us to accelerate the development and potential commercialization of current and future drug candidates.
- **Evaluate the commercialization strategies on a product-by-product basis to maximize the value of each.** As we move our drug candidates through development toward regulatory approval, we will evaluate several options for each drug candidate's commercialization strategy. These options include utilizing or expanding our own internal sales force; entering into joint marketing partnerships with other pharmaceutical or biotechnology companies, whereby we jointly sell and market the product; and out-licensing our products, whereby other pharmaceutical or biotechnology companies sell and market our product and pay us a royalty on sales. Our decision will be made separately for each product and will be based on a number of factors including capital necessary to execute on each option, size of the market and terms of potential offers from other pharmaceutical and biotechnology companies.

Our Product Candidates and Products on the Market

The following table summarizes the status of our product candidates, preclinical programs and products on the market, each of which is described in further detail below.

Program / Product	Indication	Preclinical	Phase 1	Phase 2	Phase 3	Marketed
Fosmetpantotenate (RE-024)	PKAN					
Sparsentan (RE-021)	FSGS					
	IgAN					
CNSA-001*	PKU					
NGLY1 Collaboration & Discovery Efforts	NGLY1 Deficiency					
Liquid Ursodeoxycholic Acid**	PBC					
						
						
						

*CNSA-001 is being developed in a strategic collaboration with Censa Pharmaceuticals.

**Activities underway with the intention of making a liquid formulation commercially available in the United States.

Product Candidates:

Fosmetpantotenate

We are developing fosmetpantotenate, a novel small molecule, as a potential treatment for PKAN. PKAN is a genetic neurodegenerative disorder that is typically diagnosed in the first decade of life. Symptoms of PKAN include dystonia, dysarthria, rigidity, retinal degeneration, and severe digestive problems. PKAN is estimated to affect up to 5,000 patients worldwide. There are currently no viable treatment options for patients with PKAN. Fosmetpantotenate is a phosphopantothenate replacement therapy that aims to restore levels of this key substrate in PKAN patients. Certain international health regulators have approved the initiation of dosing fosmetpantotenate in PKAN patients under physician-initiated studies in accordance with local regulations in their respective countries.

In 2015 and 2016 we filed a U.S. Investigational New Drug application ("IND"), completed the Phase I clinical trials and obtained both orphan drug and fast track designation in the United States. Additionally, we received orphan drug designation in the European Union and reached an agreement with the FDA under the Special Protocol Assessment (SPA) process for a Phase 3 clinical trial for PKAN.

In the fourth quarter of 2018, the pivotal Phase 3 FORT Study of fosmetpantotenate in PKAN completed patient enrollment. The FORT Study is designed to be registration-enabling in the U.S. and Europe, and we expect top-line data to become available in the third quarter of 2019.

Sparsentan

Sparsentan is an investigational product candidate with a dual mechanism of action, a potent ARB and selective ERA, with in vitro selectivity toward endothelin receptor type A. We have secured a license to sparsentan from Ligand Pharmaceuticals, Inc. and BMS (who referred to it as DARA). Sparsentan is currently being evaluated in two pivotal Phase 3 clinical studies in the following indications:

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- FSGS, a leading cause of end-stage renal disease and nephrotic syndrome ("NS"). There are currently no FDA approved pharmacologic treatments for FSGS and off-label treatments are limited to ACE/ARBs, steroids, and immunosuppressant agents, which are effective in only a subset of patients. Every year approximately 5,400 patients are diagnosed with FSGS and we estimate that there are up to 40,000 FSGS patients in the United States with approximately half of them being candidates for sparsentan. In 2015 and 2016 we received orphan drug designation in the United States and European Union and generated positive data from our Phase 2 DUET study of sparsentan for the treatment of FSGS. In the second quarter of 2018, we announced the initiation of the Phase 3 DUPLEX Study of sparsentan in FSGS, and enrollment continues. This pivotal DUPLEX Study is designed to include an interim analysis of modified partial remission of proteinuria. We expect that successful achievement of this endpoint will serve as the basis for submission of a New Drug Application (NDA) for sparsentan for the treatment of FSGS under the Subpart H accelerated approval pathway in the United States and Conditional Marketing Authorization ("CMA") consideration in Europe. The confirmatory endpoint of the study will compare changes in slope of estimated glomerular filtration rate, or eGFR. Top-line data from the interim analysis are expected to become available in the second half of 2020.
- IgAN is characterized by hematuria, proteinuria, and variable rates of progressive renal failure. With an estimated prevalence of more than 100,000 people in the United States and greater numbers in Europe and Asia, IgAN is the most common primary glomerular disease. Most patients are diagnosed between the ages of 16 and 35, with up to 40% progressing to end stage renal disease within 15 years. There are currently no FDA approved treatments for IgAN. The current standard of care is renin-angiotensin-aldosterone system (RAAS) blockade with immunosuppression also being commonly used for patients with significant proteinuria or rapidly progressive glomerulonephritis. In the fourth quarter of 2018, we announced that the first patient had been dosed in the PROTECT Study, a global, pivotal Phase 3 clinical trial evaluating the long-term nephroprotective potential of sparsentan for the treatment of IgAN. This PROTECT Study is a global, randomized, multicenter, double-blind, parallel-arm, active-controlled Phase 3 clinical trial evaluating the safety and efficacy of sparsentan in approximately 280 patients with IgAN aged 18 years or older. The primary efficacy endpoint in the PROTECT Study is the change in proteinuria (urine protein-to-creatinine ratio) from baseline after 36 weeks of treatment. We expect that successful achievement of this endpoint will serve as the basis for submission of an NDA for sparsentan for the treatment of IgAN under the Subpart H accelerated approval pathway in the U.S. and CMA consideration in Europe. Secondary efficacy endpoints include change in eGFR from baseline to four weeks post-cessation of randomized treatment, as well as the rate of change in eGFR over 52-week and 104-week periods following the first six weeks of randomized treatment. Top-line data from the primary endpoint are expected to become available in the first half of 2022.

CNSA-001

We are a party to a joint development agreement with Censa, a privately held biotechnology company focused on developing therapies for orphan metabolic diseases, to evaluate CNSA-001 for the treatment of PKU. CNSA-001 is an orally available, natural precursor of tetrahydrobiopterin (BH4) with the potential to provide improved phenylalanine (Phe) reduction in patients with PKU when compared to BH4.

PKU is a rare, genetic metabolic condition in which the body cannot breakdown Phe due to a missing or defective phenylalanine hydroxylase (PAH) enzyme. High Phe levels can lead to developmental and physical growth delay, executive function impairment, seizures, and microcephaly caused by toxic Phe accumulation in the brain. PKU is typically diagnosed at birth.

Under the terms of the agreement, we are providing funding for the development of CNSA-001 in PKU. Censa is responsible for the development program, which is being conducted under the oversight of a joint steering committee. As part of the agreement, we owe certain milestone payments upon achievement of specified milestones and we have the exclusive option to acquire Censa upon conclusion of a specified option period, pending clinical proof of concept of CNSA-001 in PKU.

Censa is currently conducting a Phase 2 proof-of-concept study evaluating CNSA-001 in patients with PKU and we expect top-line data to become available to us during the second quarter of 2019.

NGLY1 Deficiency Discovery Efforts

NGLY1 deficiency, is an extremely rare genetic disorder believed to be caused by a deficiency in an enzyme called N-glycanase-1, which is encoded by the gene NGLY1. The condition is characterized by symptoms such as developmental delays, seizures, complex hyperkinetic movement disorders, diminished reflexes and an inability to produce tears. There are no approved therapeutic options for NGLY1 deficiency, and current therapeutic strategies are limited to symptom management.

In September 2017, we entered into a three-way CRADA with the National Institutes of Health's National Center for Advancing Translational Sciences (NCATS) and patient advocacy foundation NGLY1.org to collaborate on research efforts aimed at the identification of potential small molecule therapeutics for NGLY1 deficiency.

Liquid Ursodeoxycholic Acid

L-UDCA is a liquid formulation of ursodeoxycholic acid being developed for the treatment of a rare liver disease called PBC. We obtained rights to L-UDCA in 2016 with the intention of making L-UDCA commercially available to the subset of PBC patients who have difficulty swallowing. There are no liquid formulations of ursodeoxycholic acid currently approved by the FDA.

Products on the Market:

Chenodal (chenodiol tablets)

Chenodal is a synthetic oral form of chenodeoxycholic acid, a naturally occurring primary bile acid synthesized from cholesterol in the liver, indicated for the treatment of radiolucent stones in well-opacifying gallbladders in patients in whom selective surgery would be undertaken except for the presence of increased surgical risk due to systemic disease or age.

Chenodal administration is known to reduce biliary cholesterol and the dissolution of radiolucent gallstones through suppression of hepatic synthesis of cholesterol, cholic acid and deoxycholic acid in the bile pool. Chenodal was first approved by the FDA in 1983 for the management of gallstones but its marketing was later discontinued due to lack of commercial success. In 2009, Nexgen Pharma Inc.'s Abbreviated New Drug Application ("ANDA") for Chenodal was approved by the FDA for the treatment of gallstones. Chenodal is manufactured under this ANDA. In 2010, Chenodal was granted orphan drug designation for the treatment of CTX, a rare autosomal recessive lipid storage disease. We acquired Chenodal in March 2014.

While Chenodal is not labeled for CTX, it has been used as the standard of care for over three decades. We are working to obtain FDA approval of Chenodal for the treatment of CTX. The prevalence of CTX is estimated in the literature to be as high as 1 in 70,000 in the overall population. Pathogenesis of CTX involves deficiency of the enzyme 27-hydroxylase (encoded by the gene CYP27A1), a rate-limiting enzyme in the synthesis of primary bile acids, including chenodeoxycholic acid ("CDCA"), from cholesterol. The disruption of primary bile acid synthesis in CTX leads to toxic accumulation of cholesterol and cholestanol in most tissues. Most patients present with intractable diarrhea, premature cataracts, tendon xanthomas, atherosclerosis, and cardiovascular disease in childhood and adolescence. Neurological manifestations of the disease, including dementia and cognitive and cerebellar deficiencies, emerge during late adolescence and adulthood. Oral administration of CDCA has been shown to normalize primary bile acid synthesis in patients with CTX.

Cholbam (cholic acid capsules)

The FDA approved Cholbam (cholic acid capsules) in March 2015, the first FDA approved treatment for pediatric and adult patients with bile acid synthesis disorders due to single enzyme defects, and for adjunctive treatment of patients with peroxisomal disorders (including Zellweger spectrum disorders). The effectiveness of Cholbam has been demonstrated in clinical trials for bile acid synthesis disorders and the adjunctive treatment of peroxisomal disorders. An estimated 200 to 300 patients are current candidates for therapy.

Kolbam, the branded name of Cholbam in Europe, is indicated in Europe for the treatment of certain inborn errors of primary bile acid synthesis, encompassing select single enzyme defects, in infants from one month of age for continuous lifelong treatment through adulthood.

Thiola (tiopronin tablets)

Thiola is approved by the FDA for the treatment of cystinuria, a rare genetic cystine transport disorder that causes high cystine levels in the urine and the formation of recurring kidney stones. The resulting long-term damage can cause loss of kidney function in addition to substantial pain and loss of productivity associated with renal colic and stone passage. The prevalence of cystinuria in the United States is estimated to be 10,000 to 12,000, indicating that there may be as many as 4,000 to 5,000 affected individuals with cystinuria in the United States that would be candidates for Thiola. An NDA has been filed for a new, more patient-friendly, formulation of Thiola and the FDA has assigned a Prescription Drug User Fee Act ("PDUFA") target action date of June 30, 2019.

Competition

The pharmaceutical and biotechnology industries are intensely competitive and subject to rapid and significant technological change. Many of our competitors are larger than our company and have substantially greater financial, marketing and technical resources than we have.

The development and commercialization of new products to treat orphan diseases is highly competitive, and we expect considerable competition from major pharmaceutical, biotechnology and specialty pharmaceutical companies. As a result, there are, and will likely continue to be, extensive research and substantial financial resources invested in the discovery and development of new orphan drug products.

Our competition will be determined in part by the potential indications for which drugs are developed and ultimately approved by regulatory authorities. The speed with which we can develop products, complete pre-clinical testing, clinical trials, approval processes, and supply commercial quantities to market are expected to be important competitive factors. We expect that competition among products approved for sale will be based on various factors, including product efficacy, safety, reliability, availability, price, reimbursement, patent position, and regulatory exclusivity.

Chenodal

There are other non-approved chenodeoxycholic acid products available outside of the United States. Furthermore, Chenodal is subject to immediate competition from compounded and generic entrants, as the ANDA for this drug has no remaining patent or nonpatent exclusivity.

Cholbam

There are currently no FDA approved treatments in the United States that compete with Cholbam.

Thiola

D-penicillamine agents, including Cuprimine® and Depen® are FDA approved for the treatment of cystinuria, as well as Wilson's Disease. Additional generic versions of D-penicillamine may become FDA-approved and enter the market.

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Captopril is not FDA approved for the treatment of cystinuria but has been prescribed for patients with cystinuria.

In 2016, Imprimis Pharmaceuticals, Inc., a specialty pharmaceutical company, announced plans to introduce a compounded form of tiopronin, the active ingredient in Thiola, in combination with potassium citrate. Compounded therapies are not subjected to the same level of safety and efficacy evaluation and may not offer the same therapeutic outcome for patients. There is no clinical data to support the compatibility of fixed dosing of tiopronin with potassium citrate. Fixed-dose combinations of therapies containing potassium are generally avoided due to the potential for fluctuations in serum potassium, which may cause serious adverse outcomes including cardiac events.

Revive Therapeutics is developing buccillamine, a dithiol derivative of tiopronin for the treatment of cystinuria. The FDA has granted orphan drug designation for the use of buccillamine for the treatment of cystinuria.

Furthermore, Thiola is subject to immediate competition from compounded and generic entrants, as the NDA for this drug has no remaining patent or nonpatent exclusivity.

Fosmetpantotenate

Based on industry reports, Apo Pharma, CoA Therapeutics, Spoonbill Foundation together with Oregon Health & Science University, and TM3 Therapeutics may have programs in pre-clinical or clinical development for the treatment of PKAN.

Sparsentan

There are currently no pharmacological treatments approved by the FDA for FSGS or IgAN in Europe or the United States. For FSGS the current standard of care includes steroids, ACE/ARBs, calcineurin inhibitors, dialysis, and renal transplant. For IgAN, the current standard of care is renin-angiotensin-aldosterone system (RAAS) blockade with immunosuppression also being commonly used in patients with significant proteinuria or rapidly progressive glomerulonephritis.

Based on industry reports, Aurinia Pharmaceuticals, Complexia Inc., Dimerix Bioscience, ChemoCentryx, BMS, Pfizer, and Reata Pharmaceuticals may have programs in clinical development for the treatment of FSGS.

Based on industry reports, Apellis Pharmaceuticals, Calliditas Therapeutics, Merck, Novartis, Omeros Corporation, and Reata Pharmaceuticals may have programs in clinical development for the treatment of IgAN.

In addition there are several other pre-clinical programs that may enter the clinic for the treatment of IgAN.

CNSA-001

We have the option to purchase Censa, which owns CNSA-001. The current competitive landscape for CNSA-001 includes:

- Two FDA- approved medications for PKU which are marketed by BioMarin Pharmaceuticals, sapropterin dihydrochloride, known as Kuvan® and pegylated recombinant phenylalanine ammonia lyase, known as PALYNZIQ™.
- Various preclinical programs which include gene-therapy projects for PKU.

Liquid Ursodeoxycholic Acid

Also known as ursodiol or UDCA, ursodeoxycholic acid is a naturally occurring hydrophilic bile acid derived from cholesterol, which is indicated for the treatment of PBC and currently prescribed only in solid forms. Introducing a liquid formulation of ursodeoxycholic acid would provide healthcare professionals with a dosing alternative for patients who have difficulty swallowing tablets or capsules, and may facilitate increased compliance. Currently the only liquid form of UDCA is from compounding pharmacies who make it at a physician's request for individual prescriptions.

Our significant recent business development activities include:

Exclusive option to purchase Censa

On December 16, 2017, we entered into a Future Acquisition Right and Joint Development Agreement with Censa, which became effective on January 4, 2018. Pursuant to the agreement, we agreed to fund certain development activities of Censa's CNSA-001 program, in an aggregate amount expected to be approximately \$17 million through proof of concept, and have the right, but not the obligation, to acquire Censa (the "Option") on the terms and subject to the conditions set forth in a separate Agreement and Plan of Merger (the "Merger Agreement"). In exchange for the Option, in January 2018, we paid Censa \$10 million, \$9 million of which was distributed to Censa's equityholders, and paid Censa an additional \$5 million upon Censa's completion of a specified development milestone set forth in the Option Agreement, all of which was distributed to Censa's equityholders.

If we exercise the Option, pursuant to the terms of the Merger Agreement, we will acquire Censa for \$65 million in upfront consideration, subject to certain adjustments, paid as a combination of 20% in cash and 80% in shares of our common stock, valued at a fixed price of \$21.40 per share; provided, however, that Censa may elect on behalf of its equityholders to receive the upfront consideration in 100% cash if the average price per share of our common stock for the ten trading days ending on the date we provide a notice of interest to exercise the Option is less than \$19.26. In addition to the upfront consideration, if we exercise the Option and acquire Censa, we would be required to make further cash payments to Censa's equityholders of up to an aggregate of \$25 million if the CNSA-001 program achieves specified development and commercial milestones.

Licenses and Royalties

Ligand License Agreement

In 2012, we entered into a license agreement with Ligand Pharmaceuticals, Inc. ("Ligand"), granting us a worldwide license for the development, manufacture and commercialization of sparsentan, which we are developing for the treatment of FSGS and IgAN. Under the license agreement, Ligand granted us a sublicense under certain of its patents and other intellectual property in connection with the development and commercialization of sparsentan. Under the license agreement, Ligand is obligated to transfer to us certain information, records, regulatory filings, materials and inventory controlled by Ligand and relating to or useful for developing sparsentan. We must use commercially reasonable efforts to develop and commercialize sparsentan in specified major market countries and other countries in which we believe it is commercially reasonable to develop and commercialize such products.

As consideration for the license, we are required to make payments upon the achievement of certain milestones, totaling up to \$114.1 million. Should we commercialize sparsentan or any products containing any of the licensed compounds, we will be obligated to pay Ligand an escalating annual royalty between 15% and 17% of net sales of all such products. Through 2018, we have made milestone payments to Ligand of \$7.2 million under the terms of the license agreement.

Under the terms of the license agreement, BMS has a right of first negotiation and Ligand has a right of second negotiation with respect to any license arrangement for a licensed compound except to the extent such rights may be waived.

The license agreement will continue until neither party has any further payment obligations under the agreement and is expected to continue for approximately 10 to 20 years from the effective date. Ligand may terminate the license agreement due to (i) our insolvency, (ii) our material uncured breach of the agreement, (iii) our failure to use commercially reasonable efforts to develop and commercialize sparsentan as described above or (iv) certain other conditions. We may terminate the license agreement due to a material uncured breach of the agreement by Ligand.

Thiola License Agreement

In 2014, we entered into a license agreement with Mission Pharmacal Company ("Mission"), pursuant to which we obtained an exclusive, royalty-bearing license to market, sell and commercialize Thiola (Tiopronin) in the United States and Canada, and a non-exclusive license to use know-how relating to Thiola to the extent necessary to market Thiola. We paid Mission an up-front license fee of \$3.0 million and through May 28, 2024 will pay guaranteed minimum royalties during each calendar year the greater of \$2.0 million or 20% of our net sales of Thiola in the United States and Canada.

Amendments to the original license agreement are as follows: In October 2015, the license agreement was amended to allow for us to secure enough active pharmaceutical ingredient ("API") to ensure an adequate level of safety stock to prevent an interruption in the supply of Thiola and to prepare for a reformulation development project. In March 2016, the license agreement was amended to, among other things, include a new formulation development project for tiopronin tablets. In November 2017, the license agreement was amended to extend the license term, at a minimum, through May 2029. In November 2018, the license agreement was amended to extend the definition of territory beyond the United States and Canada.

Intellectual Property

The proprietary nature of, and protection for, our product candidates and our discovery programs, processes and know-how are important to our business. We have sought patent protection in the United States and certain other jurisdictions for sparsentan and fosmetpantotenate, where available and when appropriate. Our policy is to pursue, maintain and defend patent rights, whether developed internally or licensed from third parties, and to protect the technology, inventions and improvements that are commercially important to the development of our business. We also rely on trade secrets relating to our proprietary technology that may be important to the development of our business.

Our commercial success will depend in part on obtaining and maintaining patent protection and trade secret protection for our current and future product candidates and the methods used to develop and manufacture them, as well as successfully defending these patents against third-party challenges. Our ability to stop third parties from making, using, selling, offering to sell, or importing our products depends on the extent to which we have rights under valid and enforceable patents or trade secrets that cover these activities. We cannot be sure that patents will be granted with respect to any of our pending patent applications or with respect to any patent applications filed by us in the future, nor can we be sure that any of our existing patents or any patents that may be granted to us in the future will be commercially useful in protecting our product candidates, discovery programs and processes.

Fosmetpantotenate

Our patent portfolio covering compounds for the treatment of PKAN is comprised of five Retrophin-owned patent families. The first of these patent families includes patents and patent applications directed to fosmetpantotenate and structural analogs thereof, pharmaceutical compositions containing fosmetpantotenate (RE-024) or analogs thereof, and methods of using fosmetpantotenate or analogs thereof in the treatment of PKAN. As of December 31, 2018, this patent family included three U.S. patents (U.S. Patent No. 8,673,883, issued March 18, 2014, which we refer to herein as the '883 patent, U.S. Patent No. 9,181,286, issued November 10, 2015, and U.S. Patent No. 9,629,862, issued April 25, 2017), and one pending U.S. patent application (Application Serial No. 16/204,692, filed November 29, 2018). In addition, as of December 31, 2018 this patent family included two granted European patents (European Patent Nos. EP2841438 and EP3112372, which we refer to herein as the European '438 patent and the European '372 patent, respectively), a granted Chinese patent, a granted Japanese patent, a granted Hong Kong patent, a granted

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Russian patent, and corresponding foreign patent applications pending in Australia, Brazil, Canada, China, Europe, Hong Kong, India, Japan, Korea, Mexico, and Russia. We expect the U.S. and foreign patents in this patent family to expire in April 2033.

Our second PKAN patent family is directed to a chemical genus that encompasses structural analogs of fosmetpantotenate, but not fosmetpantotenate itself. As of December 31, 2018, this patent family was comprised of a granted U.S. patent (U.S. Patent No. 9,896,464, issued February 20, 2018), a pending U.S. patent application (Application Serial No. 15/863,683, filed January 5, 2018) and a granted European patent (European Patent No. EP3060570, issued September 19, 2018). We expect any U.S. and European patents granted from this patent family to expire in October 2034.

Our third PKAN patent family is directed to another chemical genus that encompasses structural analogs of fosmetpantotenate, but not fosmetpantotenate itself. As of December 31, 2018, this patent family was comprised of pending patent applications in the U.S., Australia, Canada, China, Europe, India, Japan, Korea, Mexico and New Zealand.

Our fourth PKAN patent family also is directed to a chemical genus that encompasses structural analogs of fosmetpantotenate, but not fosmetpantotenate itself. As of December 31, 2018, this patent family was comprised of an international patent application filed in 2017.

Our fifth PKAN patent family is directed to a pharmaceutical formulation of fosmetpantotenate. As of December 31, 2018, this patent family was comprised of an international patent application filed in 2018.

It is possible, assuming that fosmetpantotenate achieves regulatory approval and depending upon the date of any such approval, that the term of the '883 patent may be extended under the provisions of the Hatch-Waxman Act. Patent term extension also may be available in certain foreign jurisdictions upon regulatory approval. Likewise, it is possible, assuming that fosmetpantotenate achieves regulatory approval in Europe and depending upon the date of any such approval, that the term of the European '438 patent or the term of the European '372 patent may be extended in various European countries under the provisions governing Supplementary Protection Certificates (SPCs). Should we commercialize fosmetpantotenate, we may be obligated to pay royalties of up to 5% of net sales of all such products.

Sparsentan

Our patent portfolio for sparsentan is comprised of three distinct patent families, two of which are exclusively licensed from Ligand. One of the licensed patent families is owned by BMS, which exclusively licensed it to Ligand (the "BMS patent family"), and the other is owned by Ligand (the "Ligand patent family"). The third and fourth patent families are owned by Retrophin (the "Retrophin patent family").

The BMS patent family is directed to sparsentan and structural analogs thereof, and to pharmaceutical compositions containing sparsentan or a structural analog thereof. As of December 31, 2018, this patent family included three U.S. patents (U.S. Patent Nos. 6,638,937, which we refer to herein as the '937 patent; 6,835,741; and 6,852,745), of which one (U.S. Patent No. 6,638,937) claims sparsentan and pharmaceutical compositions that contain sparsentan. In addition, as of December 31, 2018, this patent family included a granted European patent and a granted Chinese patent. With the exception of the '937 patent, which the U.S. Patent and Trade Office ("USPTO") has determined is entitled to 175 days of patent term adjustment, we expect all U.S. and foreign patents in this patent family to expire in July 2019. In view of the USPTO determination that the '937 patent is entitled to 175 days of patent term adjustment, we expect the '937 patent to expire in December 2019.

The Ligand patent family is directed to methods of using sparsentan in the treatment of various diseases, including glomerulosclerosis and IgAN. As of December 31, 2018, this patent family included two U.S. patents (U.S. Patent No. No. 9,662,312, which we refer to herein as the '312 patent, and U.S. Patent No. 9,993,461, which we refer to herein as the '461 patent), a pending U.S. application (Application Serial No. 15/938,956, filed March 28, 2018), a European patent (European Patent No. EP2732818, which we refer to herein as the European '818 patent), a pending European application, and a granted Hong Kong patent. The '312 patent and the European '818 patent claim the use of sparsentan for treating glomerulosclerosis. The '461 patent claims both the use of sparsentan for treating glomerulosclerosis and the use of sparsentan for treating IgAN. We expect the U.S. and foreign patents in this patent family to expire in March 2030.

The first Retrophin patent family consists of an international patent application (i.e., a PCT application) filed in 2017, and our second Retrophin patent family consists of a provisional patent application filed in 2018.

It is possible, assuming that sparsentan achieves regulatory approval and depending upon the date of any such approval, that the term of either the '312 patent or the '461 patent may be extended under the provisions of the Hatch-Waxman Act. Patent term extension also may be available in certain foreign jurisdictions upon regulatory approval. Likewise, it is possible, assuming that sparsentan achieves regulatory approval in Europe and depending upon the date of any such approval, that the term of the European '818 patent may be extended in various European countries under the provisions governing Supplementary Protection Certificates (SPCs).

Thiola

Our patent portfolio for Thiola is comprised of a patent family which is exclusively licensed from Mission Pharmacal (the "Mission patent family"). The Mission patent family is directed to a new formulation of Thiola. As of December 31, 2018, this patent family included a U.S. patent application filed in 2018.

Regulatory Exclusivity

If we obtain marketing approval for sparsentan, fosmetpantotenate, CNSA-001 or other drug candidates in the United States or in certain jurisdictions outside of the United States, we may be eligible for regulatory exclusivity for the approved drug. For example, in the United States, an FDA approved drug product may be eligible to receive five years of new chemical entity exclusivity or, for drugs granted an orphan designation by the FDA, seven years of orphan drug exclusivity. In Europe a new drug product approved by the EMA may receive eight years of data exclusivity and up to 11 years of marketing exclusivity or, in the case of orphan drugs, ten years of data exclusivity. There can be no assurance that we will qualify for any such regulatory exclusivity, or that any such exclusivity will prevent competitors from seeking approval solely on the basis of their own studies. See "Government Regulation" below.

Chenodal

Chenodal received orphan drug designation in the United States for the treatment of CTX in 2010. Consequently, if Chenodal is the first chenodeoxycholic acid product to gain FDA approval for the treatment of CTX, we expect it will have 7 years of marketing exclusivity in the United States for that indication. Currently Chenodal does not have regulatory exclusivity in the United States.

Cholbam (Kolbam)

Cholbam received orphan drug designation in the United States for the treatment for pediatric and adult patients with bile acid synthesis disorders due to single enzyme defects and for patients with peroxisomal disorders, and therefore is expected to have marketing exclusivity in the U.S. for these indications until March 2022.

Kolbam, the branded name of Cholbam in Europe, received marketing authorization in November 2015 from the EMA for the treatment of inborn errors of primary bile acid synthesis, encompassing select single enzyme defects. We expect Kolbam to have marketing exclusivity in Europe for these indications until September 2024.

Thiola

Thiola does not have regulatory exclusivity in the United States.

Trademarks

Our trademark portfolio includes both Retrophin-owned and Retrophin-licensed trademarks and is comprised of various U.S. and foreign registered trademarks and pending trademark applications relating to our company name, our commercial products (Thiola, Chenodal and Cholbam/Kolbam), and two of our product candidates (i.e. sparsentan and L-UDCA).

More specifically, as of December 31, 2018, our trademark portfolio included registered U.S. and foreign trademarks for the mark "RETROPHIN", a registered U.S. trademark for the Retrophin logo, one registered U.S. trademark and one registered Canadian trademark for the mark "CHENODAL", one registered U.S. trademark directed to the Chenodal logo, one registered U.S. trademark for the mark "MANCHESTER PHARMACEUTICALS", one U.S. trademark application for the mark "KEEP IT BELOW THE LINE", registered U.S. and foreign trademarks for the mark "CHOLBAM", a registered European Community trademark for the mark "KOLBAM", a registered U.S. trademark for the mark "TOTAL CARE HUB", a registered U.S. trademark for the Total Care Hub logo, a registered U.S. trademark for a leaves logo, U.S. trademark applications and foreign registered trademarks related to sparsentan, and U.S. trademark applications relating to L-UDCA. In addition, under our license agreement with Mission we have an exclusive license to use Mission's trademarks related to Thiola, including three registered U.S. trademarks and one registered Canadian trademark for the mark "THIOLA", and several U.S. trademark applications.

Trade Secrets

In addition to patents, we rely on trade secrets and know-how to develop and maintain our competitive position. We seek to protect our proprietary data and processes, in part, by confidentiality agreements and invention assignment agreements with our employees, consultants, scientific advisors, contractors, and partners. These agreements are designed to protect our proprietary information. We also seek to preserve the integrity and confidentiality of our data, trade secrets and know-how by maintaining physical security of our premises and physical and electronic security of our information technology systems. Trade secrets and know-how can be difficult to protect. Consequently, we anticipate that trade secrets and know-how will, over time, be disseminated within the industry through independent development, the publication of journal articles, and the movement of personnel skilled in the art from academic to industry scientific positions.

Manufacturing

Nexgen Pharma manufactures Chenodal, New Zealand Pharma manufactures the active pharmaceutical ingredient for Cholbam, Patheon Inc. formulates and packages Cholbam, and Mission manufactures Thiola.

We intend to continue to use our financial resources to accelerate development of our drug candidates rather than establishing our own manufacturing facilities. We intend to meet our pre-clinical and clinical trial manufacturing requirements by establishing relationships with third-party manufacturers and other service providers to perform these services for us.

Should any of our drug candidates obtain marketing approval, we anticipate establishing relationships with third-party manufacturers and other service providers in connection with the commercial production of our products. We have some flexibility in securing other manufacturers to produce

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our drug candidates; however, our alternatives may be limited due to proprietary technologies or methods used in the manufacture of some of our drug candidates.

Sales, Marketing and Distribution

During fiscal 2018, we continued to utilize our specialty sales force to market our products. In order to commercialize our clinical drug candidates if and when they are approved for sale in the United States or elsewhere, we will need to increase our marketing, sales and distribution capabilities.

Commercialization

Through deep understanding of patient and healthcare provider needs, we believe we are able to:

- serve patients living with rare disease that have limited treatment options;
- drive optimum performance of our marketed products;
- educate and train healthcare providers about our products and the diseases for which they are approved to treat;
- support access to and reimbursement coverage for our products in the U.S. without significant restrictions; and
- minimize the number of patients who discontinue treatment or have low compliance with our products by providing patients with support services and disease education, to the extent and in the manner permitted under applicable laws, to help them maximize the benefits of treatment.

Our U.S. commercial initiatives are designed to support patients living with rare diseases and clinicians treating these patients. We believe that it is possible to commercialize our products in the U.S. with a relatively small specialty sales force. The primary call points for Thiola include urologists and nephrologists. The primary call points for Cholbam are gastroenterologists, hepatologists, and metabolic specialists. We do not promote Chenodal using our sales force.

Our sales force is differentiated by its high level of experience, averaging more than 15 years in pharmaceutical sales including over five years of experience in rare disease. Our commercial management and operations team has an average of more than 15 years of pharmaceutical experience focused on specialty and rare disease.

Our small marketing team, supported by third-party agencies with rare disease experience, drives our commercialization and disease awareness efforts in the U.S. and countries where our products may be approved or available through named patient sales. Specifically, we implement a variety of marketing programs to educate physicians, including direct-to-physician contact by sales representatives, peer-to-peer educational programs, and participation in targeted medical convention programs.

We distribute our products through one direct to patient pharmacy, Eversana (formerly Dohmen Life Science Services), who also provides our comprehensive patient support services (i.e., the Total Care Hub). This patient support program (for all U.S. commercial products) includes a case-managed approach to patient education, insurance verification and reimbursement support, co-pay and other financial assistance for eligible patients, monitoring and support of adherence, and 24/7 access to pharmacist counseling.

Outside the U.S., including in the EU, we plan to continue to partner with local distributors and certain field-based personnel as necessary to conduct permitted commercial activities. Our near-term efforts are focused on securing pricing and reimbursement approval for Kolbam.

Medical Affairs

We have a medical affairs team in the U.S. which supports independent medical education programs and investigator-initiated studies by providing education and financial grants in a number of medical and disease-related areas. The responsibilities of medical affairs personnel also include providing education through the dissemination of medical information and publications, providing support in connection with our post-approval clinical commitments, and assisting in organizing scientific and medical advisory boards to obtain input from experts and practitioners on medical topics relevant to our products and diseases.

Government Regulation

Regulation by government authorities in the United States and foreign countries is a significant factor in the development, manufacture and marketing of our proposed products and in our ongoing research and product development activities. All of our products will require regulatory approval by government agencies prior to commercialization. In particular, human therapeutic products are subject to rigorous preclinical studies and clinical trials and other approval procedures of the FDA and similar regulatory authorities in foreign countries. Various federal and state statutes and regulations also govern or influence testing, manufacturing, safety, labeling, storage and record-keeping related to such products and their marketing. The process of obtaining these approvals and the subsequent compliance with appropriate federal and state statutes and regulations require the expenditure of substantial time and financial resources.

FDA Drug Approval Process

In the United States, pharmaceutical products are subject to extensive regulation by the FDA. The Federal Food, Drug, and Cosmetic Act, and other federal and state statutes and regulations, govern, among other things, the research, development, testing, manufacture, storage, recordkeeping, approval, labeling, promotion and marketing, distribution, post-approval monitoring and reporting, sampling and import and export of pharmaceutical

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products. Failure to comply with applicable U.S. requirements may subject a company to a variety of administrative or judicial sanctions, such as FDA refusal to approve pending new drug applications, or NDAs, warning or untitled letters, product recalls, product seizures, total or partial suspension of production or distribution, injunctions, fines, civil penalties and criminal prosecution.

We cannot market a drug product candidate in the United States until the drug has received FDA approval. The steps required before a drug may be marketed in the United States generally include the following:

- completion of extensive pre-clinical laboratory tests, animal studies, and formulation studies in accordance with the FDA's GLP regulations;
- submission to the FDA of an IND for human clinical testing, which must become effective before human clinical trials may begin;
- performance of adequate and well-controlled human clinical trials in accordance with Good Clinical Practices ("GCP") requirements to establish the safety and efficacy of the drug for each proposed indication;
- submission to the FDA of an NDA after completion of all pivotal clinical trials;
- satisfactory completion of an FDA pre-approval inspection of the manufacturing facility or facilities at which the active pharmaceutical ingredient, or API, and finished drug product are produced and tested to assess compliance with current Good Manufacturing Practices ("cGMPs"); and
- FDA review and approval of the NDA prior to any commercial marketing or sale of the drug in the United States.

Satisfaction of FDA pre-market approval requirements typically takes many years and the actual time required may vary substantially based upon the type, complexity and novelty of the product or disease.

Preclinical tests include laboratory evaluation of product chemistry, formulation and toxicity, as well as animal trials to assess the characteristics and potential safety and efficacy of the product. The conduct of the preclinical tests must comply with federal regulations and requirements, including good laboratory practices. The results of preclinical testing are submitted to the FDA as part of an IND along with other information, including information about product chemistry, manufacturing and controls and a proposed clinical trial protocol. Long term preclinical tests, such as animal tests of reproductive toxicity and carcinogenicity, may continue after the IND is submitted.

A 30-day waiting period after the submission of each IND is required prior to the commencement of clinical testing in humans. If the FDA has neither commented on nor questioned the IND within this 30-day period, the clinical trial proposed in the IND may begin. If the FDA raises concerns or questions about the conduct of the trial, such as whether human research subjects will be exposed to an unreasonable health risk, the IND sponsor and the FDA must resolve any outstanding FDA concerns or questions before clinical trials can proceed.

Clinical trials involve the administration of the investigational new drug to healthy volunteers or patients under the supervision of a qualified investigator. Clinical trials must be conducted in compliance with federal regulations, including GCP requirements, as well as under protocols detailing the objectives of the trial, the parameters to be used in monitoring safety and the effectiveness criteria to be evaluated. Each protocol and subsequent protocol amendments must be submitted to the FDA as part of the IND.

The FDA may order the temporary, or permanent, discontinuation of a clinical trial at any time, or impose other sanctions, if it believes that the clinical trial either is not being conducted in accordance with FDA requirements or presents an unacceptable risk to the clinical trial patients. The study protocol and informed consent information for patients in clinical trials must also be submitted to an institutional review board, or IRB, for approval at each site at which the clinical trial will be conducted. An IRB may also require the clinical trial at the site to be halted, either temporarily or permanently, for failure to comply with the IRB's requirements, or may impose other conditions.

Clinical trials to support NDAs for marketing approval are typically conducted in three sequential phases, but the phases may overlap. In Phase 1, the initial introduction of the drug into healthy human subjects or patients, the drug is tested to assess pharmacological actions, side effects associated with increasing doses and, if possible, early evidence on effectiveness. Phase 2 usually involves trials in a limited patient population to determine metabolism, pharmacokinetics, the effectiveness of the drug for a particular indication, dosage tolerance and optimum dosage, and to identify common adverse effects and safety risks. If a compound demonstrates evidence of effectiveness and an acceptable safety profile in Phase 2 evaluations, Phase 3 clinical trials, also called pivotal trials, are undertaken to obtain the additional information about clinical efficacy and safety in a larger number of patients, typically at geographically dispersed clinical trial sites, to permit the FDA to evaluate the overall benefit-risk relationship of the drug and to provide adequate information for the labeling of the drug. In most cases the FDA requires two adequate and well controlled Phase 3 clinical trials to demonstrate the efficacy of the drug. A single Phase 3 clinical trial with other confirmatory evidence may be sufficient in rare instances where the study is a large multicenter trial demonstrating internal consistency and a statistically very persuasive finding of a clinically meaningful effect on mortality, irreversible morbidity or prevention of a disease with a potentially serious outcome and confirmation of the result in a second trial would be practically or ethically impossible. A sponsor may choose to pursue a Special Protocol Assessment, or SPA, agreement with FDA for the design of a Phase 3 trial. A SPA agreement is intended to provide assurance that if the agreed upon clinical trial protocols are followed and the clinical trial endpoints are achieved, the data may serve as the primary basis for an efficacy claim in support of an NDA. An SPA agreement is not binding on the FDA if previously unrecognized public health concerns arise during the performance of the clinical trial, if other new scientific concerns regarding product candidate safety or efficacy arise or if the sponsoring company fails to comply with the agreed upon clinical trial protocols.

After completion of the required clinical testing, an NDA is prepared and submitted to the FDA. FDA approval of the NDA is required before marketing of the product may begin in the United States. The NDA must include the results of all preclinical, clinical and other testing and a

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compilation of data relating to the product's pharmacology, chemistry, manufacture and controls. The cost of preparing and submitting an NDA is substantial. The submission of most NDAs is additionally subject to a substantial application user fee, and the sponsor of an approved NDA is also subject to an annual program user fee. These fees are typically increased annually.

The FDA has 60 days from its receipt of an NDA to determine whether the application will be accepted for filing based on the agency's threshold determination that it is sufficiently complete to permit substantive review. Once the submission is accepted for filing, the FDA begins an in-depth review. The FDA has agreed to certain performance goals in the review of NDAs. Most such applications for standard review drug products are reviewed within 10 months of filing; most applications for priority review drugs are reviewed within eight months of filing. Priority review can be applied to drugs to treat serious conditions that the FDA determines offer significant improvement in safety or effectiveness. The review process for both standard and priority review may be extended by the FDA for three additional months to consider certain late-submitted information, or information intended to clarify information already provided in the submission.

The FDA may also refer applications for novel drug products, or drug products that present difficult questions of safety or efficacy, to an advisory committee—typically a panel that includes clinicians and other experts—for review, evaluation and a recommendation as to whether the application should be approved. The FDA is not bound by the recommendation of an advisory committee, but it generally follows such recommendations. Before approving an NDA, the FDA will typically inspect one or more clinical sites to assure compliance with GCPs. Additionally, the FDA will inspect the facility or the facilities at which the drug is manufactured. The FDA will not approve the product unless compliance with cGMPs is satisfactory and the NDA contains data that provide substantial evidence that the drug is safe and effective in the indication studied.

After the FDA evaluates the NDA and the manufacturing facilities, it issues either an approval letter or a complete response letter. A complete response letter generally outlines the deficiencies in the submission and may require substantial additional testing, or information, in order for the FDA to reconsider the application. If, or when, those deficiencies have been addressed to the FDA's satisfaction in a resubmission of the NDA, the FDA will issue an approval letter. The FDA has committed to reviewing such resubmissions in two or six months depending on the type of information included.

An approval letter authorizes commercial marketing of the drug with specific prescribing information for specific indications. As a condition of NDA approval, the FDA may require risk evaluation and mitigation strategies ("REMS") to ensure that the benefits of the drug outweigh the potential risks. REMS can include a medication guide, a communication plan for healthcare professionals and elements to assure safe use, such as special training and certification requirements for individuals who prescribe or dispense the drug, requirements that patients enroll in a registry and other measures that the FDA deems necessary to assure the safe use of the drug. The requirement for REMS can materially affect the potential market and profitability of the drug. Moreover, product approval may require substantial post-approval testing and surveillance to monitor the drug's safety or efficacy. Once granted, product approvals may be withdrawn if compliance with regulatory standards is not maintained or problems are identified following initial marketing.

Changes to some of the conditions established in an approved application, including changes in indications, labeling, or manufacturing processes or facilities, require submission and FDA approval of a new NDA or NDA supplement before the change can be implemented. An NDA supplement for a new indication typically requires clinical data similar to that in the original application, and the FDA uses the same procedures and actions in reviewing NDA supplements as it does in reviewing NDAs. Such supplements are typically reviewed within 10 months of receipt.

Orphan Drugs

Under the Orphan Drug Act, the FDA may grant orphan drug designation to drugs intended to treat a rare disease or condition—generally a disease or condition that affects fewer than 200,000 individuals in the U.S. Orphan drug designation must be requested before submitting an NDA. After the FDA confers orphan drug status, the generic identity of the drug and its potential orphan indication are disclosed publicly by the FDA. Orphan drug designation in and of itself does not convey any advantage in, or shorten the duration of, the regulatory review and approval process. The first NDA applicant to receive FDA approval for a particular active ingredient to treat a particular indication with FDA orphan drug designation is entitled to a seven-year exclusive marketing period in the U.S. for that product, for that indication. During the seven-year exclusivity period, the FDA may not approve any other applications to market the same drug for the same disease, except in limited circumstances, such as a showing of clinical superiority to the product with orphan drug exclusivity. Orphan drug exclusivity does not prevent FDA from approving a different drug for the same disease or condition, or the same drug for a different disease or condition. Prior to FDA approval, orphan designation provides incentives for sponsors including tax credits for clinical research expenses, the opportunity to obtain government grant funding to support clinical research, and an exemption from FDA user fees.

Fast Track Designation

Fast track is a process designed by the FDA to facilitate the development of drugs to treat serious conditions through expediting their review. The purpose is to get important new drugs to patients earlier. Fast Track addresses a broad range of serious conditions. Determining whether a condition is serious is a matter of judgment, but generally is based on whether the drug will have an impact on such factors as survival, day-to-day functioning, or the likelihood that the condition, if left untreated, will progress from a less severe condition to a more serious one.

A drug that receives Fast Track designation is eligible for some or all of the following:

- more frequent meetings with FDA to discuss the drug's development plan and ensure collection of appropriate data needed to support drug approval;
- more frequent written communication from FDA about such things as the design of the proposed clinical trials and use of biomarkers;
- eligibility for Accelerated Approval and Priority Review, if relevant criteria are met; and

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- rolling Review, which means that a drug company can submit completed sections of its Biologic License Application (BLA) or NDA for review by FDA, rather than waiting until every section is completed before the entire application can be reviewed. BLA or NDA review usually does not begin until the drug company has submitted the entire application to the FDA.

Once a drug receives Fast Track designation, early and frequent communication between the FDA and a drug company is encouraged throughout the entire drug development and review process. The frequency of communication assures that questions and issues are resolved quickly, often leading to earlier drug approval and access by patients.

Accelerated Approval

Under the FDA's Subpart H accelerated approval regulations, FDA may approve a drug for a serious or life-threatening illness that provides meaningful therapeutic benefit to patients over existing treatments based upon a surrogate endpoint that is reasonably likely to predict clinical benefit, or on a clinical endpoint that can be measured earlier than irreversible morbidity or mortality, that is reasonably likely to predict an effect on irreversible morbidity or mortality or other clinical benefit, taking into account the severity, rarity, or prevalence of the condition and the availability or lack of alternative treatments.

In clinical trials, a surrogate endpoint is a measurement of laboratory or clinical signs of a disease or condition that substitutes for a direct measurement of how a patient feels, functions, or survives. Surrogate endpoints can often be measured more easily or more rapidly than clinical endpoints. A drug candidate approved on this basis is subject to rigorous post-marketing compliance requirements, including the completion of Phase 4 or post-approval clinical trials to confirm the effect on the clinical endpoint. Failure to conduct required post-approval studies, or confirm a clinical benefit during post-marketing studies, will allow FDA to withdraw the drug from the market on an expedited basis. All promotional materials for drug candidates approved under accelerated regulations are subject to prior review by FDA.

The Hatch-Waxman Amendments: Orange Book Listing

In seeking approval for a drug through an NDA, applicants are required to list with the FDA each patent whose claims cover the applicant's product. Upon approval of a drug, each of the patents listed in the application for the drug is then published in the FDA's Approved Drug Products with Therapeutic Equivalence Evaluations, commonly known as the Orange Book. Drugs listed in the Orange Book can, in turn, be cited by potential generic competitors in support of approval of an ANDA. An ANDA provides for marketing of a drug product that has the same active ingredients in the same strengths and dosage form as the listed drug and has been shown through bioequivalence testing to be therapeutically equivalent to the listed drug. Other than the requirement for bioequivalence testing, ANDA applicants are not required to conduct, or submit results of, pre-clinical or clinical tests to prove the safety or effectiveness of their drug product. Drugs approved in this way are commonly referred to as "generic equivalents" to the listed drug, and can often be substituted by pharmacists under prescriptions written for the original listed drug.

The ANDA applicant is required to certify to the FDA concerning any patents listed for the approved product in the FDA's Orange Book. Specifically, the applicant must certify that: (i) the required patent information has not been filed; (ii) the listed patent has expired; (iii) the listed patent has not expired, but will expire on a particular date and approval is sought after patent expiration; or (iv) the listed patent is invalid or will not be infringed by the new product. The ANDA applicant may also elect to submit a section viii statement certifying that its proposed ANDA label does not contain (or carves out) any language regarding the patented method-of-use rather than certify to a listed method-of-use patent. If the applicant does not challenge the listed patents, the ANDA application will not be approved until all the listed patents claiming the referenced product have expired.

A certification that the new product will not infringe the already approved product's listed patents, or that such patents are invalid, is called a Paragraph IV certification. If the ANDA applicant has provided a Paragraph IV certification to the FDA, the applicant must also send notice of the Paragraph IV certification to the NDA and patent holders once the ANDA has been accepted for filing by the FDA. The NDA and patent holders may then initiate a patent infringement lawsuit in response to the notice of the Paragraph IV certification. The filing of a patent infringement lawsuit within 45 days of the receipt of a Paragraph IV certification automatically prevents the FDA from approving the ANDA until the earlier of 30 months, expiration of the patent, settlement of the lawsuit, or a decision in the infringement case that is favorable to the ANDA applicant.

The ANDA application also will not be approved until any applicable non-patent exclusivity listed in the Orange Book for the referenced product has expired.

Post-Approval Requirements

Once an NDA is approved, a product will be subject to certain post-approval requirements. For instance, the FDA closely regulates the post-approval marketing and promotion of drugs, including standards and regulations for direct-to-consumer advertising, off-label promotion, industry-sponsored scientific and educational activities and promotional activities involving the internet and social media. Drugs may be marketed only for the approved indications and in accordance with the provisions of the approved labeling.

Adverse event reporting and submission of periodic reports is required following FDA approval of an NDA. The FDA also may require post-marketing testing, known as Phase 4 testing, REMS, surveillance to monitor the effects of an approved product, or restrictions on the distribution or use of the product. In addition, quality-control, drug manufacture, packaging and labeling procedures must continue to conform to cGMPs after approval. Drug manufacturers and certain of their subcontractors are required to register their establishments with the FDA and certain state agencies. Registration with the FDA subjects entities to periodic unannounced inspections by the FDA, during which the agency inspects manufacturing facilities to assess compliance with cGMPs. Accordingly, manufacturers must continue to expend time, money and effort in the areas of production and quality-control to maintain compliance with cGMPs. Later discovery of previously unknown problems with a product, including adverse events of unanticipated severity or frequency, or failure to comply with regulatory requirements, may result in, among other things:

- restrictions on the marketing or manufacturing of the product, complete withdrawal of the product from the market or product recalls;

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- fines, warning letters or holds on post-approval clinical trials;
- refusal of the FDA to approve pending applications or supplements to approved applications, or suspension or revocation of product approvals;
- product seizure or detention, or refusal to permit the import or export of products; or
- injunctions or the imposition of civil or criminal penalties.

Pricing and Reimbursement

A portion of our end-user demand for our drugs comes from patients covered under Medicaid, Medicare and other federal and state government-related programs such as TRICARE and the Department of Veterans Affairs, or the VA. As required by Federal regulations, we will provide rebates and discounts in connection with these programs.

Our commercial success depends in significant part on the extent to which coverage and adequate reimbursement for these products will be available from third-party payers, including government health administration authorities, private health insurers and other organizations. Third-party payers determine which medications they will cover and establish reimbursement levels. Even if a third-party payer covers a particular product, the resulting reimbursement payment rates may not be adequate or may require co-payments that patients find unacceptably high. Patients who are prescribed medications for the treatment of their conditions, and their prescribing physicians, generally rely on third-party payers to reimburse all or part of the costs associated with their prescription drugs. Patients are unlikely to use our products unless coverage is provided and reimbursement is adequate to cover all or a significant portion of the cost of our products. Therefore, coverage and adequate reimbursement is critical to product acceptance.

Government authorities and other third-party payers are developing increasingly sophisticated methods of controlling healthcare costs, such as by limiting coverage and the amount of reimbursement for particular medications. Increasingly, third-party payers are requiring that drug companies provide them with predetermined discounts from list prices as a condition of coverage, are using restrictive formularies and preferred drug lists to leverage greater discounts in competitive classes, and are challenging the prices charged for medical products. Third party payers also are carefully evaluating the medical necessity and cost-effectiveness of medical products and services, in addition to their safety and efficacy, which may require us to conduct expensive pharmacoeconomic studies in order to demonstrate the medical necessity and cost-effectiveness of our products. Further, no uniform policy requirement for coverage and reimbursement for drug products exists among third-party payers in the United States. Therefore, coverage and reimbursement for drug products can differ significantly from payer to payer. As a result, the coverage determination process is often a time-consuming and costly process that will require us to provide scientific and clinical support for the use of our products to each payer separately, with no assurance that coverage and adequate reimbursement will be applied consistently or obtained in the first instance.

In addition, it is possible that future legislation in the United States and other jurisdictions could be enacted which could potentially impact the coverage and reimbursement rates for our products and also could further impact the levels of discounts and rebates paid to federal and state government entities. Any legislation that impacts these areas could impact, in a significant way, our ability to generate revenues from sales of products that, if successfully developed, we bring to market.

There have been a number of enacted or proposed legislative and regulatory changes affecting the healthcare system and pharmaceutical industry that could affect our commercial success. For example, in March 2010, President Obama signed into law the Patient Protection and Affordable Care Act, as amended by the Health Care and Education Reconciliation Act of 2010, (collectively, the "PPACA") a law intended to, among other things, broaden access to health insurance, reduce or constrain the growth of healthcare spending, enhance remedies against healthcare fraud and abuse, add new transparency requirements for healthcare and health insurance industries, impose new taxes and fees on pharmaceutical and medical device manufacturers and impose additional health policy reforms. Specifically, by way of example, the PPACA revised the definition of "average manufacturer price" for reporting purposes, which could increase the amount of Medicaid drug rebates to states. PPACA also increased the mandated Medicaid rebate from 15.1% to 23.1% of the average manufacturer price, expanded the rebate to Medicaid managed care utilization and increased the types of entities eligible for the federal 340B drug discount program. Some of the provisions of the PPACA have yet to be implemented, and there have been judicial and Congressional challenges to certain aspects of the PPACA, as well as recent efforts by the Trump administration to repeal or replace certain aspects of the PPACA. Since January 2017, President Trump has signed two Executive Orders and other directives designed to delay the implementation of certain provisions of the PPACA or otherwise circumvent some of the requirements for health insurance mandated by the PPACA. Concurrently, Congress has considered legislation that would repeal or repeal and replace all or part of the PPACA. While Congress has not passed comprehensive repeal legislation, two bills affecting the implementation of certain taxes under the PPACA have been signed into law. The Tax Cuts and Jobs Act of 2017 ("Tax Act"), includes a provision which repealed, effective January 1, 2019, the tax-based shared responsibility payment imposed by the ACA on certain individuals who fail to maintain qualifying health coverage for all or part of a year that is commonly referred to as the "individual mandate". On January 22, 2018, President Trump signed a continuing resolution on appropriations for fiscal year 2018 that delayed the implementation of certain PPACA-mandated fees, including the so-called "Cadillac" tax on certain high cost employer-sponsored insurance plans, the annual fee imposed on certain health insurance providers based on market share, and the medical device excise tax on non-exempt medical devices. The Bipartisan Budget Act of 2018, or the BBA, among other things, amended the PPACA, effective January 1, 2019, to increase the discount that is owed by pharmaceutical manufacturers who participate in Medicare Part D from 50 percent to 70 percent, and close the coverage gap in most Medicare drug plans, commonly referred to as the "donut hole." In July 2018, the Centers for Medicare & Medicaid Services ("CMS") published a final rule permitting further collections and payments to and from certain PPACA-qualified health plans and health insurance issuers under the PPACA risk adjustment program in response to the outcome of federal district court litigation regarding the method CMS uses to determine this risk adjustment. On December 14, 2018, a Texas U.S. District Court Judge ruled that the PPACA is unconstitutional in its entirety because the "individual mandate" was repealed by Congress as part of the Tax Act. The Texas U.S. District

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Court Judge, as well as the Trump administration and CMS, have stated that the ruling will have no immediate effect pending appeal of the decision. Congress also could consider subsequent legislation to repeal or repeal and replace other elements of the PPACA.

In addition, other legislative changes have been proposed and adopted since the PPACA was enacted. For example, in August 2011, the President signed into law the Budget Control Act of 2011, which, among other things, created the Joint Select Committee on Deficit Reduction to recommend to Congress proposals in spending reductions. The Joint Select Committee on Deficit Reduction did not achieve a targeted deficit reduction of at least \$1.2 trillion for fiscal years 2012 through 2021, triggering the legislation's automatic reduction to several government programs. This includes aggregate reductions to Medicare payments to providers of up to 2% per fiscal year, which went into effect beginning on April 1, 2013 and, due to subsequent legislative amendments, including the BBA, will stay in effect through 2027 unless additional Congressional action is taken. Additionally, in January 2013, the American Taxpayer Relief Act of 2012 was signed into law, which, among other things, reduced Medicare payments to several providers, including hospitals and imaging centers.

Moreover, the Drug Supply Chain Security Act imposes additional obligations on manufacturers of pharmaceutical products related to product tracking and tracing. Legislative and regulatory proposals have been made to expand post-approval requirements and restrict sales and promotional activities for pharmaceutical products.

There has been increasing legislative and enforcement interest in the United States with respect to drug pricing practices. Specifically, there have been several recent U.S. Congressional inquiries and numerous proposed and enacted legislation at both the state and federal levels designed to, among other things, bring more transparency to drug pricing, reduce the cost of prescription drugs under Medicare, expedite generic competition, review the relationship between pricing and manufacturer patient programs, institute drug re-importation, and reform government program reimbursement methodologies for drugs. At the federal level, the Trump administration's budget proposal for fiscal year 2019 contains further drug price control measures that could be enacted during the 2019 budget process or in other future legislation, including, for example, measures to permit Medicare Part D plans to negotiate the price of certain drugs under Medicare Part B, to allow some states to negotiate drug prices under Medicaid, and to eliminate cost sharing for generic drugs for low-income patients. Additionally, the Trump administration released a "Blueprint" to lower drug prices and reduce out of pocket costs of drugs that contains additional proposals to increase manufacturer competition, increase the negotiating power of certain federal healthcare programs, incentivize manufacturers to lower the list price of their products and reduce the out of pocket costs of drug products paid by consumers. The HHS, has already started the process of soliciting feedback on some of these measures and, at the same, is immediately implementing others under its existing authority. For example, in September 2018, CMS announced that it will allow Medicare Advantage Plans the option to use step therapy for Part B drugs beginning January 1, 2019, and in October 2018, HHS through CMS proposed a new rule that would require direct-to-consumer television advertisements of prescription drugs and biological products, for which payment is available through or under Medicare or Medicaid, to include in the advertisement the Wholesale Acquisition Cost, or list price, of that drug or biological product. On January 31, 2019, the HHS Office of Inspector General proposed modifications to federal Anti-Kickback Statute safe harbors which, among other things, will affect rebates paid by manufacturers to Medicare Part D plans, the purpose of which is to further reduce the cost of drug products to consumers. While some of these and other proposed measures may require authorization through additional legislation to become effective, Congress and the Trump administration have each indicated that it will continue to seek new legislative and/or administrative measures to control drug costs. At the state level, legislatures are increasingly 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.

We expect that the PPACA, as well as other federal and state healthcare reform measures that have been and may be adopted in the future, may result in more rigorous coverage criteria and in additional downward pressure on the price that we receive for any of our products, and could seriously harm our future revenues. In addition, it is possible that future legislation in the United States and other jurisdictions could be enacted which could potentially impact the reimbursement rates for the products we are developing and may develop in the future and also could further impact the levels of discounts and rebates paid to federal and state government entities. Any legislation that impacts these areas could impact, in a significant way, our ability to generate revenues from sales of products that, if successfully developed, we bring to market.

Health Care Regulatory Laws

In addition to FDA marketing restrictions and regulation of pharmaceutical products, several other types of state and federal laws have been applied to restrict and regulate certain business practices in the pharmaceutical industry in recent years. These laws include, without limitation, anti-kickback statutes and false claims laws, data privacy and security laws, and transparency laws regarding payments or other items of value provided to healthcare providers.

The federal healthcare program anti-kickback statute prohibits, among other things, knowingly and willfully offering, paying, soliciting or receiving remuneration to induce; or in return for; purchasing, leasing, ordering or arranging for the purchase, lease or order of any healthcare item or service reimbursable under Medicare, Medicaid, or other federally financed healthcare programs. This statute has been interpreted broadly to apply to arrangements between pharmaceutical manufacturers on the one hand and prescribers, purchasers, and formulary managers, among others, on the other. Although there are a number of statutory exceptions and regulatory safe harbors protecting certain common activities from prosecution or other regulatory sanctions, the exceptions and safe harbors are drawn narrowly, and practices that involve remuneration that may induce prescribing, purchases, or recommendations may be subject to scrutiny if they do not qualify for an exemption or safe harbor. Failure to meet all of the requirements of a particular applicable statutory exception or regulatory safe harbor does not make the conduct per se illegal under the anti-kickback statute. Instead, the legality of the arrangement will be evaluated on a case-by-case basis based on a cumulative review of all its facts and circumstances. Several courts have interpreted the statute's intent requirement to mean that if any one purpose of an arrangement involving remuneration is to induce referrals of federal healthcare covered business, the federal anti-kickback statute has been violated. Additionally, the

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PPACA amended the federal anti-kickback statute to a stricter standard such that a person or entity no longer needs to have actual knowledge of the statute or specific intent to violate it in order to have committed a violation. In addition, the PPACA codified case law that a claim including items or services resulting from a violation of the federal anti-kickback statute constitutes a false or fraudulent claim for purposes of the federal civil False Claims Act.

Federal false claims laws, including the civil False Claims Act, prohibit any person or entity from knowingly presenting, or causing to be presented, a false claim for payment to the federal government, or knowingly making, or causing to be made, a false statement to have a false claim paid. This includes claims made to programs where the federal government reimburses, such as Medicaid, as well as programs where the federal government is a direct purchaser, such as when it purchases off the Federal Supply Schedule. The False Claims Act contains qui tam provisions, which allow a private individual, or relator, to bring a civil action on behalf of the federal government alleging that the defendant submitted a false claim to the federal government, and to share in any monetary recovery. In recent years, the number of suits brought by private individuals has increased dramatically. For example, pharmaceutical and other healthcare companies have been prosecuted under these laws for allegedly inflating drug prices they report to pricing services, which in turn were used by the government to set Medicare and Medicaid reimbursement rates, and for allegedly providing free product to customers with the expectation that the customers would bill federal programs for the product. In addition, certain marketing practices, including off-label promotion, may also violate federal false claims laws.

Also, many states have similar fraud and abuse statutes or regulations, including state anti-kickback and false claims laws, that apply to items and services reimbursed under Medicaid and other state programs, or, in several states, apply regardless of the payer.

The U.S. Foreign Corrupt Practices Act, and similar worldwide anti-bribery laws generally prohibit companies and their intermediaries from making improper payments to government officials for the purpose of obtaining or retaining business. Our policies mandate compliance with these anti-bribery laws. We operate in parts of the world that have experienced governmental corruption to some degree and in certain circumstances, strict compliance with antibribery laws may conflict with local customs and practices or may require us to interact with doctors and hospitals, some of which may be state controlled, in a manner that is different than in the United States. We cannot assure you that our internal control policies and procedures will protect us from reckless or criminal acts committed by our employees or agents. Violations of these laws, or allegations of such violations, could disrupt our business and result in criminal or civil penalties or remedial measures, any of which could have a material adverse effect on our business, financial condition and results of operations and could cause the market value of our common stock to decline.

In addition, we may be subject to data privacy and security regulation by both the federal government and the states in which we conduct our business. HIPAA, as amended by the Health Information Technology for Economic and Clinical Health Act, or HITECH, and their respective implementing regulations, imposes specified requirements relating to the privacy, security and transmission of individually identifiable health information. Among other things, HITECH, through its implementing regulations, makes certain of HIPAA's privacy and security standards directly applicable to business associates, defined as a person or organization, other than a member of a covered entity's workforce, that creates, receives, maintains or transmits protected health information for or on behalf of a covered entity for a function or activity regulated by HIPAA.

Additionally, the federal Physician Payments Sunshine Act within the PPACA, and its implementing regulations, require that certain manufacturers of drugs, devices, biologicals and medical supplies for which payment is available under Medicare, Medicaid or the Children's Health Insurance Program (with certain exceptions) to annually report information related to certain payments or other transfers of value made or distributed to physicians and teaching hospitals, or to entities or individuals at the request of, or designated on behalf of, physicians and teaching hospitals and to report annually certain ownership and investment interests held by physicians and their immediate family members. We implemented compliance with the Sunshine Act starting with reporting year 2014 and continue to report as required.

Further, certain states require implementation of commercial compliance programs and marketing codes, compliance with the pharmaceutical industry's voluntary compliance guidelines, and compliance with the applicable compliance guidance promulgated by the federal government. Other various state level requirements include restricting payments or the provision of other items of value that may be made to healthcare providers and other potential referral sources; restricting various marketing practices; requiring prescription drug companies to report expenses relating to the marketing and promotion of drug products; requiring the posting of information relating to clinical studies and their outcomes; requiring the registration of sales representatives; requiring the reporting of certain information related to drug pricing; and requiring drug manufacturers to track and report information related to payments, gifts, compensation, and other items of value to physicians and other healthcare providers. Additionally, states that have not implemented these types of regulations are considering similar proposals. Compliance with these laws is difficult and time consuming, and companies that do not comply with these state laws face civil penalties.

If our operations are found to be in violation of any of the health regulatory laws described above or any other laws that apply to us, we may be subject to significant penalties, including imprisonment, criminal fines, civil monetary penalties, administrative penalties, disgorgement, exclusion from participation in federal healthcare programs, contractual damages, injunctions, recall or seizure of products, total or partial suspension of production, reputational harm, administrative burdens, additional oversight and reporting obligations if we become subject to a corporate integrity agreement or similar agreement to resolve allegation of non-compliance with these laws, diminished profits and future earnings, and the curtailment or restructuring of our operations, any of which could adversely affect our ability to operate our business and our results of operations.

Foreign Regulation

In addition to regulations in the United States, we will be subject to a variety of foreign regulations governing clinical trials and commercial sales and distribution of our products. Whether or not we obtain FDA approval for a product, we must obtain approval by the comparable regulatory authorities of foreign countries before we can commence clinical trials and approval of foreign countries or economic areas, such as the European Union, before we may market products in those countries or areas. The approval process and requirements governing the conduct of clinical trials, product licensing, pricing and reimbursement vary greatly from place to place, and the time may be longer or shorter than that required for FDA approval.

Other Laws and Regulatory Processes

We are subject to a variety of financial disclosure and securities trading regulations as a public company in the United States, including laws relating to the oversight activities of the Securities and Exchange Commission ("SEC"), and Nasdaq rules under which our stock is listed. In addition, the Financial Accounting Standards Board ("FASB"), the SEC, and other bodies that have jurisdiction over the form and content of our accounts, our financial statements and other public disclosures are constantly considering and interpreting proposals and existing pronouncements designed to ensure that companies best display relevant and transparent information relating to their respective businesses.

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 used in connection with our research 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 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.

Employees

As of January 31, 2019, we had 214 full-time employees.

Available Information

We were incorporated in the state of Delaware in February 2011. Our website address is www.retrophin.com. We post links on our website to the following filings as soon as reasonably practicable after they are electronically filed with or furnished to the SEC: annual reports on Form 10-K, quarterly reports on Form 10-Q, current reports on Form 8-K, proxy statements, and any amendments to those reports filed or furnished pursuant to Section 13 or 15(d) of the Securities Exchange Act of 1934, as amended. All such filings are available through our website free of charge. Our filings may also be read and copied at the SEC's Public Reference Room at 100 F Street, NE, Washington, DC 20549. Information on the operation of the Public Reference Room may be obtained by calling the SEC at 1-800-SEC-0330. The SEC also maintains an internet site at www.sec.gov that contains reports, proxy and information statements, and other information regarding issuers that file electronically with the SEC.

ITEM 1A. RISK FACTORS

Our business, as well as an investment in our common stock, is highly speculative in nature and involves a high degree of risk. Our securities should be purchased only by persons who can afford to lose their entire investment. Carefully consider the risks and uncertainties described below together with all of the other information included herein, including the financial statements and related notes, before deciding to invest in our common stock. If any of the following risks actually occur, they could adversely affect our business, prospects, financial condition and results of operations. In such event(s), the market price of our common stock could decline and result in a loss of part or all of your investment. Accordingly, prospective investors should carefully consider, along with other matters referred to herein, the following risk factors in evaluating our business before purchasing any shares of our common stock.

Risks Related to the Development of our Product Candidates

Our clinical trials may fail to demonstrate the safety and efficacy of our product candidates which could prevent or significantly delay their regulatory approval.

Before obtaining regulatory approval for the sale of any of our product candidates, we must subject these product candidates to extensive preclinical and clinical testing to demonstrate their safety and efficacy for humans. Clinical trials are expensive, time-consuming and may take years to complete.

We may experience numerous unforeseen events during, or as a result of, preclinical or nonclinical testing and the clinical trial process that could delay or prevent our ability to obtain regulatory approval or commercialize our product candidates, including:

- our preclinical or nonclinical tests or clinical trials may produce negative or inconclusive results, and we may decide, or regulators may require us, to conduct additional preclinical testing or clinical trials or we may abandon projects that we expect to be promising;
- regulators may require us to conduct studies of the long-term effects associated with the use of our product candidates;
- regulators or institutional review boards may not authorize us to commence a clinical trial or conduct a clinical trial at a prospective trial site;
- the FDA or any non-United States regulatory authority may impose conditions on us regarding the scope or design of our clinical trials or may require us to resubmit our clinical trial protocols to institutional review boards for re-inspection due to changes in the regulatory environment;
- the number of patients required for our clinical trials may be larger than we anticipate or participants may drop out of our clinical trials at a higher rate than we anticipate;

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- our third-party contractors or clinical investigators may fail to comply with regulatory requirements or fail to meet their contractual obligations to us in a timely manner;
- we might have to suspend or terminate one or more of our clinical trials if we, regulators or institutional review boards determine that the participants are being exposed to unacceptable health risks;
- regulators or institutional review boards may require that we hold, suspend or terminate clinical research for various reasons, including noncompliance with regulatory requirements;
- the cost of our clinical trials may be greater than we anticipate;
- the supply or quality of our product candidates or other materials necessary to conduct our clinical trials may be insufficient or inadequate or we may not be able to reach agreements on acceptable terms with prospective clinical research organizations; and
- the effects of our product candidates may not be the desired effects or may include undesirable side effects or the product candidates may have other unexpected characteristics.

These risks and uncertainties impact all of our clinical programs. We will only obtain regulatory approval to commercialize a product candidate if we can demonstrate to the satisfaction of the FDA, and in the case of foreign commercialization, to the applicable foreign regulatory authorities, in well-designed and conducted clinical trials, that our product candidates are safe and effective and otherwise meet the appropriate standards required for approval for a particular indication.

Our product development costs will also increase if we experience delays in testing or approvals. We do not know whether any preclinical tests or clinical trials will be initiated as planned, will need to be restructured or will be completed on schedule, if at all. Significant preclinical or clinical trial delays also could shorten the patent protection period during which we may have the exclusive right to commercialize our product candidates. Such delays could allow our competitors to bring products to market before we do and impair our ability to commercialize our products or product candidates.

If we are required to conduct additional clinical trials or other testing of our product candidates beyond those that we currently contemplate, if we are unable to successfully complete our clinical trials or other testing, if the results of these trials or tests are not positive or are only modestly positive or if there are safety concerns, we may:

- be delayed in obtaining, or may not be able to obtain, marketing approval for one or more of our product candidates;
- obtain approval for indications that are not as broad as intended or entirely different than those indications for which we sought approval; and
- have the product removed from the market after obtaining marketing approval.

Our product candidates are intended to treat PKAN, FSGS and IgAN, each of which is a rare disease. Given that these development candidates are still undergoing required testing, we may not be able to initiate or continue clinical trials if we are unable to locate a sufficient number of eligible patients willing and able to participate in the clinical trials required by the FDA or foreign regulatory agencies. In addition, as other companies and researchers may be concurrently developing therapies for the same or similar indications that we are focused on, we could face competition for a limited number of patients, investigators and clinical trial sites willing to participate in clinical trials. Our inability to enroll a sufficient number of patients for any of our current or future clinical trials would result in significant delays or may require us to abandon one or more clinical trials altogether.

Success in preclinical testing and early clinical trials does not ensure that later clinical trials will be successful, and initial results from a clinical trial do not necessarily predict final results.

Success in preclinical testing and early clinical trials does not ensure that later clinical trials will be successful, and initial results from a clinical trial do not necessarily predict final results. For example, there can be no assurance that the favorable responses we have seen with the physician-initiated treatment of fosmetpantotenate in PKAN patients outside the United States will translate to positive data in the Phase 3 clinical trial of fosmetpantotenate or that the positive results from the DUET study of sparsentan in FSGS will be repeated in the Phase 3 clinical trial. Similarly, there can be no assurance that our clinical experience with sparsentan in FSGS will translate to favorable data in IgAN, which patient population has not previously been treated with sparsentan prior to the Phase 3 trial currently being conducted. We cannot assure that any current or future clinical trials of fosmetpantotenate, sparsentan and/or CNSA-001 will ultimately be successful.

Before obtaining regulatory approval to conduct clinical trials of our product candidates, we must conduct extensive preclinical tests to demonstrate the safety of our product candidates in animals. Preclinical testing is expensive, difficult to design and implement, and can take many years to complete. In addition, during the clinical development process, additional nonclinical toxicology studies are routinely conducted concurrently with the clinical development of a product candidate. If any of our product candidates show unexpected findings in concurrent toxicology studies, we could experience potentially significant delays in, or be required to abandon, development of that product candidate. A failure of one or more of our nonclinical studies can occur at any stage of testing.

Communications and/or feedback from the FDA related to our current or planned future clinical trials does not guarantee any particular outcome from regulatory review for such clinical trials.

Communications and/or feedback from the FDA related to our current or future clinical trials does not guarantee any particular outcome from regulatory review for such clinical trials. For example, although we have obtained a Special Protocol Assessment ("SPA") agreement from the FDA

for the Phase 3 clinical trial of fosmetpantotenate for the treatment of PKAN, this agreement does not guarantee any particular outcome from regulatory review. The SPA is intended to provide assurance that if the agreed upon clinical trial protocols are followed and the clinical trial endpoints are achieved, the data may serve as the primary basis for an efficacy claim in support of an NDA. However, a SPA is not a guarantee of an approval of a product candidate or any permissible claims about the product candidate. In particular, a SPA agreement is not binding on the FDA if previously unrecognized public health concerns arise during the performance of the clinical trial, if other new scientific concerns regarding product candidate safety or efficacy arise or if the sponsoring company fails to comply with the agreed upon clinical trial protocols. Moreover, a SPA does not address all of the variables and details that may go into planning for or conducting a clinical trial, and changes in the protocol for a clinical trial can invalidate a SPA or require that the FDA agree in writing to the modified protocol. In addition, while a SPA addresses the requirements for submission of an NDA, the results of the related clinical trial may not support FDA approval. There can be no assurance that the Phase 3 clinical trial for fosmetpantotenate will demonstrate that fosmetpantotenate is safe and effective for treating PKAN or that the data will support an application for approval by the FDA. In the third quarter of 2019, we plan to un-blind the data from our ongoing pivotal Phase 3 trial of fosmetpantotenate. If the results of this trial are not positive, or are not viewed sufficiently favorably, the market price of our common stock could decline significantly. In addition, following the initial 24 week study period, patients in this trial are eligible to participate in an open label extension. Even if the initial un-blinded results from the trial are viewed positively, it is possible that negative consequences of the treatment could be observed in the future, which could adversely impact the regulatory and commercial prospects of fosmetpantotenate and our business may suffer.

In addition, in 2018, we initiated the following Phase 3 clinical trials of sparsentan: 1) a single Phase 3 clinical trial designed to serve as the basis for an NDA and MAA filing for sparsentan for the treatment of FSGS (the "DUPLEX Study"), and 2) a single Phase 3 clinical trial designed to serve as the basis for an NDA and MAA filing for sparsentan for the treatment of IgAN (the "PROTECT Study"). We are conducting the DUPLEX Study and the PROTECT Study under the Subpart H pathway for potential accelerated approval in the US, and in the EU we plan to pursue potential Conditional Marketing Authorization, in both jurisdictions based on change in proteinuria. Recognition of change in proteinuria as a surrogate endpoint in kidney disease is a relatively new regulatory development, and, as the field continues to evolve, new learnings may impact regulatory viewpoints. We expect that the FDA's and EMA's determination as to whether the sufficiency of the data supports an accelerated approval in either jurisdiction will be made during the application review process. There can be no assurance that even if we achieve statistical significance on the interim or primary endpoints for the DUPLEX Study and/or the PROTECT Study, as applicable, that the FDA or EMA will deem that sufficient to grant accelerated approval or Conditional Marketing Authorization.

Although we received feedback from the FDA at an End of Phase 2 meeting for the sparsentan FSGS program during which the FDA communicated that it was open to accepting a substantial treatment effect on proteinuria in the DUPLEX Study as a basis for accelerated approval pursuant to Subpart H of the FDA regulations and although we subsequently gained alignment that our statistical modeling supported initiating a Phase 3 trial that proceeds on the Subpart H pathway, there can be no guarantee that the data generated from the study will be sufficient to serve as the basis for an NDA filing, including an NDA under Subpart H for accelerated approval. In addition, our statistical modeling that supports proceeding with the Duplex Study on the Subpart H pathway is based on data from other FSGS studies. To the extent that the model population is not representative of the Duplex Study population, the FDA may not agree that the new results continue to support a Subpart H pathway. Furthermore, even if sparsentan is granted accelerated approval for FSGS, there can be no assurance that the post-marketing confirmatory data will support full approval of sparsentan as a treatment for FSGS.

Also, although we have reached agreement with the FDA regarding the initiation of the PROTECT Study and the trial began in December 2018, we continue to have regulatory interactions regarding certain details of the study. There can be no assurance that the study will proceed as planned and there can be no guarantee that the data generated from the study will be sufficient to serve as the basis for an NDA filing, including an NDA under Subpart H for accelerated approval or support Conditional Marketing Authorization in the EU. Furthermore, even if sparsentan is granted accelerated approval for IgAN, there can be no assurance that the post-marketing confirmatory data will support full approval of sparsentan as a treatment for IgAN.

In addition, because both the DUPLEX Study and PROTECT Study are evaluating the same compound for the treatment of chronic kidney diseases and utilizing similar endpoints, the risk of success or failure for the two studies may, depending on the outcomes of the studies, end up being correlated.

Even if we receive regulatory approval for any product candidates, we will be subject to ongoing regulatory obligations and continued regulatory review, which may result in significant additional expense.

Any regulatory approvals that we receive for any product candidates may be subject to significant limitations on the approved indicated uses for which the product may be marketed or to the conditions of approval, or contain requirements for potentially costly post-marketing testing, including Phase 4 clinical trials, and surveillance to monitor the safety and efficacy of the product candidate.

In addition, if the FDA or a comparable foreign regulatory authority approves any product candidates, those products will be subject to extensive and ongoing regulatory requirements, including for the manufacturing, labeling, packaging, distribution, adverse event reporting, storage, advertising, promotion, import, export, recordkeeping, conduct of potential post-marketing studies and post-market submission requirements. These requirements include submissions of safety and other post-marketing information and reports, registration, as well as continued compliance with current good manufacturing practices and good clinical practices, for any clinical trials that we conduct post-approval. Later discovery of previously unknown problems with a product, including adverse events of unanticipated severity or frequency, undesirable side effects caused by the product, problems encountered by our third-party manufacturers or manufacturing processes, or failure to comply with regulatory requirements, either before or after product approval, may result in, among other things:

- restrictions on the marketing, manufacturing, or distribution of the product;

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- requirements to include additional warnings on the label;
- requirements to create or enhance a medication guide outlining the risks to patients;
- withdrawal of the product from the market;
- voluntary or mandatory product recalls;
- requirements to change the way the product is administered or for us to conduct additional clinical trials;
- fines, warning or untitled letters or holds on clinical trials;
- refusal by the FDA to approve pending applications or supplements to approved applications filed by us or our strategic partners, or suspension or revocation of product license approvals;
- product seizure or detention, or refusal to permit the import or export of products;
- injunctions or the imposition of civil or criminal penalties; and
- harm to our reputation.

For example, we have certain post-marketing requirements and commitments associated with Cholbam. Further, we face risks relating to the post marketing obligations and commercial acceptance of Cholbam, which was approved by the FDA on March 17, 2015. If the regulatory approval for Chenodal, Cholbam and/or Thiola are withdrawn for any reason, it would have a material adverse impact on our sales and profitability.

The independent clinical investigators and contract research organizations that we rely upon to conduct our clinical trials may not be diligent, careful or timely, and may make mistakes, in the conduct of our trials.

We depend on independent clinical investigators and contract research organizations (“CROs”) to conduct our clinical trials under agreements with us. The CROs play a significant role in the conduct of our clinical trials. Failure of the CROs to meet their obligations could adversely affect clinical development of our product candidates. The independent clinical investigators are not our employees and we cannot control the timing or amount of resources they devote to our studies. If their performance is substandard, it could delay or prevent approval of our FDA applications. Moreover, these independent investigators and CROs may also have relationships with other commercial entities, some of which may compete with us. If independent investigators and CROs allocate their resources to assist our competitors at our expense, it could harm our competitive position.

We have limited control over the development activities of Censa’s CNSA-001 program and our investment in Censa’s CNSA-001 program may be adversely affected.

We have entered into a joint development agreement with Censa to evaluate CNSA-001 for the treatment of PKU. Under this agreement, we have agreed to fund certain development activities of Censa’s CNSA-001 program, in an aggregate amount expected to be approximately \$17 million through proof of concept. However, we have limited control over the development activities of Censa’s CNSA-001 program and face the risk that the development program for CNSA-001 will not be successful, that Censa does not conduct the development activities in a timely manner, or that the development program for CNSA-001 may cost more than expected to reach proof of concept. If any of these issues arise, our investment in Censa’s CNSA-001 program may be adversely affected and our business may suffer.

Risks Related to the Commercialization of Our Products

The commercial success of Chenodal, Cholbam and Thiola depends on them being considered to be effective drugs with advantages over other therapies.

The commercial success of our products Chenodal, Cholbam and Thiola depends on them being considered to be effective drugs with advantages over other therapies. A number of factors, as discussed in greater detail below, may adversely impact the degree of acceptance of these products, including their efficacy, safety, price and benefits over competing therapies, as well as the coverage and reimbursement policies of third-party payers, such as government and private insurance plans.

If unexpected adverse events are reported in connection with the use of any of these products, physician and patient acceptance of the product could deteriorate and the commercial success of such product could be adversely affected. We are required to report to the FDA events associated with our products relating to death or injury. Adverse events could result in additional regulatory controls, such as a requirement for costly post-approval clinical studies or revisions to our approved labeling which could limit the indications or patient population for a product or could even lead to the withdrawal of a product from the market.

A number of recent regulatory and legislative initiatives have been introduced to encourage generic competition for pharmaceutical products, and if a generic version of any of our products enters the market, sales of that product would be negatively impacted.

Chenodal and Thiola are subject to immediate competition from compounded and generic entrants, as the ANDA and NDA for these drug products have no remaining patent or nonpatent exclusivity. There have been a number of recent regulatory and legislative initiatives designed to encourage generic competition for pharmaceutical products, including expedited review procedures for generic manufacturers and incentives designed to spur generic competition of branded drugs. Also, the FDA and the U.S. Federal Trade Commission have been focused on brand companies’ denial of drug supply to potential generic competitors for testing, and the U.S. Congress has been considering a legislatively defined private right of action

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under which generic companies could bring suit against companies who refuse access to product for bioequivalence testing. We cannot currently predict the specific outcome or impact on our business of such regulatory actions or legislation. In October 2018, we were named as a defendant in a lawsuit brought by a purported generic company seeking to acquire samples of Thiola in order to conduct bioequivalence studies.

Under the Hatch-Waxman Amendments of the Federal Food, Drug, and Cosmetic Act (the "FDC Act"), a pharmaceutical manufacturer may file an ANDA, seeking approval of a generic copy of an approved innovator product or an NDA under Section 505(b)(2) that relies on the FDA's prior findings of safety and effectiveness in approving the innovator product. A Section 505(b)(2) NDA may be for a new or improved version of the original innovator product. The Hatch-Waxman Amendments also provide for certain periods of regulatory exclusivity, which preclude FDA approval (or in some circumstances, FDA acceptance) of an ANDA or Section 505(b)(2) NDA. In addition, the FDC Act provides, subject to certain exceptions, a period during which an FDA-approved drug may be afforded orphan drug exclusivity. In addition to the benefits of regulatory exclusivity, an innovator NDA holder may have patents claiming the active ingredient, product formulation or an approved use of the drug, which would be listed with the product in the FDA publication, "Approved Drug Products with Therapeutic Equivalence Evaluations," known as the "Orange Book." If there are patents listed in the Orange Book, a generic or Section 505(b)(2) applicant that seeks to market its product before expiration of the patents must include in the ANDA what is known as a "Paragraph IV certification," challenging the validity or enforceability of, or claiming non-infringement of, the listed patent or patents. Notice of the certification must be given to the innovator, too, and if within 45 days of receiving notice the innovator sues to enforce its patents, approval of the ANDA is stayed for 30 months, or as lengthened or shortened by the court.

If a generic version of Chenodal, Thiola or any of our products is approved, sales of that product would be negatively impacted, which would have a material adverse impact on our sales and profitability.

Changes in reimbursement practices of third-party payers could affect the demand for our products and the prices at which they are sold.

The business and financial condition of healthcare-related businesses will continue to be affected by efforts of governments and third-party payers to contain or reduce the cost of healthcare through various means. In the United States and some foreign jurisdictions, there have been a number of legislative and regulatory changes and proposed changes regarding the healthcare system that could prevent or delay marketing approval for fosmetopantotenate, sparsentan, CNSA-001 and L-UDCA, or any other product candidate that we develop, restrict or regulate post-approval activities and affect our ability to profitably sell fosmetopantotenate, sparsentan, CNSA-001 and L-UDCA or any other product candidate for which we obtain marketing approval.

Our products are sold to patients whose healthcare costs are met by third-party payers, such as government programs, private insurance plans and managed-care programs. These third-party payers are increasingly attempting to contain healthcare costs by limiting both coverage and the level of reimbursement for medical products and services. Levels of reimbursement, if any, may be decreased in the future, and future healthcare reform legislation, regulations or changes to reimbursement policies of third party payers may otherwise adversely affect the demand for and price levels of our products, which could have a material adverse effect on our sales and profitability.

Economic, social, and congressional pressure may result in individuals and government entities increasingly seeking to achieve cost savings through mechanisms that limit coverage or payment for our products. For example, state Medicaid programs are increasingly requesting manufacturers to pay supplemental rebates and requiring prior authorization for use of drugs. Managed care organizations continue to seek price discounts and, in some cases, to impose restrictions on the coverage of particular drugs. Government efforts to reduce Medicaid expenses may lead to increased use of managed care organizations by Medicaid programs. This may result in managed care organizations influencing prescription decisions for a larger segment of the population and a corresponding constraint on prices and reimbursement for our products.

We are dependent on third parties to manufacture and distribute our pharmaceutical products who may not fulfill their obligations.

We have no manufacturing capabilities and rely on third party manufacturers who are sole source suppliers for manufacturing of Chenodal, Cholbam and Thiola. The facilities used by our third party manufacturers must be approved by the FDA, or in the case of Kolbam in the European Union, the European Medicines Agency. Our dependence on third parties for the manufacture of our products may harm our profit margin on the sale of products and our ability to deliver products on a timely and competitive basis. If our third party manufacturers are unable to manufacture to specifications or in compliance with applicable regulatory requirements, our ability to commercialize our products will be adversely impacted and could affect our ability to gain market acceptance for our products and negatively impact our revenues.

We currently have no in-house distribution channels for Chenodal, Cholbam or Thiola and we are dependent on a third-party distributor, Dohmen Life Sciences Services, an Eversana Company, to distribute such products. We rely on this distributor for all of our proceeds from sales of Chenodal, Cholbam and Thiola in the United States. The outsourcing of our distribution function is complex, and we may experience difficulties that could reduce, delay or stop shipments of such products. If we encounter such distribution problems, and we are unable to quickly enter into a similar agreement with another distributor on substantially similar terms, distribution of Chenodal, Cholbam and/or Thiola could become disrupted, resulting in lost revenues, provider dissatisfaction, and/or patient dissatisfaction.

Governments outside the United States tend to impose strict price controls and reimbursement approval policies, which may adversely affect our prospects for generating revenue.

In some countries, particularly European Union countries, the pricing of prescription pharmaceuticals is subject to governmental control. In these countries, pricing negotiations with governmental authorities can take considerable time (6 to 12 months or longer) after the receipt of marketing approval for a product. 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 candidate to other available therapies. If reimbursement of our products is unavailable or limited in scope or amount, or if pricing is set at unsatisfactory levels, our prospects for generating revenue outside of the United States, if any, could be adversely affected and our business may suffer.

If we are unable to establish sales and marketing capabilities or enter into agreements with third parties to market and sell our product candidates, we may be unable to generate product revenue outside of the United States.

We may not be able to rely on orphan drug exclusivity for Cholbam/Kolbam or any of our products.

Regulatory authorities in some jurisdictions, including the United States and Europe, may designate drugs for relatively small patient populations as orphan drugs. We have obtained orphan designation for Cholbam/Kolbam in the United States and the European Union. Generally, if a product with an orphan drug designation subsequently receives the first marketing approval for the indication for which it has such designation, that product is entitled to a period of marketing exclusivity, which precludes the applicable regulatory authority from approving another marketing application for the same drug for the same indication for that time period. The applicable period is seven years in the United States and ten years in Europe. Even though we have been awarded orphan drug exclusivity for Cholbam in the United States, we may not be able to maintain it. For example, if a competitive product that contains the same active moiety and treats the same disease as our product is shown to be clinically superior to our product, any orphan drug exclusivity we have obtained will not block the approval of such competitive product and we may effectively lose orphan drug exclusivity. Similarly, if a competitive product that contains the same active moiety and treats the same disease as our product candidate is approved for orphan drug exclusivity before our product candidate, we may not be able to obtain approval for our product candidate until the expiration of the competitive product's orphan drug exclusivity unless our product candidate is shown to be clinically superior to the competitive product.

Risks Related to our Products and Product Candidates

Our products may not achieve or maintain expected levels of market acceptance or commercial success.

The success of our products is dependent upon achieving and maintaining market acceptance. Commercializing products is time consuming, expensive and unpredictable. There can be no assurance that we will be able to, either by ourselves or in collaboration with our partners or through our licensees, successfully commercialize new products or current products or gain market acceptance for such products. New product candidates that appear promising in development may fail to reach the market or may have only limited or no commercial success.

Further, the discovery of significant problems with a product similar to one of our products that implicate (or are perceived to implicate) an entire class of products could have an adverse effect on sales of the affected products. Accordingly, new data about our products, or products similar to our products, could negatively impact demand for our products due to real or perceived side effects or uncertainty regarding efficacy and, in some cases, could result in product withdrawal.

Our current products and any products that we bring to the market, including fosmetopantotenate, sparsentan, CNSA-001 and L-UDCA, if they receive marketing approval, may not gain market acceptance by physicians, patients, third-party payers, and others in the medical community. If these products do not achieve an adequate level of acceptance, we may not generate significant product revenue and we may not become profitable. The degree of market acceptance of our current products and product candidates, if approved for commercial sale, will depend on a number of factors, including:

- the prevalence and severity of any side effects, including any limitations or warnings contained in a product's approved labeling;
- the efficacy and potential advantages over alternative treatments;
- the pricing of our product candidates;
- relative convenience and ease of administration;
- the willingness of the target patient population to try new therapies and of physicians to prescribe these therapies;
- the strength of marketing and distribution support and timing of market introduction of competitive products;
- publicity concerning our products or competing products and treatments; and
- sufficient third-party insurance coverage and reimbursement.

Even if a potential or current product displays a favorable efficacy and safety profile in preclinical and clinical trials, market acceptance of the product will not be known until after it is launched. Our efforts to educate patients, the medical community, and third-party payers on the benefits of our product may require significant resources and may never be successful. Such efforts to educate the marketplace may require more resources than are required by the conventional marketing technologies employed by our competitors.

If the market opportunities for our products and product candidates are smaller than we believe they are, our revenues may be adversely affected and our business may suffer.

Certain of the diseases that our current and future product candidates are being developed to address, such as PKAN, PKU, FSGS and IgAN, are relatively rare. Our projections of both the number of people who have these diseases, as well as the subset of people with these diseases who have the potential to benefit from treatment with our product candidates, may not be accurate.

Currently, most reported estimates of the prevalence of PKAN and FSGS are based on studies of small subsets of the population of specific geographic areas, which are then extrapolated to estimate the prevalence of the diseases in the broader world population. As new studies are performed the estimated prevalence of these diseases may change. There can be no assurance that the prevalence of PKAN and FSGS in the study populations accurately reflect the prevalence of these diseases in the broader world population. If our estimates of the prevalence of PKAN, PKU, FSGS, or IgAN or of the number of patients who may benefit from treatment with fosmetpantotenate, sparsentan, and CNSA-001 prove to be incorrect, the market opportunities for our product candidates may be smaller than we believe they are, our prospects for generating revenue may be adversely affected and our business may suffer.

Our product candidates may cause undesirable side effects or have other properties that could delay or prevent their regulatory approval or commercialization.

Undesirable side effects caused by our product candidates could interrupt, delay or halt clinical trials and could result in the denial of regulatory approval by the FDA or other regulatory authorities for any or all targeted indications, and in turn prevent us from commercializing our product candidates and generating revenues from their sale.

In addition, if any of our product candidates receive marketing approval and we or others later identify undesirable side effects caused by the product:

- regulatory authorities may require the addition of restrictive labeling statements;
- regulatory authorities may withdraw their approval of the product; and
- we may be required to change the way the product is administered or conduct additional clinical trials.

Any of these events could prevent us from achieving or maintaining market acceptance of the affected product or could substantially increase the costs and expenses of commercializing the product candidate, which in turn could delay or prevent us from generating significant revenues from its sale or adversely affect our reputation.

We do not currently have patent protection for certain of our products and product candidates. If we are unable to obtain and maintain protection for the intellectual property relating to our technology and products, the value of our technology and products will be adversely affected.

Our success will depend in large part on our ability to obtain and maintain protection in the United States and other countries for the intellectual property covering, or incorporated into, our technology and products. The patent situation in the field of biotechnology and pharmaceuticals generally is highly uncertain and involves complex legal, technical, scientific and factual questions. We may not be able to obtain additional issued patents relating to our technology or products. Even if issued, patents issued to us or our licensors may be challenged, narrowed, invalidated, held to be unenforceable or circumvented, which could limit our ability to stop competitors from marketing similar products or reduce the term of patent protection we may have for our products. Changes in either patent laws or in interpretations of patent laws in the United States and other countries may diminish the value of our intellectual property or narrow the scope of our patent protection. Fosmetpantotenate is covered by our U.S. Patent No. 8,673,883, which was granted in 2014 and expires in 2033. In addition, our U.S. Patent No. 9,181,286, which was granted on November 10, 2015 and expires in 2033, covers the use of fosmetpantotenate for the treatment of PKAN, and our U.S. Patent No. 9,629,862, which was granted on April 25, 2017 and also expires in 2033, covers pharmaceutical compositions that contain fosmetpantotenate. Sparsentan is covered by U.S. Patent No. 6,638,937, which expires in 2019 and to which we have an exclusive license. In addition, U.S. Patent No. 9,662,312, to which we also have an exclusive license and which was granted on May 30, 2017 and expires in 2030, covers the use of sparsentan for treating glomerulosclerosis, including FSGS. And U.S. Patent No. 9,993,461, to which we also have an exclusive license and which was granted on June 12, 2018 and expires in 2030, covers the use of sparsentan for treating IgA nephropathy as well as glomerulosclerosis, including FSGS.

For products we develop based on a new chemical entity not previously approved by the FDA, we expect that in addition to the protection afforded by our patent filings that we will be able to obtain either five years regulatory exclusivity via the provisions of the FDC Act and possibly seven years regulatory exclusivity via the orphan drug provisions of the FDC Act. In addition, we may be able to obtain up to five years patent term extension (to compensate for regulatory approval delay) for a patent covering such a product.

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

- we or our licensors were the first to make the inventions covered by each of our pending patent applications;
- 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 patents issued to us or our licensors that provide a basis for commercially viable products will provide us with any competitive advantages or will not be challenged by third parties;

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- we will develop additional proprietary technologies that are patentable;
- we will file patent applications for new proprietary technologies promptly or at all;
- the claims we make in our patents will be upheld by patent offices in the United States and elsewhere;
- our patents will not expire prior to or shortly after commencing commercialization of a product; and
- the patents of others will not have a negative effect on our ability to do business.

We have negotiated a license agreement with Ligand Pharmaceuticals for the rights to sparsentan which we are initially developing for the treatment of FSGS and IgAN. This license subjects us to various commercialization, reporting and other obligations. If we were to default on our obligations, we could lose our rights to sparsentan. We have obtained a U.S. and European patent covering the use of sparsentan for treating glomerulosclerosis, including FSGS, and a second U.S. patent covering both the use of sparsentan for treating IgAN and the use of sparsentan for treating glomerulosclerosis, including FSGS. However, we cannot be certain that we will be able to obtain patent protection for various other potential indications for sparsentan, or whether, if granted, we would be able to enforce such patents.

Our patents also may not afford us protection against competitors with similar technology. Because patent applications in the United States and many other jurisdictions are typically not published until 18 months after filing, or in some cases not at all, and because publications of discoveries in the scientific literature often lag behind the actual discoveries, neither we nor our licensors can be certain that we or they were the first to make the inventions claimed in our or their issued patents or pending patent applications, or that we or they were the first to file for protection of the inventions set forth in these patent applications. If a third party has also filed a United States patent application prior to the effective date of the relevant provisions of the America Invents Act (i.e. before March 16, 2013) covering our product candidates or a similar invention, we may have to participate in an adversarial proceeding, known as an interference, declared by the USPTO to determine priority of invention in the United States. The costs of these proceedings could be substantial and it is possible that our efforts could be unsuccessful, resulting in a loss of our United States patent position.

We cannot assure you that third parties will not assert patent or other intellectual property infringement claims against us with respect to technologies used in our products. If patent infringement suits were brought against us, we may be unable to commercialize some of our products which could severely harm our business. Litigation proceedings, even if not successful, could result in substantial costs and harm our business.

We expect to rely on orphan drug status to develop and commercialize certain of our product candidates, but our orphan drug designations may not confer marketing exclusivity or other expected commercial benefits.

We expect to rely on orphan drug exclusivity for fosmetpantotenate and sparsentan and potential future product candidates that we may develop. Orphan drug status currently confers seven years of marketing exclusivity in the United States under the FDC Act, and up to ten years of marketing exclusivity in Europe for a particular product in a specified indication. The FDA and EMA have granted orphan designation for Chenodal, fosmetpantotenate and sparsentan for the treatment of CTX, PKAN and FSGS, respectively. While we have been granted these orphan designations, we will not be able to rely on these designations to exclude other companies from manufacturing or selling these molecules for the same indication beyond these time frames. Furthermore, any marketing exclusivity in Europe can be reduced from ten years to six years if the initial designation criteria have significantly changed since the market authorization of the orphan product.

For any product candidate for which we have been granted orphan drug designation in a particular indication, it is possible that another company also holding orphan drug designation for the same product candidate will receive marketing approval for the same indication before we do. If that were to happen, our applications for that indication may not be approved until the competing company's period of exclusivity expires. Even if we are the first to obtain marketing authorization for an orphan drug indication in the United States, there are circumstances under which a competing product may be approved for the same indication during the seven-year period of marketing exclusivity, such as if the later product is shown to be clinically superior to our orphan product, or if the later product is deemed a different product than ours. Further, the seven-year marketing exclusivity would not prevent competitors from obtaining approval of the same product candidate as ours for indications other than those in which we have been granted orphan drug designation, or for the use of other types of products in the same indications as our orphan product.

Any drugs we develop may become subject to unfavorable pricing regulations, third-party reimbursement practices or healthcare reform initiatives, thereby harming our business.

In March 2010, President Obama signed the Patient Protection and Affordable Care Act, as amended by the Health Care and Education Reconciliation Act of 2010 (collectively, the "PPACA"), a sweeping law intended to broaden access to health insurance, reduce or constrain the growth of healthcare spending, enhance remedies against fraud and abuse, add new transparency requirements for healthcare and health insurance industries, impose new taxes and fees on the health industry and impose additional health policy reforms. The PPACA revised the definition of "average manufacturer price" for reporting purposes, which could increase the amount of Medicaid drug rebates to states. The PPACA also increased the mandated Medicaid rebate from 15.1% to 23.1% of the average manufacturer price, expanded the rebate to Medicaid managed care utilization and increased the types of entities eligible for the federal 340B drug discount program. Further, the law imposes a significant annual fee on companies that manufacture or import certain branded prescription drug products. There have been judicial, Congressional, and political challenges to certain aspects of the PPACA, as well as recent efforts by the Trump administration to repeal or replace certain aspects of the PPACA. Since January 2017, President Trump has signed two Executive Orders and other directives designed to delay the implementation of certain provisions of the PPACA or otherwise circumvent some of the requirements for health insurance mandated by the PPACA. Concurrently, Congress has considered legislation that would repeal or repeal and replace all or part of the PPACA. While Congress has not passed comprehensive repeal legislation, two bills affecting the implementation of certain taxes under the PPACA have been signed into law. The Tax Act of 2017 includes a

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provision which repealed, effective January 1, 2019, the tax-based shared responsibility payment imposed by the PPACA on certain individuals who fail to maintain qualifying health coverage for all or part of a year that is commonly referred to as the "individual mandate". On January 22, 2018, President Trump signed a continuing resolution on appropriations for fiscal year 2018 that delayed the implementation of certain PPACA-mandated fees, including the so-called "Cadillac" tax on certain high cost employer-sponsored insurance plans, the annual fee imposed on certain health insurance providers based on market share, and the medical device excise tax on non-exempt medical devices. The Bipartisan Budget Act of 2018 ("BBA"), among other things, amends the PPACA, effective January 1, 2019, to increase the discount that is owed by pharmaceutical manufacturers who participate in Medicare Part D from 50 percent to 70 percent, and closes the coverage gap in most Medicare drug plans, commonly referred to as the "donut hole". In July 2018, the Centers for Medicare and Medicaid Services ("CMS") published a final rule permitting further collections and payments to and from certain PPACA qualified health plans and health insurance issuers under the PPACA risk adjustment program in response to the outcome of federal district court litigation regarding the method CMS uses to determine this risk adjustment. On December 14, 2018, a Texas U.S. District Court Judge ruled that the PPACA is unconstitutional in its entirety because the "individual mandate" was repealed by Congress as part of the Tax Act of 2017. While the Texas U.S. District Court Judge, as well as the Trump administration and CMS, have stated that the ruling will have no immediate effect pending appeal of the decision, it is unclear how this decision, subsequent appeals, and other efforts to repeal and replace the PPACA will impact the PPACA and our business.

In addition, other legislative changes have been proposed and adopted since the PPACA was enacted. For example, in August 2011, the President signed into law the Budget Control Act of 2011, which, among other things, created the Joint Select Committee on Deficit Reduction to recommend to Congress proposals in spending reductions. The Joint Select Committee on Deficit Reduction did not achieve a targeted deficit reduction of at least \$1.2 trillion for fiscal years 2012 through 2021, triggering the legislation's automatic reduction to several government programs. This includes aggregate reductions to Medicare payments to providers of up to 2% per fiscal year, which went into effect beginning on April 1, 2013 and, due to subsequent legislative amendments, including the BBA, will stay in effect through 2027 unless additional Congressional action is taken. Additionally, in January 2013, the American Taxpayer Relief Act of 2012 was signed into law, which, among other things, reduced Medicare payments to several providers, including hospitals and imaging centers.

If we are unable to obtain coverage and adequate reimbursement from governments or third-party payers for any products that we may develop or if we are unable to obtain acceptable prices for those products, our prospects for generating revenue and achieving profitability will suffer.

Our prospects for generating revenue and achieving profitability will depend heavily upon the availability of coverage and adequate reimbursement for the use of our approved product candidates from governmental and other third-party payers, both in the United States and in other markets. Reimbursement by a third-party payer may depend upon a number of factors, including the third-party payer's determination that use of a product is:

- a covered benefit under its health plan;
- safe, effective and medically necessary;
- appropriate for the specific patient;
- cost-effective; and
- neither experimental nor investigational.

Obtaining reimbursement approval for a product from each government or other third-party payer is a time consuming and costly process that could require us to provide supporting scientific, clinical and cost effectiveness data for the use of our products to each payer. We may not be able to provide data sufficient to gain acceptance with respect to reimbursement or we might need to conduct post-marketing studies in order to demonstrate the cost-effectiveness of any future products to such payers' satisfaction. Such studies might require us to commit a significant amount of management time and financial and other resources. Even when a payer determines that a product is eligible for reimbursement, the payer may impose coverage limitations that preclude payment for some uses that are approved by the FDA or non-United States regulatory authorities. Also prior authorization for a product may be required. In addition, there is a risk that full reimbursement may not be available for high-priced products. Moreover, eligibility for coverage does not imply that any product will be reimbursed in all cases or at a rate that allows us to make a profit or even cover our costs. Interim payments for new products, if applicable, may also not be sufficient to cover our costs and may not be made permanent.

A primary trend in the United States healthcare industry and elsewhere is toward cost containment. We expect the changes made by PPACA, other legislation impacting the Medicare program and the 340B program, and the increasing emphasis on managed care to continue to put pressure on pharmaceutical product pricing. As these concerns continue to grow over the need for tighter oversight, there remains the possibility that the Health Resources and Services Administration or another agency under the HHS will propose regulations or that Congress will explore changes to the 340B program through legislation. For example, a bill was introduced in 2018 that would require hospitals to report their low-income utilization of the program. Further, the Centers for Medicare & Medicaid Services issued a final rule that would revise the Medicare hospital outpatient prospective payment system for calendar year 2019, including a new reimbursement methodology for drugs purchased under the 340B program for Medicare patients at the hospital setting and recently announced the same change for physician-based practices under 340B in 2019. In addition, HHS has set January 1, 2019, as the effective date of the final rule setting forth the calculation of the ceiling price and application of civil monetary penalties. Pursuant to the final rule, after January 1, 2019, manufacturers must calculate 340B program ceiling prices on a quarterly basis. Moreover, manufacturers could be subject to a \$5,000 penalty for each instance where they knowingly and intentionally overcharge a covered entity under the 340B program.

Further, there has been increasing legislative and enforcement interest in the United States with respect to drug pricing practices, including several recent U.S. congressional inquiries and proposed federal and enacted state legislation designed to, among other things, increase drug pricing transparency, expedite generic competition, review relationships between pricing and manufacturer patient assistance programs, and reform

government program drug reimbursement methodologies. At the federal level, the Trump administration's budget proposal for fiscal year 2019 contains further drug price control measures that could be enacted during the 2019 budget process or in other future legislation, including, for example, measures to permit Medicare Part D plans to negotiate the price of certain drugs under Medicare Part B, to allow some states to negotiate drug prices under Medicaid, and to eliminate cost sharing for generic drugs for low-income patients. Further, the current administration released a "Blueprint" to lower drug prices and reduce out of pocket costs of drugs that contains additional proposals to increase manufacturer competition, increase the negotiating power of certain federal healthcare programs, incentivize manufacturers to lower the list price of their products and reduce the out of pocket costs of drug products paid by consumers. The HHS has already started the process of soliciting feedback on some of these measures and, at the same time, is immediately implementing others under its existing authority. For example, in September 2018, CMS announced that it will allow Medicare Advantage Plans the option to use step therapy for Part B drugs beginning January 1, 2019, and in October 2018, CMS proposed a new rule that would require direct-to-consumer television advertisements of prescription drugs and biological products, for which payment is available through or under Medicare or Medicaid, to include in the advertisement the Wholesale Acquisition Cost, or list price, of that drug or biological product. On January 31, 2019, the HHS Office of Inspector General proposed modifications to the Federal Anti-Kickback Statute discount safe harbor for the purpose of reducing the cost of drug products to consumers which, among other things, if finalized, will affect discounts paid by manufacturers to Medicare Part D plans, Medicaid managed care organizations and pharmacy benefit managers working with these organizations. Although a number of these, and other potential proposals, may require authorization through additional legislation to become effective, Congress and the Trump administration have each indicated that it will continue to seek new legislative and/or administrative measures to control drug costs. At the state level, legislatures are increasingly 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.

Any reduction in reimbursement from Medicare, Medicaid or other government-funded programs may result in a similar reduction in payments from private payors. The implementation of cost containment measures or other healthcare reforms may prevent us from being able to generate revenue, attain profitability or commercialize our drugs. Additionally, we are currently unable to predict what additional legislation or regulation, if any, relating to the healthcare industry may be enacted in the future or what effect recently enacted federal legislation or any such additional legislation or regulation would have on our business.

We face potential product liability exposure far in excess of our limited insurance coverage.

The use of any of our potential products in clinical trials, and the sale of any approved products, may expose us to liability claims. These claims might be made directly by consumers, health care providers, pharmaceutical companies or others selling our products. We have obtained limited product liability insurance coverage for our clinical trials in the amount of \$10 million per occurrence and \$10 million in the aggregate. However, our insurance may not reimburse us or may not be sufficient to reimburse us for any expenses or losses we may suffer. Moreover, insurance coverage is becoming increasingly expensive, and 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. We intend to expand our insurance coverage to include the sale of commercial products if we obtain marketing approval for product candidates in development, but we may be unable to obtain commercially reasonable product liability insurance for any products approved for marketing. On occasion, juries have awarded large judgments in class action lawsuits based on drugs that had unanticipated side effects. A successful product liability claim or series of claims brought against us would decrease our cash reserves and could cause our stock price to fall.

We face substantial competition, which may result in others discovering, developing or commercializing products before or more successfully than we do. Our operating results will suffer if we fail to compete effectively.

Several of our competitors have substantially greater financial, research and development, distribution, manufacturing and marketing experience and resources than we do and represent substantial long-term competition for us. Other companies may succeed in developing and marketing products that are more effective and/or less costly than any products that may be developed and marketed by us, or that are commercially accepted before any of our products. Factors affecting competition in the pharmaceutical and drug industries vary, depending on the extent to which a competitor is able to achieve a competitive advantage based on its proprietary technology and ability to market and sell drugs. The industry in which we compete is characterized by extensive research and development efforts and rapid technological progress. Although we believe that our orphan drug status for Cholbam and proprietary position with respect to fosmetopantotenate and sparsentan may give us a competitive advantage, new developments are expected to continue and there can be no assurance that discoveries by others will not render such potential products noncompetitive. Furthermore, competitors could enter the market with generic versions of our products.

Our competitive position also depends on our ability to enter into strategic alliances with one or more large pharmaceutical and contract manufacturing companies, attract and retain qualified personnel, develop effective proprietary products, implement development and marketing plans, obtain patent protection, secure adequate capital resources and successfully sell and market our approved products. There can be no assurance that we will be able to successfully achieve all of the foregoing objectives.

Use of third parties to manufacture and distribute our products and product candidates may increase the risk that we will not have sufficient quantities of our product and product candidates or such quantities at an acceptable cost, and clinical development and commercialization of our product and product candidates could be delayed, prevented or impaired.

We do not own or operate manufacturing facilities for clinical or commercial production of our products. We have limited personnel with experience in drug manufacturing and we lack the resources and the capabilities to manufacture any of our product candidates on a clinical or commercial scale. We outsource all manufacturing and packaging of our preclinical, clinical, and commercial products to third parties. The manufacture of

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pharmaceutical products requires significant expertise and capital investment, including the development of advanced manufacturing techniques and process controls. Manufacturers of pharmaceutical products often encounter difficulties in production, particularly in scaling up initial production and in maintaining required quality control. These problems include difficulties with production costs and yields and quality control, including stability of the product candidate.

We do not currently have any agreements with third-party manufacturers for the long-term commercial supply of any of our development stage product candidates. We may be unable to enter into agreements for commercial supply with third-party manufacturers, or may be unable to do so on acceptable terms. Even if we enter into these agreements, the manufacturers of each product candidate will be single source suppliers to us for a significant period of time. Reliance on third-party manufacturers entails risks to which we may not be subject if we manufactured our product candidates or products ourselves, including:

- reliance on the third party for regulatory compliance and quality assurance;
- limitations on supply availability resulting from capacity and scheduling constraints of the third parties;
- impact on our reputation in the marketplace if manufacturers of our products fail to meet the demands of our customers;
- the possible breach of the manufacturing agreement by the third party because of factors beyond our control; and
- the possible termination or nonrenewal of the agreement by the third party, based on its own business priorities, at a time that is costly or inconvenient for us.

The failure of any of our contract manufacturers to maintain high manufacturing standards could result in injury or death of clinical trial participants or patients using our products. Such failure could also result in product liability claims, product recalls, product seizures or withdrawals, delays or failures in testing or delivery, cost overruns or other problems that could seriously harm our business or profitability.

Our contract manufacturers will be required to adhere to FDA regulations setting forth cGMP. These regulations cover all aspects of the manufacturing, testing, quality control and recordkeeping relating to our product candidates and any products that we may commercialize. Our manufacturers may not be able to comply with cGMP regulations or similar regulatory requirements outside the United States. Our manufacturers are subject to unannounced inspections by the FDA, state regulators and similar regulators outside the United States. Our failure, or the failure of our third-party manufacturers, to comply with applicable regulations could result in sanctions being imposed on us, including fines, injunctions, civil penalties, failure of regulatory authorities to grant marketing approval of our product candidates, delays, suspension or withdrawal of approvals, license revocation, seizures or recalls of product candidates or products, operating restrictions and criminal prosecutions, any of which could significantly and adversely affect regulatory approval and supplies of our product candidates.

Our product and any products that we may develop may compete with other product candidates and products for access to manufacturing facilities. There are a limited number of manufacturers that operate under cGMP regulations and that are both capable of manufacturing for us and willing to do so. If the third parties that we engage to manufacture products for our developmental or commercial products should cease to continue to do so for any reason, we likely would experience interruptions in cash flows and/or delays in advancing our clinical trials while we identify and qualify replacement suppliers, and we may be unable to obtain replacement supplies on terms that are favorable to us. Later relocation to another manufacturer will also require notification, review and other regulatory approvals from the FDA and other regulators and will subject our production to further cost and instability in the availability of our product candidates. In addition, if we are not able to obtain adequate supplies of our product candidates, or the drug substances used to manufacture them, it will be more difficult for us to sell our products and to develop our product candidates. This could greatly reduce our competitiveness.

Our current and anticipated future dependence upon others for the manufacture of our product candidates may adversely affect our future profit margins and our ability to develop product candidates and commercialize any products that obtain regulatory approval on a timely and competitive basis.

Materials necessary to manufacture our products and product candidates may not be available on commercially reasonable terms, or at all, which may delay the development and commercialization of our products and product candidates.

We rely on the manufacturers of our products and product candidates to purchase from third-party suppliers the materials necessary to produce the compounds for our preclinical and clinical studies and rely on these other manufacturers for commercial distribution if we obtain marketing approval for any of our product candidates. Suppliers may not sell these materials to our manufacturers at the time we need them or on commercially reasonable terms and all such prices are susceptible to fluctuations in price and availability due to transportation costs, government regulations, price controls, and changes in economic climate or other foreseen circumstances. We do not have any control over the process or timing of the acquisition of these materials by our manufacturers. Moreover, we currently do not have any agreements for the commercial production of these materials. If our manufacturers are unable to obtain these materials for our preclinical and clinical studies, product testing and potential regulatory approval of our product candidates would be delayed, significantly impacting our ability to develop our product candidates. If our manufacturers or we are unable to purchase these materials after regulatory approval has been obtained for our product candidates, the commercial launch of our product candidates would be delayed or there would be a shortage in supply, which would materially affect our ability to generate revenues from the sale of our product candidates.

Risks Related to Our Business

Our limited operating history makes it difficult to evaluate our future prospects, and our profitability in the future is uncertain.

We face the problems, expenses, difficulties, complications and delays, many of which are beyond our control, associated with any business in its early stages and have a limited operating history on which an evaluation of our prospects can be made. Such prospects should be considered in light of the risks, expenses and difficulties frequently encountered in the establishment of a business in a new industry, characterized by a number of market entrants and intense competition, and in the shift from development to commercialization of new products based on innovative technologies.

We have experienced significant growth over the past three years in the number of our employees and the scope of our operations. We have added sales and marketing, compliance and legal functions in addition to expansion of all functions to support a commercial organization. To manage our anticipated future growth, we must continue to implement and improve our managerial, operational and financial systems, expand our facilities and continue to recruit and train additional qualified personnel. Due to our limited resources, we may not be able to effectively manage the expansion of our operations or recruit and train additional qualified personnel. The physical expansion of our operations may lead to significant costs and may divert our management and business development resources. Any inability on the part of our management to manage growth could delay the execution of our business plans or disrupt our operations.

Factors that may inhibit our efforts to commercialize our products without strategic partners or licensees include:

- our inability to recruit and retain adequate numbers of effective sales and marketing personnel;
- the inability of sales personnel to obtain access to or educate adequate numbers of physicians to prescribe our products;
- the lack of complementary products to be offered by our sales personnel, which may put us at a competitive disadvantage against companies with broader product lines;
- unforeseen costs associated with expanding our own sales and marketing team for new products or with entering into a partnering agreement with an independent sales and marketing organization; and
- efforts by our competitors to commercialize competitive products.

Moreover, though we generate revenues from product sales arrangements, we may incur significant operating losses over the next several years. Our ability to achieve profitable operations in the future will depend in large part upon successful in-licensing of products approved by the FDA, selling and manufacturing these products, completing development of our products, obtaining regulatory approvals for these products, and bringing these products to market. The likelihood of the long-term success of our company must be considered in light of the expenses, difficulties and delays frequently encountered in the development and commercialization of new drug products, competitive factors in the marketplace, as well as the regulatory environment in which we operate.

In addition, we may encounter unforeseen expenses, difficulties, complications, delays and other known and unknown factors.

We will likely experience fluctuations in operating results and could incur substantial losses.

We expect that our operating results will vary significantly from quarter-to-quarter and year-to-year as a result of investments in research and development, specifically our clinical and preclinical development activities. We have not completed development of any drugs and we anticipate that our expenses will increase substantially as we:

- continue our ongoing clinical development of fosmetpantotenate for the treatment of PKAN;
- continue the open label portion of DUET and conduct the planned Phase 3 trials of sparsentan indications;
- continue funding the clinical development of CNSA-001 for PKU;
- complete requirements necessary for an NDA filing of L-UDCA and;
- assuming FDA approval, the commercial launch of the next generation of Thiola;
- continue the research and development of additional product candidates;
- expand our sales and marketing infrastructure to commercialize our current products and any new products for which we may obtain regulatory approval; and
- expand operational, financial, and management information systems and personnel, including personnel to support product development efforts and our obligations as a public company.

To attain and sustain profitability, we must succeed in developing and commercializing drugs with significant market potential. This will require us to be successful in a range of challenging activities, including the discovery of product candidates, successful completion of preclinical testing and clinical trials of our product candidates, obtaining regulatory approval for these product candidates and manufacturing, marketing and selling those products for which we may obtain regulatory approval. We are only in the preliminary stages of these activities. We may not be successful enough in these activities to generate revenues that are substantial enough to achieve profitability. Even if we do achieve profitability, we may not be able to sustain or increase profitability on a quarterly or annual basis. Our failure to become or remain profitable could depress the market price of our common stock and could impair our ability to raise capital, expand our business, diversify our product offerings or continue our operations. A decline in the market price of our common stock may also cause a loss of a part or all of your investment.

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Negative publicity regarding any of our products could impair our ability to market any such product and may require us to spend time and money to address these issues.

If any of our products or any similar products distributed by other companies prove to be, or are asserted to be, harmful to consumers and/or subject to FDA enforcement action, our ability to successfully market and sell our products could be impaired. Because of our dependence on patient and physician perceptions, any adverse publicity associated with illness or other adverse effects resulting from the use or misuse of our products or any similar products distributed by other companies could limit the commercial potential of our products and expose us to potential liabilities.

We may not have sufficient insurance to cover our liability in any current or future litigation claims either due to coverage limits or as a result of insurance carriers seeking to deny coverage of such claims.

We face a variety of litigation-related liability risks. Our certificate of incorporation, bylaws, other applicable agreements, and/or Delaware law require us to indemnify (and advance expenses to) our current and past directors and officers and employees from reasonable expenses related to the defense of any action arising from their service to us, including circumstances under which indemnification is otherwise discretionary. While our directors and officers are included in a director and officer liability insurance policy, which covers all our directors and officers in some circumstances, our insurance coverage does not cover all of our indemnification obligations and may not be adequate to cover any indemnification or other claims against us. In addition, the underwriters of our present coverage may seek to avoid coverage in certain circumstances based upon the terms of the respective policies. If we incur liabilities that exceed our coverage under our directors and officers insurance policy or incur liabilities not covered by our insurance, we would have to self-fund any indemnification amounts owed to our directors and officers and employees in which case our results of operations and financial condition could be materially adversely affected. Further, if D&O insurance becomes prohibitively expensive to maintain in the future, we may be unable to renew such insurance on economic terms or unable to renew such insurance at all. The lack of D&O insurance may make it difficult for us to retain and attract talented and skilled directors and officers to serve our company, which could adversely affect our business.

We may need substantial funding and may be unable to raise capital when needed, which would force us to delay, reduce or eliminate our product development programs or commercialization efforts.

We expect our general and research and development expenses to increase in connection with our ongoing and planned activities, particularly as we conduct Phase 3 clinical trials of fosmetopantotenat and sparsentan, continue funding the clinical development of CNSA-001 and potentially acquire Censa, complete requirements for filings of L-UDCA, and conduct any other later-stage clinical trials of our product candidates. In addition, subject to obtaining regulatory approval of any of our product candidates, we expect to incur significant commercialization expenses for product sales and marketing, securing commercial quantities of product from our manufacturers, and product distribution. We currently have no additional commitments or arrangements for any additional financing to fund the research and development and commercial launch of our product candidates.

Management believes our ability to continue our operations depends on our ability to sustain and grow revenue, results of operations and our ability to access capital markets when necessary to accomplish our strategic objectives. Management believes that we may incur losses in the immediate future. We expect that our operating results will vary significantly from quarter-to-quarter and year-to-year as a result of investments in research and development, specifically our clinical and preclinical development activities. We expect to finance our cash needs from cash on hand and results of operations, and depending on results of operations we may either need additional equity or debt financing, or need to enter into strategic alliances on products in development to continue our operations until we can achieve sustained profitability and positive cash flows from operating activities. Additional funds may not be available to us when we need them on terms that are acceptable to us, or at all. If adequate funds are not available to us on a timely basis, we may be required to reduce or eliminate research development programs or commercial efforts.

Our future capital requirements will depend on many factors, including:

- the progress and results of our pre-clinical and clinical studies of fosmetopantotenat, sparsentan, CNSA-001 and other drug candidates;
- the costs, timing and outcome of regulatory review of our product candidates;
- the number and development requirements of other product candidates that we pursue;
- the costs of commercialization activities, including product marketing, sales and distribution;
- the emergence of competing technologies and other adverse market developments;
- the costs of preparing, filing and prosecuting patent applications and maintaining, enforcing and defending intellectual property related claims;
- debt service obligations on the 2019 Notes and 2025 Notes;
- the extent to which we acquire or invest in businesses, products and technologies, including the extent to which we exercise our option to acquire Censa; and
- our ability to establish collaborations and obtain milestone, royalty or other payments from any such collaborators.

The market price for shares of our common stock may be volatile and purchasers of our common stock could incur substantial losses.

The price of our stock is likely to be volatile. The stock market in general, and the market for biotechnology companies in particular, have experienced extreme volatility that has often been unrelated to the operating performance of particular companies. The market price for our common stock may be influenced by many factors, including:

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- results of clinical trials of our product candidates or those of our competitors;
- our entry into or the loss of a significant collaboration;
- regulatory or legal developments in the United States and other countries, including changes in the health care payment systems;
- our ability to obtain and maintain marketing approvals from the FDA or similar regulatory authorities outside the United States;
- variations in our financial results or those of companies that are perceived to be similar to us;
- changes in the structure of healthcare payment systems;
- market conditions in the pharmaceutical and biotechnology sectors and issuance of new or changed securities analysts' reports or recommendations;
- general economic, industry and market conditions;
- results of clinical trials conducted by others on drugs that would compete with our product candidates;
- developments or disputes concerning patents or other proprietary rights;
- public concern over our product candidates or any products approved in the future;
- litigation;
- communications from government officials regarding health care costs or pharmaceutical pricing;
- future sales or anticipated sales of our common stock by us or our stockholders; and
- the other factors described in this "Risk Factors" section.

In addition, the stock markets, and in particular, the Nasdaq Global Market, have experienced extreme price and volume fluctuations that have affected and continue to affect the market prices of equity securities of many pharmaceutical companies. The realization of any of the above risks or any of a broad range of other risks, including those described in these "Risk Factors" could have a dramatic and material adverse impact on the market price of our common stock.

We may be unable to successfully integrate new products or businesses we may acquire.

We intend to expand our product pipeline by pursuing acquisition of pharmaceutical products. If an acquisition is consummated, including the extent to which we exercise our option to acquire Censa, the integration of the acquired business, product or other assets into our company may also be complex and time-consuming and, if such businesses, products and assets are not successfully integrated, we may not achieve the anticipated benefits, cost-savings or growth opportunities. Potential difficulties that may be encountered in the integration process include the following:

- integrating personnel, operations and systems, while maintaining focus on producing and delivering consistent, high quality products;
- coordinating geographically dispersed organizations;
- distracting employees from operations;
- retaining existing customers and attracting new customers; and
- managing inefficiencies associated with integrating the operations of the Company.

Furthermore, these acquisitions and other arrangements, even if successfully integrated, may fail to further our business strategy as anticipated, expose us to increased competition or challenges with respect to our products or geographic markets, and expose us to additional liabilities associated with an acquired business, product, technology or other asset or arrangement. Any one of these challenges or risks could impair our ability to realize any benefit from our acquisitions or arrangements after we have expended resources on them.

If we are unable to maintain an effective and specialized sales force, we will not be able to commercialize our products successfully.

In order to successfully commercialize our products, we have built a specialized sales force. Factors that may hinder our ability to successfully market and commercially distribute our products include:

- inability of sales personnel to obtain access to or convince adequate numbers of physicians to prescribe our products;
- inability to recruit, retain and effectively manage adequate numbers of effective sales personnel;
- lack of complementary products to be offered by sales personnel, which may put us at a competitive disadvantage relative to companies that have more extensive product lines; and
- unforeseen delays, costs and expenses associated with maintaining our sales organization.

If we are unable to maintain our sales force for our products, we may not be able to generate sufficient product revenue.

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We will need to continue to expend significant time and resources to train our sales forces to be credible, persuasive and compliant in discussing our products with the specialists treating the patients indicated under the product's label. In addition, if we are unable to effectively train our sales force and equip them with effective marketing materials our ability to successfully commercialize our products could be diminished, which would have a material adverse effect on our business, results of operations and financial condition.

Product liability lawsuits against us could cause us to incur substantial liabilities and to limit commercialization of any products that we may develop.

Our business exposes us to potential liability risks inherent in the research, development, manufacturing and marketing of pharmaceutical products. If any of our product candidates in clinical trials or commercialized products harm people we may be subject to costly and damaging product liability claims. We have clinical trial insurance and commercial product liability coverage. However, this insurance may not be adequate to cover all claims. We may be exposed to product liability claims and product recalls, including those which may arise from misuse or malfunction of, or design flaws in, such products, whether or not such problems directly relate to the products and services we have provided. If we cannot successfully defend ourselves against claims that our product candidates or products caused injuries, we will incur substantial liabilities. Regardless of merit or eventual outcome, liability claims may result in:

- decreased demand for any product candidates or products that we may develop;
- damage to our reputation;
- regulatory investigations that could require costly recalls or product modifications;
- withdrawal of clinical trial participants;
- costs to defend the related litigation;
- substantial monetary awards to trial participants or patients, including awards that substantially exceed our product liability insurance, which we would then be required to pay from other sources, if available, and would damage our ability to obtain liability insurance at reasonable costs, or at all, in the future;
- loss of revenue;
- the diversion of management's attention from managing our business; and
- the inability to commercialize any products that we may develop.

A successful product liability claim or a series of claims brought against us could cause our stock price to fall and, if judgments exceed our insurance coverage, could decrease our available cash and adversely affect our business.

We are involved in certain litigation matters, any of which could result in substantial costs, divert management's attention and otherwise have a material adverse effect on our business, operating results or financial condition.

We are involved in certain litigation matters, including those described in Note 11 of the Consolidated Financial Statements included in this report. Although we intend to vigorously defend our interests in each matter, there is no guarantee that we will be successful and we may have to pay damages awards or otherwise may enter into settlement arrangements in connection with such matters. Any such payments or settlement arrangements could have material adverse effects on our business, operating results or financial condition. Even if we are successful in defending our interests in each matter, litigation with respect to such matters could result in substantial costs and significant adverse impact on our reputation and divert management's attention and resources, which could have a material adverse effect on our business, operating results or financial condition.

We are subject to significant ongoing regulatory obligations and oversight, which may result in significant additional expense and may limit our commercial success.

We are subject to significant ongoing regulatory obligations, such as safety reporting requirements and additional post-marketing obligations, including regulatory oversight of the promotion and marketing of our products. In addition, the manufacture, quality control, labeling, packaging, safety surveillance, adverse event reporting, storage and recordkeeping for our products are subject to extensive and ongoing regulatory requirements. If we become aware of previously unknown problems with any of our products, a regulatory agency may impose restrictions on our products, our contract manufacturers or us. If we, our products and product candidates, or the manufacturing facilities for our products and product candidates fail to comply with applicable regulatory requirements, a regulatory agency, including the FDA, may send enforcement letters, mandate labeling changes, suspend or withdraw regulatory approval, suspend any ongoing clinical trials, refuse to approve pending applications or supplements filed by us, suspend or impose restrictions on manufacturing operations, request a recall of, seize or detain a product, seek criminal prosecution or an injunction, or impose civil or criminal penalties or monetary fines. In such instances, we could experience a significant drop in the sales of the affected products, our product revenues and reputation in the marketplace may suffer, and we could become the target of lawsuits.

We are also subject to regulation by national, regional, state and local agencies, including but not limited to the FDA, CMS, Department of Justice, the Federal Trade Commission, the HHS Office of Inspector General and other regulatory bodies. The FDC Act, Social Security Act, Public Health Service Act and other federal and state statutes and regulations govern to varying degrees the research, development, manufacturing and commercial activities relating to prescription pharmaceutical products, including preclinical testing, clinical research, approval, production, labeling,

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sale, distribution, post-market surveillance, advertising, dissemination of information, promotion, marketing, and pricing to government purchasers and government health care programs. Our manufacturing partners are subject to many of the same requirements.

Companies may not promote drugs for "off-label" uses—that is, uses that are not described in the product's labeling and that differ from those approved by the FDA or other applicable regulatory agencies. However, a company may share truthful and not misleading information that is otherwise consistent with the product's labeling. A company that is found to have improperly promoted off-label uses may be subject to significant liability, including civil and administrative remedies as well as criminal sanctions. In addition, management's attention could be diverted from our business operations and our reputation could be damaged.

The federal health care program Anti-Kickback statute prohibits, among other things, knowingly and willfully offering, paying, soliciting, or receiving remuneration to induce or in return for purchasing, leasing, ordering or arranging for the purchase, lease or order of any health care item or service reimbursable under Medicare, Medicaid or other federally financed healthcare programs. This statute has been interpreted broadly to apply to arrangements that pharmaceutical companies have with prescribers, purchasers and formulary managers, among others. Further, the PPACA, among other things, amends the intent requirement of the federal anti-kickback statute so that a person or entity no longer needs to have actual knowledge of this statute or specific intent to violate it. In addition, the PPACA provides that the government may assert that a claim including items or services resulting from a violation of the federal anti-kickback statute constitutes a false or fraudulent claim for purposes of the civil False Claims Act. Although there are a number of statutory exceptions and regulatory safe harbors under the federal anti-kickback statute protecting certain common manufacturer business arrangements and activities from prosecution, the exceptions and safe harbors are drawn narrowly and an arrangement must meet all of the conditions specified in order to be fully protected from scrutiny under the federal anti-kickback statute. We seek to comply with the exceptions and safe harbors whenever possible, but our practices, such as our patient assistance programs and prompt pay discounts with certain customers, may not in all cases meet all of the criteria for protection from anti-kickback liability and may be subject to scrutiny.

The federal false claims laws, including the federal False Claims Act, prohibit any person or entity from knowingly presenting, or causing to be presented, a false claim for payment to the federal government, or knowingly making, or causing to be made, a false statement to get a false claim paid. Additionally, the civil monetary penalties statute imposes penalties against any person or entity that, among other things, is determined to have presented or caused to be presented a claim to a federal health program that the person knows or should know is for an item or service that was not provided as claimed or is false or fraudulent. Many pharmaceutical and other health care companies have been investigated and have reached substantial financial settlements with the federal government under the federal False Claims Act for a variety of alleged marketing activities, including providing free product to customers with the expectation that the customers would bill federal programs for the product; providing consulting fees, grants, free travel, and other benefits to physicians to induce them to prescribe the company's products; and inflating prices reported to private price publication services, which may be used by states to set drug payment rates under government health care programs. Companies have been prosecuted for causing false claims to be submitted because of the marketing of their products for unapproved uses. Pharmaceutical and other health care companies have also been prosecuted on other legal theories of Medicare and Medicaid fraud.

Legislative and regulatory proposals have been made to expand post-approval requirements and restrict sales and promotional activities for pharmaceutical products. It is not clear 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 on the marketing approvals of any Retrophin products, if any, may be. In addition, increased scrutiny by the U.S. Congress of the FDA's approval process may significantly delay or prevent marketing approval, as well as subject Retrophin to more stringent product labeling and post-marketing testing and other requirements.

We also could become subject to government investigations and related subpoenas. Such subpoenas are often associated with previously filed qui tam actions, or lawsuits filed under seal under the federal False Claims Act. Qui tam actions are brought by private plaintiffs suing on behalf of the federal government for alleged violations of the federal False Claims Act. The time and expense associated with responding to such 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. Responding to government investigations, defending any claims raised, and any resulting fines, restitution, damages and penalties, settlement payments or administrative actions, as well as any related actions brought by stockholders or other third parties, could have a material impact on our reputation, business and financial condition and divert the attention of our management from operating our business.

The number and complexity of both federal and state laws continues to increase, and additional governmental resources are being added to enforce these laws and to prosecute companies and individuals who are believed to be violating them. In particular, the PPACA 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, amendments to the federal False Claims Act that make it easier for the government and whistleblowers to pursue cases for alleged kickback and false claim violations and, for payments made on or after August 1, 2013, public reporting of payments by pharmaceutical manufacturers to physicians and teaching hospitals nationwide. We anticipate that government scrutiny of pharmaceutical sales and marketing practices will continue for the foreseeable future and subject us to the risk of further government investigations and enforcement actions. 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.

The U.S. Foreign Corrupt Practices Act, and similar worldwide anti-bribery laws generally prohibit companies and their intermediaries from making improper payments to government officials for the purpose of obtaining or retaining business. Our policies mandate compliance with these anti-bribery laws. We operate in parts of the world that have experienced governmental corruption to some degree and in certain circumstances, strict compliance with antibribery laws may conflict with local customs and practices or may require us to interact with doctors and hospitals, some of which may be state controlled, in a manner that is different than in the United States. We cannot assure that our internal control policies and

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procedures will protect us from reckless or criminal acts committed by our employees or agents. Violations of these laws, or allegations of such violations, could disrupt our business and result in criminal or civil penalties or remedial measures, any of which could have a material adverse effect on our business, financial condition and results of operations and could cause the market value of our common stock to decline.

The federal Health Insurance Portability and Accountability Act of 1996 ("HIPAA"), created new federal criminal statutes that prohibit, among other actions, knowingly and willfully executing, or attempting to execute, a scheme to defraud any healthcare benefit program, including private third-party payers, and knowingly and willfully falsifying, concealing or covering up a material fact or making any materially false, fictitious or fraudulent statement in connection with the delivery of or payment for healthcare benefits, items or services. Like the federal anti-kickback statute, the PPACA amended the intent standard for certain healthcare fraud provisions under HIPAA such that a person or entity no longer needs to have actual knowledge of the statute or specific intent to violate it in order to have committed a violation.

Additionally, the federal Physician Payments Sunshine Act within the PPACA, and its implementing regulations, require that certain manufacturers of drugs, devices, biologicals and medical supplies to report annually information related to certain payments or other transfers of value made or distributed to physicians and teaching hospitals, or to entities or individuals at the request of, or designated on behalf of, physicians and teaching hospitals and to report annually certain ownership and investment interests held by physicians and their immediate family members. Moreover, the Drug Supply Chain Security Act imposes new obligations on manufacturers of pharmaceutical products related to product tracking and tracing. We are not sure whether additional legislative changes will be enacted, or whether the current regulations, guidance or interpretations will be changed, or what the impact of such changes on our business, if any, may be.

Also, many states have similar fraud and abuse statutes or regulations, including state anti-kickback and false claims laws, that apply to items and services reimbursed under Medicaid and other state programs, or, in several states, apply regardless of the payer. Further, certain states require implementation of commercial compliance programs and marketing codes, compliance with the pharmaceutical industry's voluntary compliance guidelines, and compliance with the applicable compliance guidance promulgated by the federal government. Other various state level requirements include restricting payments or the provision of other items of value that may be made to healthcare providers and other potential referral sources; restricting various marketing practices; requiring prescription drug companies to report expenses relating to the marketing and promotion of drug products; requiring the posting of information relating to clinical studies and their outcomes; requiring the registration of sales representatives; requiring the reporting of certain information related to drug pricing; and requiring drug manufacturers to track and report information related to payments, gifts, compensation, and other items of value to physicians and other healthcare providers.

In addition, we may be subject to data privacy and security regulation by both the federal government and the states in which we conduct our business. HIPAA, as amended by the Health Information Technology for Economic and Clinical Health Act ("HITECH"), and their respective implementing regulations, imposes specified requirements relating to the privacy, security and transmission of individually identifiable health information. Among other things, HITECH, through its implementing regulations, makes certain of HIPAA's privacy and security standards directly applicable to business associates, defined as a person or organization, other than a member of a covered entity's workforce, that creates, receives, maintains or transmits protected health information for or on behalf of a covered entity for a function or activity regulated by HIPAA. International data protection laws also impose strict obligations on the ability to process health related and other personal information of citizens of member states, including in relation to collection, analysis and transfer. The EU General Data Protection Regulation was officially adopted in April 2016 and has been in effect since May 2018. The EU General Data Protection Regulation introduced new data protection requirements in the European Union, as well as substantial fines for breaches of the data protection rules. The EU General Data Protection Regulation 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 to ensure compliance with the new EU data protection rules. Additionally, California recently enacted legislation known as the California Consumer Privacy Act (the "CCPA"), which creates new individual privacy rights for consumers (as that word is broadly defined in the law) and places increased privacy and security obligations on entities handling personal data of consumers or households. When it goes into effect on January 1, 2020, the CCPA will require covered companies to provide new disclosures to California consumers, provide such consumers new ways to opt-out of certain sales of personal information, and allow for a new cause of action for data breaches. Legislators have stated that amendments will be proposed to the CCPA before it goes into effect, but it remains unclear what, if any, modifications will be made to this legislation or how it will be interpreted. As currently written, the CCPA will likely impact (possibly significantly) our business activities and exemplifies the vulnerability of our business to not only cyber threats but also the evolving regulatory environment related to personal data and protected health information.

If we or any of our partners fail to comply with applicable regulatory requirements, we or they could be subject to a range of regulatory actions that could affect our or our partners' ability to commercialize our products and could harm or prevent sales of the affected products, or could substantially increase the costs and expenses of commercializing and marketing our products. Any threatened or actual government enforcement action could also generate adverse publicity and require that we devote substantial resources that could otherwise be used in other aspects of our business. Compliance with applicable federal and state laws is difficult and time consuming, and companies that violate them may face substantial penalties. The potential sanctions include criminal fines, civil monetary penalties, administrative penalties, disgorgement, exclusion from participation in federal health care programs, individual imprisonment, injunctions, recall or seizure of products, total or partial suspension of production, reputational harm, administrative burdens, additional oversight and reporting obligations if we become subject to a corporate integrity agreement or similar agreement to resolve allegation of non-compliance with these laws, diminished profits and future earnings, and the curtailment or restructuring of our operations, and other sanctions. Because of the breadth of these laws, it is possible that some of our business activities could be subject to challenge under one or more of these laws. Such a challenge, irrespective of the underlying merits of the challenge or the ultimate outcome of the matter, could have a material adverse effect on our business, financial condition, results of operations and growth prospects.

If we are not able to obtain and maintain required regulatory approvals, we will not be able to commercialize our products, and our ability to generate revenue will be materially impaired.

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Our product candidates, once approved, and the activities associated with their manufacture, marketing, distribution, and sales are subject to extensive regulation by the FDA and other regulatory agencies in the United States and by comparable authorities in other countries. Failure to adhere to regulations set out by these bodies for one or more of our commercial products could prevent us from commercializing the product candidate in the jurisdiction of the regulatory authority. We have only limited experience in meeting the regulatory requirements incumbent on the sale of drugs in the United States and elsewhere, and expect to rely on third-parties to assist us in these processes. If these third parties fail to adequately adhere to the regulations governing drug distribution and promotion we may be unable to sell our products, which could have a material effect on our ability to generate revenue.

Our product candidates and the activities associated with their development and commercialization, including testing, manufacture, safety, efficacy, recordkeeping, labeling, storage, approval, advertising, promotion, sale and distribution, are subject to comprehensive regulation by the FDA and other regulatory agencies in the United States and by comparable authorities in other countries. Failure to obtain regulatory approval for a product candidate will prevent us from commercializing the product candidate in the jurisdiction of the regulatory authority. We have only limited experience in filing and prosecuting the applications necessary to obtain regulatory approvals and expect to rely on third-party contract research organizations to assist us in this process.

Securing FDA approval requires the submission of extensive preclinical and clinical data and supporting information to the FDA for each therapeutic indication to establish the product candidate's safety and efficacy. Securing FDA approval also requires the submission of information about the product manufacturing process to, and successful inspection of manufacturing facilities by, the FDA. Our future products may not be effective, may be only moderately effective or may prove to have undesirable or unintended side effects, toxicities or other characteristics that may preclude our obtaining regulatory approval or prevent or limit commercial use.

Our product candidates may fail to obtain regulatory approval for many reasons, including:

- our failure to demonstrate to the satisfaction of the FDA or comparable regulatory authorities that a product candidate is safe and effective for a particular indication;
- the results of clinical trials may not meet the level of statistical significance required by the FDA or comparable regulatory authorities for approval;
- our inability to demonstrate that a product candidate's benefits outweigh its risks;
- our inability to demonstrate that the product candidate presents an advantage over existing therapies;
- the FDA's or comparable regulatory authorities' disagreement with the manner in which we interpret the data from preclinical studies or clinical trials;
- failure of the third-party manufacturers with which we contract for clinical or commercial supplies to satisfactorily complete an FDA pre-approval inspection of the facility or facilities at which the product is manufactured to assess compliance with the FDA's cGMP regulations to assure that the facilities, methods and controls are adequate to preserve the drug's identity, strength, quality and purity; and
- a change in the approval policies or regulations of the FDA or comparable regulatory authorities or a change in the laws governing the approval process.

The process of obtaining regulatory approvals is expensive, often takes many years, if approval is obtained at all, and can vary substantially based upon a variety of factors, including the type, complexity and novelty of the product candidates involved. Changes in regulatory approval policies during the development period, changes in or the enactment of additional statutes or regulations, or changes in regulatory review for each submitted product application may cause delays in the approval or rejection of an application. The FDA and non-United States regulatory authorities have substantial discretion in the approval process and may refuse to accept any application or may decide that our data is insufficient for approval and require additional preclinical, clinical or other studies. In addition, varying interpretations of the data obtained from preclinical and clinical testing could delay, limit or prevent regulatory approval of a product candidate. Any regulatory approval we ultimately obtain may be limited or subject to restrictions or post approval commitments that render the approved product not commercially viable. Any FDA or other regulatory approval of our product candidates, once obtained, may be withdrawn, including for failure to comply with regulatory requirements or if clinical or manufacturing problems follow initial marketing.

Our internal computer systems, or those of our CROs or other contractors and vendors who host our applications or consultants, may fail or suffer security breaches, which could result in a material disruption of our product development programs.

Despite the implementation of security measures, our internal computer systems and those of our CROs and other contractors or vendors who host our applications and those of our consultants are vulnerable to damage or disruption from computer viruses, software bugs, unauthorized access including cyber-attack, natural disasters, terrorism, war, and telecommunication, equipment and electrical failures. While we have not, to our knowledge, experienced any significant system failure, accident or security breach to date, if such an event were to occur and cause interruptions in our operations, it could result in a material disruption of our programs. For example, the loss of clinical trial data from completed or ongoing clinical trials for any of our product candidates could result in delays in our regulatory approval efforts and significantly increase our costs to recover or reproduce the data. To the extent that any disruption or security breach results in a loss of or damage to our data or applications, or inappropriate disclosure or theft of confidential or proprietary information, we could incur liability, the further development of our product candidates could be delayed, our competitive position could be compromised, or our business reputation could be harmed.

Comprehensive tax reform could adversely affect our business and financial condition.

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On December 22, 2017, President Trump signed into law the Tax Act of 2017 that significantly revises the Internal Revenue Code of 1986, as amended. The Tax Act of 2017, among other things, contains significant changes to corporate taxation, including reduction of the corporate tax rate from a top marginal rate of 35% to a flat rate of 21%, limitation of the tax deduction for interest expense to 30% of adjusted earnings (except for certain small businesses), limitation of the deduction for net operating losses to 80% of current year taxable income and elimination of net operating loss carrybacks, one time taxation of offshore earnings at reduced rates regardless of whether they are repatriated, elimination of U.S. tax on foreign earnings (subject to certain important exceptions), immediate deductions for certain new investments instead of deductions for depreciation expense over time, and modifying or repealing many business deductions and credits. Notwithstanding the reduction in the corporate income tax rate, the overall impact of the Tax Act of 2017 is uncertain and our business and financial condition could be adversely affected. In addition, it is uncertain if and to what extent various states will conform to the Tax Act of 2017. The impact of this tax reform on holders of our common stock is also uncertain and could be adverse. We urge our stockholders to consult with their legal and tax advisors with respect to the Tax Act of 2017 and the potential tax consequences of investing in or holding our common stock.

Our effective tax rate may fluctuate, and we may incur obligations in tax jurisdictions in excess of accrued amounts.

Our effective tax rate is derived from a combination of applicable tax rates in the various places that we operate. In preparing our financial statements, we estimate the amount of tax that will become payable in each of such places. Nevertheless, our effective tax rate may be different than experienced in the past due to numerous factors, including passage of the Tax Act of 2017, changes in the mix of our profitability from state to state, the results of examinations and audits of our tax filings, our inability to secure or sustain acceptable agreements with tax authorities, changes in accounting for income taxes and changes in tax laws. Any of these factors could cause us to experience an effective tax rate significantly different from previous periods or our current expectations and may result in tax obligations in excess of amounts accrued in our financial statements.

Our ability to use net operating losses to offset future taxable income may be subject to limitations.

As of December 31, 2018, we had federal net operating loss, or NOL, carryforwards of \$36.5 million. Our federal NOL carryforwards generated in tax years ending on or prior to December 31, 2017, are only permitted to be carried forward for 20 years under applicable U.S. tax law. Under the Tax Act of 2017, our federal NOLs generated in tax years ending after December 31, 2017, may be carried forward indefinitely, but the deductibility of such federal NOLs generated in tax years beginning after December 31, 2017, is limited. It is uncertain if and to what extent various states will conform to the Tax Act of 2017. In addition, under Sections 382 and 383 of the Internal Revenue Code of 1986, as amended, and corresponding provisions of state law, if a corporation undergoes an "ownership change," which is 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 NOL carryforwards and other pre-change U.S. tax attributes (such as research tax credits) to offset its post-change income or taxes may be limited. It is possible that we have experienced an ownership change limitation in the past. In addition, we may experience ownership changes in the future as a result of subsequent shifts in our stock ownership, some of which may be outside of our control.

As a result, our pre-2018 NOL carryforwards may expire prior to being used, and our NOL carryforwards generated in 2018 and thereafter will be subject to a percentage limitation. In addition, it is possible that we have in the past undergone, and in the future may undergo, additional ownership changes that could limit our ability to use all of our pre-change NOLs and other pre-change tax attributes (such as research tax credits) to offset our post-change income or taxes. Similar provisions of state tax law may also apply to limit our use of accumulated state tax attributes. In addition, at the 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 a result, we may be unable to use all or a material portion of our NOLs and other tax attributes, which would harm our future operating results by effectively increasing our future tax obligations.

We are dependent on information technology systems, infrastructure and data, which exposes us to data security risks.

We are dependent upon our own or third-party information technology systems, infrastructure and data, including mobile technologies, to operate our business. The multitude and complexity of our computer systems may make them vulnerable to service interruption or destruction, disruption of data integrity, malicious intrusion, or random attacks. Likewise, data privacy or security incidents or breaches by employees or others may pose a risk that sensitive data, including our intellectual property, trade secrets or personal information of our employees, patients, customers or other business partners may be exposed to unauthorized persons or to the public. Cyber-attacks are increasing in their frequency, sophistication and intensity. Cyber-attacks could include the deployment of harmful malware, denial-of-service, social engineering and other means to affect service reliability and threaten data confidentiality, integrity and availability. Our business partners face similar risks and any security breach of their systems could adversely affect our security posture. A security breach or privacy violation that leads to disclosure or modification of or prevents access to patient information, including personally identifiable information or protected health information, could harm our reputation, compel us to comply with federal and/or state breach notification laws and foreign law equivalents, subject us to mandatory corrective action, require us to verify the correctness of database contents and otherwise subject us to litigation or other liability under laws and regulations that protect personal data, any of which could disrupt our business and/or result in increased costs or loss of revenue. Moreover, the prevalent use of mobile devices that access confidential information increases the risk of data security breaches, which could lead to the loss of confidential information, trade secrets or other intellectual property. While we have invested, and continue to invest, in the protection of our data and information technology infrastructure, there can be no assurance that our efforts will prevent service interruptions, or identify breaches in our systems, that could adversely affect our business and operations and/or result in the loss of critical or sensitive information, which could result in financial, legal, business or reputational harm to us. In addition, our liability insurance may not be sufficient in type or amount to cover us against claims related to security breaches, cyber-attacks and other related breaches.

Risks Related to our Indebtedness and Investments

Our indebtedness could adversely affect our financial condition.

As of December 31, 2018, we had approximately \$298.6 million of total debt outstanding, classified as short and long term. As a result of our indebtedness, a portion of our cash flow will be required to pay interest and principal on the 2019 Notes and 2025 Notes if the notes are not converted to shares of common stock prior to maturity. We may not generate sufficient cash flow from operations or have future borrowings available to enable us to repay our indebtedness or to fund other liquidity needs.

Our indebtedness pursuant to the 2019 Notes and 2025 Notes could have important consequences. For example, it could:

- make it more difficult for us to satisfy our obligations with respect to any other debt we may incur in the future;
- increase our vulnerability to general adverse economic and industry conditions;
- require us to dedicate a substantial portion of our cash flow from operations to payments on our indebtedness and related interest, thereby reducing the availability of our cash flow to fund working capital, capital expenditures and other general corporate purposes;
- limit our flexibility in planning for, or reacting to, changes in our business and the industry in which we operate;
- increase our cost of borrowing;
- place us at a competitive disadvantage compared to our competitors that may have less debt; and
- limit our ability to obtain additional financing for working capital, capital expenditures, acquisitions, debt service requirements or general corporate purposes.

We expect to use cash flow from operations and outside financings to meet our current and future financial obligations, including funding our operations, debt service and capital expenditures. Our ability to make these payments depends on our future performance, which will be affected by financial, business, economic and other factors, many of which we cannot control. Our business may not generate sufficient cash flow from operations in the future, which could result in our being unable to repay indebtedness, or to fund other liquidity needs. If we do not generate sufficient cash from operations, we may be forced to reduce or delay our business activities and capital expenditures, sell assets, obtain additional debt or equity capital or restructure or refinance all or a portion of our debt, including the 2019 Notes and 2025 Notes, on or before maturity. We cannot make any assurances that we will be able to accomplish any of these alternatives on terms acceptable to us, or at all. In addition, the terms of existing or future indebtedness may limit our ability to pursue any of these alternatives.

We may be unable to raise the funds necessary to repurchase the 2019 Notes and 2025 Notes for cash following a fundamental change, or to pay any cash amounts due upon conversion, and our future indebtedness may limit our ability to repurchase the 2019 Notes and 2025 Notes or pay cash upon their conversion.

Noteholders may require us to repurchase their 2025 Notes following a fundamental change at a cash repurchase price generally equal to the principal amount of the 2025 Notes to be repurchased, plus accrued and unpaid interest to, but excluding, the fundamental change repurchase date. In addition, upon conversion, we will satisfy part or all of our conversion obligation in cash unless we elect to settle conversions solely in shares of our common stock.

Further, the indenture governing the 2019 Notes contains a similar definition of fundamental change, and noteholders of the 2019 Notes will likely have the right to require us to repurchase their 2019 Notes for cash upon the occurrence of a fundamental change at a repurchase price equal to 100% of the principal amount of the 2019 Notes to be repurchased, plus accrued and unpaid interest, if any.

We may not have enough available cash or be able to obtain financing at the time we are required to repurchase the 2025 Notes and/or the 2019 Notes or pay the cash amounts due upon conversion of the 2025 Notes. In addition, applicable law, regulatory authorities and the agreements governing our future indebtedness may restrict our ability to repurchase the 2025 Notes and/or the 2019 Notes or pay the cash amounts due upon conversion of the 2025 Notes. Our failure to repurchase the 2025 Notes and/or the 2019 Notes or to pay the cash amounts due upon conversion of the 2025 Notes when required will constitute a default under the base and supplemental indentures that will govern the 2025 Notes, which we refer to collectively as the "indenture," and/or a default under the indenture governing the 2019 Notes. A default under the indenture and/or the indenture governing the 2019 Notes or the fundamental change itself could also lead to a default under agreements governing our other indebtedness, which may result in that other indebtedness becoming immediately payable in full. We may not have sufficient funds to satisfy all amounts due under the other indebtedness, the 2019 Notes and the 2025 Notes.

A default under the 2025 Notes may have a material adverse effect on our financial condition.

If an event of default under the 2025 Notes occurs, the principal amount of the 2025 Notes, plus accrued and unpaid interest (including additional interest, if any) may be declared immediately due and payable, subject to certain conditions set forth in the indenture governing such notes. Events of default include, but are not limited to:

- failure to pay (for more than 30 days) interest when due;
- failure to pay principal when due;
- failure to deliver shares of common stock upon conversion of a 2025 Notes;

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- failure to provide notice of a fundamental change;
- acceleration on our other indebtedness in excess of \$10 million (other than indebtedness that is non-recourse to us); or
- certain types of bankruptcy or insolvency involving us.

Accordingly, the occurrence of a default under the 2025 Notes, unless cured or waived, may have a material adverse effect on our results of operations.

Provisions of the 2019 Notes and 2025 Notes could discourage an acquisition of us by a third party.

Certain provisions of the 2019 Notes and 2025 Notes could make it more difficult or more expensive for or prevent a third party to acquire us. Upon the occurrence of certain transactions constituting a fundamental change, holders of the 2019 Notes and 2025 Notes will have the right, at their option, to require us to repurchase all of their 2019 Notes and 2025 Notes or any portion of the principal amount of such Notes in integral multiples of \$1,000. We may also be required to increase the conversion rate for conversions in connection with certain fundamental changes.

Conversion of the Notes may dilute the ownership interest of existing stockholders, including holders who had previously converted their 2019 Notes and 2025 Notes.

To the extent we issue shares of common stock upon conversion of the 2019 Notes and 2025 Notes, the conversion of some or all of the 2019 Notes and 2025 Notes will dilute the ownership interests of existing stockholders. Any sales in the public market of shares of the common stock issuable upon such conversion could adversely affect prevailing market prices of shares of our common stock. In addition, the existence of the 2019 Notes and 2025 Notes may encourage short selling by market participants because the conversion of the 2019 Notes and 2025 Notes could depress the price of shares of our common stock.

ITEM 1B. UNRESOLVED STAFF COMMENTS

None.

ITEM 2. PROPERTIES

We lease the following locations to conduct our business:

Location	Address	Lease Expiration	Square Feet
San Diego, California (corporate headquarters)	3721/3661 Valley Centre Drive Suites 200, 225, 250 & 275	July 31, 2024	45,446

We believe these facilities are adequate to conduct our business.

ITEM 3. LEGAL PROCEEDINGS

The information required by this Item is incorporated herein by reference to Note 11 of the Consolidated Financial Statements included in this report.

ITEM 4. MINE SAFETY DISCLOSURES

Not applicable.

PART II

ITEM 5. MARKET FOR REGISTRANT'S COMMON EQUITY, RELATED STOCKHOLDER MATTERS AND ISSUER PURCHASES OF EQUITY SECURITIES

Market Information

Our common stock is listed for quotation on the Nasdaq Global Market under the trading symbol "RTRX" and is part of the Nasdaq Biotechnology Index (Nasdaq: NBI).

As of February 25, 2019, the last reported sale price of our Common Stock as reported by the Nasdaq was \$21.39.

As of February 25, 2019, we had approximately 192 holders of record of our common stock.

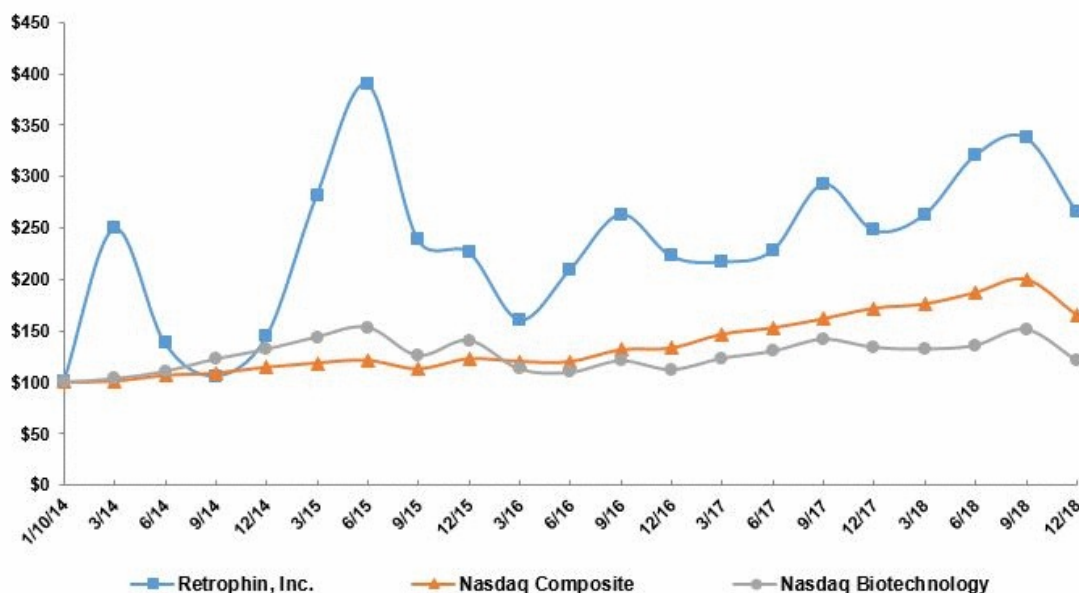
Performance Graph

The following is not deemed "filed" with the Securities and Exchange Commission and is not to be incorporated by reference into any filing we make under the Securities Act of 1933, as amended, whether made before or after the date hereof and irrespective of any general incorporation by reference language in such filing.

Our common stock is traded on the Nasdaq Global Market and is a component of both the Nasdaq Composite Index and the Nasdaq Biotechnology Index. The total return for our common stock and for each index assumes the reinvestment of dividends, although dividends have never been declared on our common stock, and is based on the returns of the component companies weighted according to their capitalizations as of the end of each monthly period. The Nasdaq-Composite tracks the aggregate price performance of equity securities of companies traded on the Nasdaq National Market. The Nasdaq Biotechnology Index contains securities and tracks the aggregate price performance of equity securities of Nasdaq-listed companies classified according to the Industry Classification Benchmark as either Biotechnology or Pharmaceuticals which also meet other eligibility criteria. The comparisons shown in the graph are based upon historical data and we caution that the stock price performance shown in the graph is not indicative of, nor intended to forecast, the potential future performance of our stock.

COMPARISON OF 5 YEAR CUMULATIVE TOTAL RETURN*

Among Retrophin, Inc., the Nasdaq Composite Index and the Nasdaq Biotechnology Index



*\$100 invested on 1/10/14 in stock or 12/31/13 in index, including reinvestment of dividends. Fiscal year ending December 31.

Dividends

Since inception we have not paid any dividends on our common stock. We currently do not anticipate paying any cash dividends in the foreseeable future on our common stock. Although we intend to retain our earnings, if any, to finance the exploration and growth of our business, our Board of Directors will have the discretion to declare and pay dividends in the future. Payment of dividends in the future will depend upon our earnings, capital requirements and other factors which our Board of Directors may deem relevant.

ITEM 6. SELECTED FINANCIAL DATA

The following table presents selected historical financial data of the Company for the periods indicated. The selected historical financial information is derived from the audited Consolidated Financial Statements of the Company referred to under Item 8 of this Annual Report on Form 10-K, and previously published historical financial statements. The following selected financial data should be read in conjunction with Item 7 - Management's Discussion and Analysis of Financial Condition and Results of Operations, and the Company's Consolidated Financial Statements, including the notes thereto, included elsewhere herein.

Selected historical financial data (in thousands, except share and per share amounts):

Consolidated Statement of Operations:	For the year ended December 31,				
	2018	2017	2016	2015	2014
Net product sales	\$ 164,246	\$ 154,937	\$ 133,591	\$ 99,892	\$ 28,203
Total operating expenses	244,286	208,728	191,805	150,640	108,011
Operating loss	(80,040)	(53,791)	(58,214)	(50,748)	(79,808)
Total other income (expenses), net	(21,827)	(4,572)	632	156,215	(33,590)
Income (Loss) before benefit (provision) for income taxes	(101,867)	(58,363)	(57,582)	105,467	(113,398)
Income tax benefit (provision)	(811)	(1,368)	9,679	11,770	2,460
Net income (loss)	\$ (102,678)	\$ (59,731)	\$ (47,903)	\$ 117,237	\$ (110,938)
Per Share Data:					
Net Income (loss) per common share, basic	\$ (2.54)	\$ (1.54)	\$ (1.29)	\$ 3.49	\$ (4.43)
Net Income (loss) per common share, diluted	\$ (2.54)	\$ (1.54)	\$ (1.29)	\$ 3.17	\$ (4.43)
Weighted average common shares outstanding, basic	40,433,171	38,769,816	36,997,865	33,560,249	25,057,509
Weighted average common shares outstanding, diluted	40,433,171	38,769,816	38,288,012	37,581,439	25,057,509

Balance Sheet data:	As of December 31,				
	2018	2017	2016	2015	2014
Cash, cash equivalents and marketable securities	\$ 471,541	\$ 300,630	\$ 255,873	\$ 229,604	\$ 27,760
Working capital (deficit)	391,057	240,139	249,090	214,951	(70,205)
Total assets	709,160	520,346	525,282	512,264	134,973
Long-term debt	195,091	45,077	44,422	43,766	42,790
Total stockholders' equity (deficit)	\$ 318,253	\$ 293,134	\$ 307,767	\$ 299,971	\$ (37,251)

Note: Cash dividends were not paid during the above periods.

ITEM 7. MANAGEMENT'S DISCUSSION AND ANALYSIS OF FINANCIAL CONDITION AND RESULTS OF OPERATIONS

The following discussion should be read in conjunction with our audited Consolidated Financial Statements, including the notes thereto.

Overview

We are a biopharmaceutical company headquartered in San Diego, California, focused on identifying, developing and delivering life-changing therapies to people living with rare diseases.

Research and Development Programs:

Fosmetpantotenate

We are evaluating fosmetpantotenate, a novel small molecule, in a Phase 3 clinical study as a potential treatment for PKAN. PKAN is a genetic neurodegenerative disorder that is typically diagnosed in the first decade of life. Symptoms of PKAN include dystonia, dysarthria, rigidity, retinal degeneration, and severe digestive problems. PKAN is estimated to affect up to 5,000 patients worldwide. There are currently no viable treatment options for patients with PKAN. Fosmetpantotenate is a phosphopantothenate replacement therapy that aims to restore levels of this key substrate

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in PKAN patients. Certain international health regulators have approved the initiation of dosing fosmetpantotenate in PKAN patients under physician-initiated studies in accordance with local regulations in their respective countries.

In 2015 and 2016 we filed an IND, completed the Phase I clinical trial and obtained both orphan drug and fast track designation in the United States. Additionally, we received orphan drug designation in the European Union and reached an agreement with the FDA under the SPA process for a Phase 3 clinical trial for PKAN.

In the fourth quarter of 2018, the pivotal Phase 3 FORT study of fosmetpantotenate in PKAN completed patient enrollment. The FORT Study is designed to be registration-enabling in the U.S. and Europe, and we expect top-line data to become available in the third quarter of 2019.

Sparsentan

Sparsentan is an investigational product candidate with a dual mechanism of action, a potent ARB and a selective ERA, with in vitro selectivity toward endothelin receptor type A. We have secured a license to sparsentan from Ligand and BMS (who referred to it as DARA). Sparsentan is currently being evaluated in two pivotal Phase 3 clinical studies in the following indications:

- FSGS, a leading cause of end-stage renal disease and NS. There are currently no FDA approved pharmacologic treatments for FSGS and off-label treatments are limited to ACE/ARBs, steroids, and immunosuppressant agents, which are effective in only a subset of patients. Every year approximately 5,400 patients are diagnosed with FSGS and we estimate that there are up to 40,000 FSGS patients in the United States with approximately half of them being candidates for sparsentan. In 2015 and 2016 we received orphan drug designation in the United States and European Union and generated positive data from our Phase 2 DUET study in FSGS. In the second quarter of 2018, we announced the initiation of the Phase 3 DUPLEX study of sparsentan in FSGS, and enrollment continues. This pivotal DUPLEX Study is designed to include an interim analysis of modified partial remission of proteinuria. We expect that successful achievement of this endpoint will serve as the basis for Subpart H accelerated submission of sparsentan in the United States and CMA consideration in Europe. The confirmatory endpoint of the study will compare changes in slope of estimated glomerular filtration rate, or eGFR. Top-line data from the interim analysis are expected to become available in the second half of 2020.
- IgAN is characterized by hematuria, proteinuria, and variable rates of progressive renal failure. With an estimated prevalence of more than 100,000 people in the United States and greater numbers in Europe and Asia, IgAN is the most common primary glomerular disease. Most patients are diagnosed between the ages of 16 and 35, with up to 40% progressing to end stage renal disease within 15 years. There are currently no FDA approved treatments for IgAN. The current standard of care is renin-angiotensin-aldosterone system (RAAS) blockade with immunosuppression also being commonly used for patients with significant proteinuria or rapidly progressive glomerulonephritis. In the fourth quarter of 2018, we announced that the first patient had been dosed in the PROTECT Study, a global, pivotal Phase 3 clinical trial evaluating the long-term nephroprotective potential of sparsentan for the treatment of IgAN. This PROTECT Study is a global, randomized, multicenter, double-blind, parallel-arm, active-controlled Phase 3 clinical trial evaluating the safety and efficacy of sparsentan in approximately 280 patients with IgAN aged 18 years or older. The primary efficacy endpoint in the PROTECT Study is the change in proteinuria (urine protein-to-creatinine ratio) from baseline after 36 weeks of treatment. We expect that successful achievement of this endpoint will serve as the basis for submission of an NDA for sparsentan for the treatment of IgAN under the Subpart H accelerated submission pathway in the U.S. and CMA consideration in Europe. Secondary efficacy endpoints include change in eGFR from baseline to four weeks post-cessation of randomized treatment, as well as the rate of change in eGFR over 52-week and 104-week periods following the first six weeks of randomized treatment. Top-line data from the primary endpoint are expected to become available in the first half of 2022.

CNSA-001

Effective in the first quarter of 2018, we entered into a Future Acquisition Right and Joint Development Agreement with Censa. Pursuant to the agreement, we agreed to fund certain development activities of Censa's CNSA-001 program, in an aggregate amount expected to be approximately \$17 million through proof of concept, and has the right, but not the obligation, to acquire Censa (the "Option") on the terms and subject to the conditions set forth in a separate Agreement and Plan of Merger (the "Merger Agreement"). In exchange for the Option, we paid Censa \$10 million, and an additional \$5 million upon Censa's completion of a specified development milestone set forth in the Option Agreement.

If we exercise the Option, pursuant to the terms of the Merger Agreement, we will acquire Censa for \$65 million in upfront consideration, subject to certain adjustments, paid as a combination of 20% in cash and 80% in shares of our common stock, valued at a fixed price of \$21.40 per share; provided, however, that Censa may elect on behalf of its equityholders to receive the upfront consideration in 100% cash if the average price per share of our common stock for the ten trading days ending on the date we provide a notice of interest to exercise the Option is less than \$19.26. In addition to the upfront consideration, if we exercise the Option and acquire Censa, we would be required to make further cash payments to Censa's equityholders of up to an aggregate of \$25 million if the CNSA-001 program achieves specified development and commercial milestones.

Censa, a privately held biotechnology company focused on developing therapies for the orphan metabolic diseases, is developing CNSA-001 for the treatment of phenylketonuria (PKU). CNSA-001 is an orally bioavailable form of a natural precursor of BH4 with the potential to provide improved Phe reduction in patients with PKU when compared to BH4. Preclinical research has suggested CNSA-001 may provide improved bioavailability, plasma stability and tissue exposure, leading to higher intracellular BH4 levels and subsequent greater Phe reduction when compared to the current standard of care in PKU. In pre-clinical models, CNSA-001 has also shown an ability to cross the blood-brain barrier which, if supported by clinical data, may lead to broader utility in additional indications such as primary BH4 deficiency (PBD) and Segawa syndrome.

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PKU is a rare, genetic metabolic condition in which the body cannot breakdown Phe due to a missing or defective phenylalanine hydroxylase (PAH) enzyme. High Phe levels can lead to developmental and physical growth delay, executive function impairment, seizures, and microcephaly caused by toxic Phe accumulation in the brain. PKU is typically diagnosed at birth.

Censa is currently conducting a Phase 2 proof-of-concept study evaluating CNSA-001 in patients with PKU, and we expect top-line data to become available to us during the second quarter of 2019.

NGLY1 Deficiency Discovery Efforts

N-glycanase deficiency, or NGLY1 deficiency, is an extremely rare genetic disorder believed to be caused by a deficiency in an enzyme called N-glycanase-1, which is encoded by the gene NGLY1. The condition is characterized by symptoms such as developmental delays, seizures, complex hyperkinetic movement disorders, diminished reflexes and an inability to produce tears. There are no approved therapeutic options for NGLY1 deficiency, and current therapeutic strategies are limited to symptom management.

In September 2017, we entered a three-way CRADA with the National Institutes of Health's National Center for Advancing Translational Sciences (NCATS) and patient advocacy foundation NGLY1.org to collaborate on research efforts aimed at the identification of potential small molecule therapeutics for NGLY1 deficiency.

Liquid Ursodeoxycholic Acid

L-UDCA is a liquid formulation of ursodeoxycholic acid being developed for the treatment of a rare liver disease called PBC. We obtained L-UDCA in 2016 with the intention of making L-UDCA commercially available to the subset of PBC patients who have difficulty swallowing. There are no liquid formulations of ursodeoxycholic acid currently approved by the FDA.

We currently sell the following three products:

Chenodal (chenodiol tablets)

Chenodal is a synthetic oral form of chenodeoxycholic acid, a naturally occurring primary bile acid synthesized from cholesterol in the liver, indicated for the treatment of radiolucent stones in well-opacifying gallbladders in patients in whom selective surgery would be undertaken except for the presence of increased surgical risk due to systemic disease or age.

Chenodal administration is known to reduce biliary cholesterol and the dissolution of radiolucent gallstones through suppression of hepatic synthesis of cholesterol, cholic acid and deoxycholic acid in the bile pool. Chenodal was first approved by the FDA in 1983 for the management of gallstones but its marketing was later discontinued due to lack of commercial success. In 2009, Nexgen Pharma Inc.'s ANDA for Chenodal was approved by the FDA for the treatment of gallstones. Chenodal is manufactured under this ANDA. In 2010, Chenodal was granted orphan drug designation for the treatment of CTX, a rare autosomal recessive lipid storage disease. We acquired Chenodal in March 2014.

While Chenodal is not labeled for CTX, it has been used as the standard of care for over three decades. We are working to obtain FDA approval of Chenodal for the treatment of CTX. The prevalence of CTX is estimated in the literature to be as high as 1 in 70,000 in the overall population. Pathogenesis of CTX involves deficiency of the enzyme 27-hydroxylase (encoded by the gene CYP27A1), a rate-limiting enzyme in the synthesis of primary bile acids, including CDCA, from cholesterol. The disruption of primary bile acid synthesis in CTX leads to toxic accumulation of cholesterol and cholestanol in most tissues. Most patients present with intractable diarrhea, premature cataracts, tendon xanthomas, atherosclerosis, and cardiovascular disease in childhood and adolescence. Neurological manifestations of the disease, including dementia and cognitive and cerebellar deficiencies, emerge during late adolescence and adulthood. Oral administration of CDCA has been shown to normalize primary bile acid synthesis in patients with CTX.

Cholbam (cholic acid capsules)

The FDA approved Cholbam (cholic acid capsules) in March 2015, the first FDA approved treatment for pediatric and adult patients with bile acid synthesis disorders due to single enzyme defects, and for adjunctive treatment of patients with peroxisomal disorders (including Zellweger spectrum disorders). The effectiveness of Cholbam has been demonstrated in clinical trials for bile acid synthesis disorders and the adjunctive treatment of peroxisomal disorders. An estimated 200 to 300 patients are current candidates for therapy.

Thiola (tiopronin tablets)

Thiola is approved by the FDA for the treatment of cystinuria, a rare genetic cystine transport disorder that causes high cystine levels in the urine and the formation of recurring kidney stones. The resulting long-term damage can cause loss of kidney function in addition to substantial pain and loss of productivity associated with renal colic and stone passage. The prevalence of cystinuria in the United States is estimated to be 10,000 to 12,000, indicating that there may be as many as 4,000 to 5,000 affected individuals with cystinuria in the United States that would be candidates for Thiola. We are currently developing a new, more patient-friendly, formulation which has a PDUFA target action date of June 30, 2019.

Financial Overview

Research and Development Costs

Research and development costs include expenses related to sparsentan, fosmetpantotate, CNSA-001 and our other pipeline programs. We expense all research and development costs as they are incurred. Our research and development costs are comprised of salaries and bonuses, benefits, non-

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cash share-based compensation, license fees, milestones under license agreements, costs paid to third-party contractors to perform research, conduct clinical trials, and develop drug materials and delivery methods, and associated overhead expenses and facilities costs. We charge direct internal and external program costs to the respective development programs. We also incur indirect costs that are not allocated to specific programs because such costs benefit multiple development programs and allow us to increase our pharmaceutical development capabilities. These consist of internal shared resources related to the development and maintenance of systems and processes applicable to all of our programs.

We record expenses in connection with our clinical trials under contracts with contract research organizations (CROs) that support conducting and managing clinical trials. The financial terms and activities of these agreements vary from contract to contract and may result in uneven expense levels. Generally, these agreements set forth activities that drive the recording of expenses such as start-up, initiation activities, enrollment, treatment of patients, or the completion of other clinical trial activities.

Expenses related to clinical trials are accrued based on our estimates and/or representations from service providers regarding work performed, including actual level of patient enrollment, completion of patient studies and progress of the clinical trials. Other incidental costs related to patient enrollment or treatment are accrued when reasonably certain. If the amounts we are obligated to pay under our clinical trial agreements are modified (for instance, as a result of changes in the clinical trial protocol or scope of work to be performed), we adjust our accruals accordingly on a prospective basis. Revisions to our contractual payment obligations are charged to expense in the period in which the facts that give rise to the revision become reasonably certain.

We currently have three Phase 3 clinical trials in process that are in varying stages of activity, with ongoing non-clinical support studies. As such, clinical trial expenses will vary depending on the all the factors set forth above and may fluctuate significantly from quarter to quarter.

At any point in time, we typically have various early stage research and drug discovery projects. Our internal resources, employees and infrastructure are not directly tied to any one research or drug discovery project and are typically deployed across multiple projects. As such, we do not maintain information regarding costs incurred for these early stage research and drug discovery programs on a project-specific basis.

We routinely engage vendors and service providers for scientific research, clinical trial, regulatory compliance, manufacturing and other consulting services. We also make grants to research and non-profit organizations to conduct research which may lead to new intellectual properties that we may subsequently license under separately negotiated license agreements. Such grants may be funded in lump sums or installments.

The following table summarizes our research and development expenses during the years ended December 31, 2018, 2017 and 2016. The internal costs include personnel, facility costs, and discovery and research related activities associated with our pipeline. The external program costs reflect external costs attributable to our clinical development candidates and preclinical candidates selected for further development. Such expenses primarily include third-party contract costs relating to clinical trial activities, nonclinical studies and manufacturing.

	For the Year Ended December 31,		
	<i>(in thousands)</i>		
	2018	2017	2016
External service provider costs:			
Fosmetpantotenate	\$ 21,330	\$ 16,571	\$ 12,625
Sparsentan	39,826	20,237	21,064
Censa	16,831	—	—
Other product candidates	242	331	1,407
General	16,258	15,827	10,958
Total external service provider costs:	94,487	52,966	46,054
Internal personnel costs:	29,270	25,202	24,768
Total research and development	\$ 123,757	\$ 78,168	\$ 70,822

We expect our research and development expenses to increase during fiscal 2019 as we focus on clinical trials for our key product candidates, advance our discovery research projects into the preclinical stage and continue our early stage research. The process of conducting preclinical studies and clinical trials necessary to obtain regulatory approval is costly and time consuming.

Most of our product development programs are in clinical trials which are highly uncertain and may not result in approved products. Completion dates and completion costs can vary significantly for each product candidate and are difficult to project. Given the uncertainty associated with clinical trial enrollments and the risks inherent in the development process, we are unable to determine the duration and completion costs of current or future clinical trials of our product candidates, or if and to what extent we will generate revenues, if any, from the commercialization and sale of any of our product candidates.

Selling, General and Administrative

Selling, general and administrative expenses consist of salaries and bonuses, benefits, non-cash share-based compensation, legal and other professional fees, rent, depreciation and amortization, travel, insurance, business development, sales and marketing programs, and other operating expenses.

Other Income/Expenses

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Other income/expenses consists of interest income and expense, finance expense, loss on the extinguishment of debt, change in fair value of derivative instruments and miscellaneous other income/expenses.

License Agreements

Ligand License Agreement

In 2012, we entered into a license agreement with Ligand, granting us a worldwide license for the development, manufacture and commercialization of sparsentan, which we are developing in connection with the treatment of FSGS. Under the license agreement, Ligand granted us a sublicense under certain of its patents and other intellectual property in connection with the development and commercialization of sparsentan. Under the license agreement, Ligand is obligated to transfer to us certain information, records, regulatory filings, materials and inventory controlled by Ligand and relating to or useful for developing sparsentan. We must use commercially reasonable efforts to develop and commercialize sparsentan in specified major market countries and other countries in which we believe it is commercially reasonable to develop and commercialize such products.

As consideration for the license, we are required to make payments upon the achievement of certain milestones, totaling up to \$114.1 million. Should we commercialize sparsentan or any products containing any of the licensed compounds, we will be obligated to pay Ligand an escalating annual royalty between 15% and 17% of net sales of all such products. Through 2018, we made milestone payments to Ligand of \$7.2 million under the license agreement.

Under the terms of the license agreement, BMS has a right of first negotiation and Ligand has a right of second negotiation with respect to any license arrangement for a licensed compound, except to the extent such rights may be waived.

The license agreement will continue until neither party has any further payment obligations under the agreement and is expected to continue for approximately 10 to 20 years from the effective date. Ligand may terminate the license agreement due to (i) our insolvency, (ii) our material uncured breach of the agreement, (iii) our failure to use commercially reasonable efforts to develop and commercialize sparsentan as described above or (iv) certain other conditions. We may terminate the license agreement due to a material uncured breach of the agreement by Ligand.

Thiola License Agreement

In 2014, we entered into a license agreement with Mission, pursuant to which we obtained an exclusive, royalty-bearing license to market, sell and commercialize Thiola (tiopronin) in the United States and Canada, and a non-exclusive license to use know-how relating to Thiola to the extent necessary to market Thiola. We paid Mission an up-front license fee of \$3.0 million and will pay guaranteed minimum royalties during each calendar year the greater of \$2.0 million or 20% of our net sales of Thiola in the United States and Canada.

In October 2015, the license agreement was amended to allow for us to secure enough API to ensure an adequate level of safety stock to prevent an interruption in the supply of Thiola and to prepare for a reformulation development project.

In March 2016, the license agreement was amended to, among other things, include a new formulation development project for tiopronin tablets.

In November 2017, we amended the license agreement to extend the term through May of 2029.

In November 2018, the license agreement was amended to extend the territorial rights beyond the United States and Canada. As consideration for the expanded territory we paid an up-front fee of \$0.3 million and will pay guaranteed minimum royalties equaling the greater of \$0.1 million or 20% of our Thiola net sales generated from outside of the United States during each calendar year.

See Note 9 to Consolidated Financial Statements for further discussion.

Results of Operations

Net Product Sales

The following table provides information regarding net product sales (*in thousands*):

	Year Ended December 31,			Year Ended December 31,		
	2018	2017	Change	2017	2016	Change
Thiola	\$ 89,176	\$ 82,311	\$ 6,865	\$ 82,311	\$ 71,199	\$ 11,112
Bile acid products	75,070	72,626	2,444	72,626	62,392	10,234
Total net product revenues	\$ 164,246	\$ 154,937	\$ 9,309	\$ 154,937	\$ 133,591	\$ 21,346

Net product sales for the years ended December 31, 2018, 2017 and 2016 were \$164.2 million, \$154.9 million and \$133.6 million, respectively, and consisted of sales of Thiola, Chenodal and Cholbam ("Bile acid products"), less allowances for government and commercial rebates and patient assistance programs.

The increase in net product sales for the year ended December 31, 2018 as compared to the same period in 2017, is due to increased patient counts for all products.

The increase in net product sales for the year ended December 31, 2017 as compared to the same period in 2016, is due to increased patient counts for all products.

We use a direct-to-patient distributor. Under this distribution model, we record revenues when customers take control of the product (at delivery).

Operating Expenses

The following table provides information regarding operating expenses (*in thousands*):

	Year Ended December 31,			Year Ended December 31,		
	2018	2017	Change	2017	2016	Change
Cost of goods sold	\$ 5,527	\$ 3,605	\$ 1,922	\$ 3,605	\$ 4,554	\$ (949)
Research and development	123,757	78,168	45,589	78,168	70,822	7,346
Selling, general and administrative	103,654	101,333	2,321	101,333	91,941	9,392
Change in fair value of contingent consideration	11,590	19,389	(7,799)	19,389	18,383	1,006
Restructuring	(242)	3,608	(3,850)	3,608	893	2,715
Legal fee settlement	—	2,625	(2,625)	2,625	5,212	(2,587)
	<u>\$ 244,286</u>	<u>\$ 208,728</u>	<u>\$ 35,558</u>	<u>\$ 208,728</u>	<u>\$ 191,805</u>	<u>\$ 16,923</u>

2018 versus 2017 results

Operating expenses for the year ended December 31, 2018, were \$244.3 million compared to \$208.7 million for the year ended December 31, 2017, an increase of \$35.6 million.

Cost of goods sold increased by \$1.9 million due primarily to higher inventory reserves.

Research and development costs increased by \$45.6 million due to ongoing clinical trials for sparsentan, fosmetpantotenate and CNSA-001, and non-clinical studies to support the clinical trials.

Selling, general and administrative expenses increased by \$2.3 million, which reflects additional marketing initiatives to support our commercial portfolio and corporate initiatives.

The following table provides the change in the fair value of contingent consideration of \$7.8 million, which was due to changes in revenue forecasts, discount factors and timing of payments (*in thousands*):

	Year Ended December 31,		
	2018	2017	Change
Chenodal	\$ 6,556	\$ 13,446	\$ (6,890)
Cholbam	4,034	11,643	(7,609)
L-UDCA	1,000	(5,700)	6,700
	<u>\$ 11,590</u>	<u>\$ 19,389</u>	<u>\$ (7,799)</u>

Restructuring expense decreased \$3.9 million as we completed the consolidation of our research and development function to San Diego in 2017.

Legal fee settlement expense of \$2.6 million in 2017 relates to amounts we advanced for legal fees to our former Chief Executive Officer in defense of litigation for his actions while holding that title. See Note 11 to the Consolidated Financial Statements for further discussion.

2017 versus 2016 results

Our operating expenses for the year ended December 31, 2017 were \$208.7 million compared to \$191.8 million for the year ended December 31, 2016, an increase of \$16.9 million.

Cost of goods sold decreased by \$0.9 million due to higher inventory reserves and a one-time termination fee from a distribution partner in 2016, partially offset by increased product sales.

Research and development costs increased by \$7.3 million due to costs associated with clinical trial activity for fosmetpantotenate and sparsentan, and non-clinical studies to support the clinical trials.

Selling, general and administrative expenses increased by \$9.4 million due to increases in professional fees of \$3.3 million, selling and marketing expenses of \$3.4 million, intangible asset amortization of \$1.3 million, and compensation expense of \$2.0 million, offset by miscellaneous decreases of \$0.6 million.

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The following table provides the change in the fair value of contingent consideration of \$1.0 million, which was due to changes in revenue forecasts, discount factors and timing of payments (*in thousands*):

	Year Ended December 31,		
	2017	2016	Change
Chenodal	\$ 13,446	\$ 15,743	\$ (2,297)
Cholbam	11,643	4,940	6,703
L-UDCA	(5,700)	(2,300)	(3,400)
	<u>\$ 19,389</u>	<u>\$ 18,383</u>	<u>\$ 1,006</u>

Restructuring expense increased \$2.7 million due to the consolidation of our research and development function to San Diego in 2017.

Legal fee settlement expense of \$2.6 million relates to amounts we advanced for legal fees to our former Chief Executive Officer in defense of litigation for his actions while holding that title. See Note 11 to the Consolidated Financial Statements for further discussion.

Other Income/Expenses

The following table provides information regarding other income (expenses) (*in thousands*):

	Year Ended December 31,			Year Ended December 31,		
	2018	2017	Change	2017	2016	Change
Other income (expense), net	\$ (474)	\$ 1,107	\$ (1,581)	\$ 1,107	\$ (264)	\$ 1,371
Interest income	5,499	3,234	2,265	3,234	3,975	(741)
Interest expense	(9,810)	(4,422)	(5,388)	(4,422)	(4,734)	312
Loss on extinguishment of debt	(17,042)	—	(17,042)	—	—	—
Change in fair value of derivative instruments	—	(4,491)	4,491	(4,491)	1,655	(6,146)
	<u>\$ (21,827)</u>	<u>\$ (4,572)</u>	<u>\$ (17,255)</u>	<u>\$ (4,572)</u>	<u>\$ 632</u>	<u>\$ (5,204)</u>

Other expense for the year ended December 31, 2018 was \$21.8 million compared to other expense of \$4.6 million for the year ended December 31, 2017, which represents an increase of \$17.3 million. The change was primarily attributable to the loss on the partial extinguishment of our 4.5% senior convertible notes due in 2019 and the increase in interest expense related to the 2.50% senior convertible notes due 2025, offset by a loss on the change in fair value of derivative instruments from 2017.

Other expense for the year ended December 31, 2017 was \$4.6 million compared to other income of \$0.6 million for the year ended December 31, 2016, which represents a variance of \$5.2 million. The change was primarily attributable to the change in fair value of derivative instruments, driven by changes in our stock price.

Income Tax Benefit (Provision):

We follow ASC 740, Income Taxes, which requires recognition of deferred tax assets and liabilities for the expected future tax consequences of events that have been included in the financial statements or tax returns. Under this method, deferred tax assets and liabilities are based on the differences between the financial statement and tax basis of assets and liabilities using enacted tax rates in effect for the year in which the differences are expected to reverse. Deferred tax assets are reduced by a valuation allowance to the extent management concludes it is more likely than not that the asset will not be realized.

The standard addresses the determination of whether tax benefits claimed or expected to be claimed on a tax return should be recorded in the financial statements. Under ASC 740, we may recognize the tax benefit from an uncertain tax position only if it is more likely than not that the tax position will be sustained on examination by the tax authorities, based on the technical merits of the position. The tax benefits recognized in the financial statements from such a position should be measured based on the largest benefit that has a greater than fifty percent likelihood of being realized upon ultimate settlement. ASC 740 also provides guidance on de-recognition, classification, interest and penalties on income taxes, accounting in interim periods and requires increased disclosures. Our policy is to record estimated interest and penalty related to the underpayment of income taxes or unrecognized tax benefits as a component of its income tax provision.

Tax expense for fiscal 2018 of \$0.8 million decreased from the fiscal 2017 tax expense of \$1.4 million primarily due to the impacts from the Tax Act of 2017.

Off-Balance Sheet Arrangements

We do not have any off-balance sheet arrangements, except for operating leases.

Liquidity and Capital Resources

We believe that our available cash and short-term investments as of the date of this filing will be sufficient to fund our anticipated level of operations in the near term. Management believes that our operating results will vary from quarter to quarter and year to year depending upon various factors including revenues, general and administrative expenses, and research and development expenses.

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For the years ended December 31, 2018 and 2017, we had the following financial performance (*in thousands*):

	December 31, 2018	December 31, 2017
Revenue	\$ 164,246	\$ 154,937
Net loss	(102,678)	(59,731)
Cash & cash equivalents	102,873	99,394
Short term investments	368,668	201,236
Accumulated deficit	(270,017)	(177,655)
Stockholders' equity	318,253	293,134
Working capital	\$ 391,057	\$ 240,139
Working capital ratio	4.74	3.80

Borrowings

Convertible Senior Notes Due 2025

On September 10, 2018, we completed a registered underwritten public offering of \$276 million aggregate principal amount of 2.50% Convertible Senior Notes due 2025 ("2025 Notes"), and entered into a base indenture and supplemental indenture agreement ("2025 Indenture") with respect to the 2025 Notes. The 2025 Notes will mature on September 15, 2025 ("Maturity Date"), unless earlier repurchased, redeemed, or converted. The 2025 Notes are senior unsecured obligations of ours and bear interest at an annual rate of 2.50%, payable semi-annually in arrears on March 15 and September 15 of each year, beginning on March 15, 2019.

The net proceeds from the issuance of the 2025 Notes were approximately \$267.2 million, after deducting commissions and the offering expenses payable by us. A portion of the net proceeds from the 2025 Notes were used by us to repurchase \$23.4 million aggregate principal amount of its then-outstanding 4.5% senior convertible notes due in 2019 in privately-negotiated transactions.

The initial conversion rate for the 2025 Notes is 25.7739 shares of our common stock per \$1,000 principal amount of 2025 Notes, which represents an initial conversion price of approximately \$38.80 per share. If a "make-whole fundamental change" (as defined in the 2025 Indenture) occurs, then we will, in certain circumstances, increase the conversion rate for a specified period of time.

The 2025 Notes will be redeemable, in whole or in part, at our option at any time, and from time to time, on or after September 15, 2022 and, in the case of any partial redemption, on or before the 40th scheduled trading day before the Maturity Date, at a cash redemption price equal to the principal amount of the 2025 Notes to be redeemed, plus accrued and unpaid interest, if any, to, but excluding, the redemption date, but only if the last reported sale price per share of our common stock exceeds 130% of the conversion price on each of at least 20 trading days during the 30 consecutive trading days ending on, and including, the trading day immediately before the date we send the related redemption notice. If a fundamental change (as defined in the 2025 Indenture) occurs, then, subject to certain exceptions, holders may require us to repurchase their 2025 Notes at a cash repurchase price equal to the principal amount of the 2025 Notes to be repurchased, plus accrued and unpaid interest, if any, to, but excluding, the fundamental change repurchase date.

The effective interest rate on the liability components of the 2025 Notes for the period from the date of issuance was 7.7%.

Convertible Senior Notes Due 2019

On May 29, 2014, we entered into a Note Purchase Agreement relating to a private placement by us of \$46.0 million aggregate principal senior convertible notes due in 2019 (the "2019 Notes") which are convertible into shares of our common stock at an initial conversion price of \$17.41 per share. The conversion price is subject to customary anti-dilution protection. The 2019 Notes bear interest at a rate of 4.5% per annum, payable semiannually in arrears on May 15 and November 15 of each year. The 2019 Notes mature on May 30, 2019 unless earlier converted or repurchased in accordance with their terms, and there are no contractual payments due prior to that date. If the debt holders convert we would be required to issue 1,297,530 shares of common stock assuming that we had not undergone a fundamental change.

In September 2018, we used part of the net proceeds from the issuance of the 2025 Notes discussed above to repurchase \$23.4 million aggregate principal value of the 2019 Notes in privately-negotiated transactions for approximately \$40.2 million in cash. The partial repurchase of the 2019 Notes resulted in a \$17.0 million loss on early extinguishment of debt, reflected in the Consolidated Statement of Operations and Comprehensive Income (Loss). At December 31, 2018, approximately \$22.6 million of principal remained outstanding on the 2019 Notes.

Interest Expense

Total interest expense recognized for the years ended December 31, 2018, 2017 and 2016 was \$9.8 million, \$4.4 million, and \$4.7 million, respectively.

License Agreement Obligations

See discussion above under the header "License Agreements".

Funding Requirements

We believe that our available cash and short-term investments as of the date of this filing will be sufficient to fund our anticipated level of operations for the near term. This belief is based on many factors; however, some factors are beyond our control. Factors affecting our financing requirements include, but are not limited to:

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- revenue growth of our marketed products;
- the rate of progress and cost of our clinical trials, preclinical studies and other discovery and research and development activities;
- the timing of, and costs involved in, seeking and obtaining marketing approvals for our products, and in maintaining quality systems standards for our products;
- our ability to manufacture sufficient quantities of our products to meet expected demand;
- the costs of preparing, filing, prosecuting, maintaining and enforcing any patent claims and other intellectual property rights, litigation costs and the results of litigation;
- our ability to enter into collaboration, licensing or distribution arrangements and the terms and timing of these arrangements;
- the potential need to expand our business, resulting in additional payroll and other overhead expenses;
- the potential acquisition or in-licensing of other products or technologies; and
- the emergence of competing products or other adverse market developments.

Future capital requirements will also depend on the extent to which we acquire or invest in additional complementary businesses, products and technologies.

Cash Flows

The following table summarizes our cash flows for the periods set forth below (*in thousands*):

	Twelve Months Ended December 31,		
	2018	2017	2016
Net cash provided by (used in) operating activities	\$ (24,958)	\$ 7,403	\$ (3,441)
Net cash provided by (used in) investing activities	(203,291)	45,602	10,370
Net cash provided by (used in) financing activities	231,863	5,445	(3,849)
Net increase in cash	3,614	58,450	3,080
Effect of exchange rate changes on cash	(135)	(58)	117
Cash & cash equivalents, beginning of period	99,394	41,002	37,805
Cash & cash equivalents, end of period	\$ 102,873	\$ 99,394	\$ 41,002

Management considers marketable securities to be available to fund current operations, and they are classified as available for sale and included within current assets in our Consolidated Balance Sheets. Therefore, cash on-hand includes marketable securities and was \$471.5 million as of December 31, 2018.

Cash Flows from Operating Activities

Operating activities used \$25.0 million of cash during the year ended December 31, 2018 compared to \$7.4 million of cash provided for the year ended December 31, 2017. After excluding non-cash adjustments, the variance is primarily due to changes in operating accounts, payments related to the change in fair value of contingent consideration, and increased research and development expenses.

Operating activities provided \$7.4 million of cash during the year ended December 31, 2017 compared to \$3.4 million of cash used for the year ended December 31, 2016. After excluding non-cash adjustments, the variance is primarily due to increased product sales exceeding increased operating expenses.

Cash Flows from Investing Activities

Cash used by investing activities for the year ended December 31, 2018 was \$203.3 million compared to \$45.6 million of cash provided for the year ended December 31, 2017. The variance of \$248.9 million was primarily driven by the proceeds from the issuance of the 2025 Notes being invested in marketable securities, offset by higher maturities for marketable securities in 2018 and proceeds from the final note receivable payment of \$47.5 million in 2017.

Cash provided by investing activities for the year ended December 31, 2017 was \$45.6 million compared to \$10.4 million of cash provided for the year ended December 31, 2016. The variance of \$35.2 million is primarily due to proceeds from the maturity of the note receivable being retained in cash in 2017 versus being used to purchase marketable securities in 2016.

Cash Flows from Financing Activities

For the year ended December 31, 2018, cash provided by financing activities was \$231.9 million compared to cash provided of \$5.4 million during the year ended December 31, 2017. The variance is primarily due to net proceeds received from the issuance of the 2025 Notes offset by the repurchase of the 2019 Notes.

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For the year ended December 31, 2017, cash provided by financing activities was \$5.4 million compared to cash used of \$3.8 million during the year ended December 31, 2016. The variance is primarily due to lower contingent consideration payments in 2017, as well as an \$8.0 million payment in 2016 for a sales related milestone for Cholbam achieved in the third quarter of that year.

Contractual Commitments

The following table summarizes our principal contractual commitments, excluding open orders that support normal operations, as of December 31, 2018 (*in thousands*):

	Total	Less than 1 year	1-3 years	3-5 years	More than 5 years
Operating lease	\$ 14,025	\$ 2,343	\$ 4,899	\$ 5,198	\$ 1,585
Note payable, including contractual interest	367,351	32,014	18,000	18,000	299,337
Sales support services	2,221	416	833	833	139
Product supply contracts	7,710	4,365	2,355	990	—
Purchase order commitments	261	261	—	—	—
	<u>\$ 391,568</u>	<u>\$ 39,399</u>	<u>\$ 26,087</u>	<u>\$ 25,021</u>	<u>\$ 301,061</u>

Critical Accounting Policies and Estimates

See Note 2 to the Consolidated Financial Statements for discussion.

Recently Issued Accounting Pronouncements

See Note 2 to the Consolidated Financial Statements for discussion.

ITEM 7A. QUANTITATIVE AND QUALITATIVE DISCLOSURES ABOUT MARKET RISK

Our primary exposure to market risk is related to changes in interest rates. As of December 31, 2018, we had cash equivalents and marketable securities of approximately \$471.5 million, consisting of money market funds, U.S. government agency debt, corporate debt and commercial paper. This exposure to market risk is interest rate sensitivity, which is affected by changes in the general level of U.S. interest rates, particularly because our investments are in short-term marketable securities. Our marketable securities are subject to interest rate risk and will fall in value if market interest rates increase. Due to the short-term duration of our investment portfolio and the low risk profile of our investments, a one percent change in interest rates would have approximately a \$2.4 million impact on our investments. We carry our investments based on publicly available information.

ITEM 8. FINANCIAL STATEMENTS AND SUPPLEMENTARY DATA

The Consolidated Financial Statements and supplementary data of Retrophin, Inc. required by this Item are described in Item 15 of this Annual Report on Form 10-K and are presented beginning on page F-1.

ITEM 9. CHANGES IN AND DISAGREEMENTS WITH ACCOUNTANTS ON ACCOUNTING AND FINANCIAL DISCLOSURE

Not applicable.

ITEM 9A. CONTROLS AND PROCEDURES

We maintain disclosure controls and procedures that are designed to ensure that information required to be disclosed in our Exchange Act reports is recorded, processed, summarized and reported within the timelines specified in the SEC's rules and forms, and that such information is accumulated and communicated to our management, including our Chief Executive Officer and Chief Financial Officer, as appropriate, to allow timely decisions regarding required disclosure. In designing and evaluating the disclosure controls and procedures, management recognized that any controls and procedures, no matter how well designed and operated, can only provide reasonable assurance of achieving the desired control objectives, and in reaching a reasonable level of assurance, management necessarily was required to apply its judgment in evaluating the cost-benefit relationship of possible controls and procedures.

As required by SEC Rule 13a-15(b), we carried out an evaluation, under the supervision and with the participation of our management, including our Chief Executive Officer and Chief Financial Officer, of the effectiveness of the design and operation of our disclosure controls and procedures as of the end of the year covered by this report. Based on the foregoing, our Chief Executive Officer and Chief Financial Officer concluded that our disclosure controls and procedures were effective at the reasonable assurance level.

Management's Report on Internal Control Over Financial Reporting

Internal control over financial reporting refers to the process designed by, or under the supervision of, our Chief Executive Officer and Chief Financial Officer, and effected by our board of directors, management and other personnel, 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, and 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 our assets;
- (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 our receipts and expenditures are being made only in accordance with authorization of our management and directors; and
- (3) Provide reasonable assurance regarding prevention or timely detection of unauthorized acquisition, use or disposition of our assets that could have a material effect on the financial statements.

Internal control over financial reporting cannot provide absolute assurance of achieving financial reporting objectives because of its inherent limitations. Internal control over financial reporting is a process that involves human diligence and compliance and is subject to lapses in judgment and breakdowns resulting from human failures. Internal control over financial reporting also can be circumvented by collusion or improper management override. Because of such limitations, there is a risk that material misstatements may not be prevented or detected on a timely basis by internal control over financial reporting. However, these inherent limitations are known features of the financial reporting process. Therefore, it is possible to design into the process safeguards to reduce, though not eliminate, this risk. Management is responsible for establishing and maintaining adequate internal control over financial reporting for the company.

Management has used the framework set forth in the report entitled Internal Control-Integrated Framework (2013 framework) published by the Committee of Sponsoring Organizations of the Treadway Commission, known as COSO, to evaluate the effectiveness of our internal control over financial reporting. Based on this assessment, our Chief Executive Officer and Chief Financial Officer concluded that our internal control over financial reporting was effective as of December 31, 2018. BDO USA, LLP, our independent registered public accounting firm, has issued an attestation report on our internal control over financial reporting as of December 31, 2018, which is included herein.

Changes in Internal Control Over Financial Reporting

There have not been any changes in our internal control over financial reporting during the quarter ended December 31, 2018 that have materially affected, or are reasonably likely to materially affect, our internal control over financial reporting.

Report of Independent Registered Public Accounting Firm

Shareholders and Board of Directors

Retrophin, Inc.

San Diego, California

Opinion on Internal Control over Financial Reporting

We have audited Retrophin, Inc. and its subsidiaries' (the "Company's") internal control over financial reporting as of December 31, 2018, based on criteria established in *Internal Control - Integrated Framework (2013)* issued by the Committee of Sponsoring Organizations of the Treadway Commission (the "COSO criteria"). In our opinion, the Company maintained, in all material respects, effective internal control over financial reporting as of December 31, 2018, based on the COSO criteria.

We also have audited, in accordance with the standards of the Public Company Accounting Oversight Board (United States) ("PCAOB"), the consolidated balance sheets of the Company and subsidiaries as of December 31, 2018 and 2017, the related consolidated statements of operations and comprehensive income (loss), stockholders' equity (deficit), and cash flows for each of the three years in the period ended December 31, 2018, and the related notes and our report dated February 26, 2019 expressed an unqualified opinion thereon.

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 Item 9A, 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 U.S. federal securities laws and the applicable rules and regulations of the Securities and Exchange Commission and the PCAOB.

We conducted our audit of internal control over financial reporting 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, and testing and evaluating the design and operating effectiveness of internal control based on the assessed risk. Our audit also included 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.

/s/BDO USA, LLP

San Diego, California

February 26, 2019

ITEM 9B. OTHER INFORMATION

Executive Compensation

On February 25, 2019, the Compensation Committee (the "Compensation Committee") of the Board of Directors (the "Board") approved the following 2018 performance-based bonuses for our executive officers pursuant to the 2018 Executive Officer Annual Bonus Plan.

- Laura Clague, our Chief Financial Officer, was granted a performance-based bonus equal to \$185,150;
- Neil McFarlane, our Chief Operating Officer, was granted a performance-based bonus equal to \$228,436;
- Noah Rosenberg, our Chief Medical Officer, was granted a performance-based bonus equal to \$97,750 (pro-rated amount for partial year of service);
- William Rote, our SVP, Research & Development, was granted a performance-based bonus equal to \$194,258; and
- Elizabeth Reed, our SVP, General Counsel and Corporate Secretary, was granted a performance-based bonus equal to \$161,000.

Additionally, on February 25, 2019, the Compensation Committee approved the following annual base salary increases for our executive officers:

- Laura Clague had her annual base salary increased to \$422,625;
- Neil McFarlane had his annual base salary increased to \$511,498;
- Noah Rosenberg had his annual base salary increased to \$433,500;
- William Rote had his annual base salary increased to \$434,969; and
- Elizabeth Reed had her annual base salary increased to \$400,000.

Eric Dube, our President and Chief Executive Officer, joined the Company in 2019 and therefore was not eligible for a 2018 cash bonus or a 2019 annual base salary increase.

2019 Executive Officer Annual Bonus Plan

On February 25, 2019, the Compensation Committee approved the adoption of the 2019 Retrophin, Inc. Executive Officer Annual Bonus Plan (the "Bonus Plan"). Each participant in the Bonus Plan has been assigned a target bonus percentage of such participant's current base salary for 2019. Pursuant to the terms of the Bonus Plan, the target bonus percentage is set at 60% of base salary for our Chief Executive Officer and 50% of base salary for our other executive officers.

The amount payable to our Chief Executive Officer under the Bonus Plan will be based entirely on the determination by the Compensation Committee or the Board of the achievement by the Company of corporate performance goals. The amounts payable to each other executive officer participant under the Bonus Plan will be apportioned to 75% based on the determination by the Compensation Committee or the Board of the achievement by the Company of corporate performance goals and 25% based on the determination of the individual performance of the respective executive officer. Depending on actual corporate performance during 2019, the Compensation Committee or the Board may, in their sole discretion, determine a corporate goal achievement percentage under the Bonus Plan within a range between 0% and 150%.

The corporate performance goals under the Bonus Plan for 2019 relate to (i) total revenues, (ii) business development objectives, (iii) CMC/manufacturing objectives for our development and commercial stage programs, (iv) progress of the sparsentan Phase 3 FSGS clinical trial, (v) progress of the sparsentan Phase 3 IGA nephropathy trial, (vi) progress of the fosmetpantotenate program, (vii) commercial objectives, and (viii) objectives related to the investment community.

The foregoing description of the terms of the Bonus Plan is qualified in its entirety by reference to the Bonus Plan, a copy of which is attached hereto as Exhibit 10.32 and is incorporated herein by reference.

PART III

Item 10. DIRECTORS, EXECUTIVE OFFICERS AND CORPORATE GOVERNANCE

Information required by this item will be contained in our Definitive Proxy Statement for our 2019 Annual Meeting of Stockholders, to be filed pursuant to Regulation 14A with the Securities and Exchange Commission within 120 days of December 31, 2018. Such information is incorporated herein by reference.

Item 11. EXECUTIVE COMPENSATION

Information required by this item will be contained in our Definitive Proxy Statement for our 2019 Annual Meeting of Stockholders, to be filed pursuant to Regulation 14A with the Securities and Exchange Commission within 120 days of December 31, 2018. Such information is incorporated herein by reference.

Item 12. SECURITY OWNERSHIP OF CERTAIN BENEFICIAL OWNERS AND MANAGEMENT AND RELATED STOCKHOLDER MATTERS

Information required by this item will be contained in our Definitive Proxy Statement for our 2019 Annual Meeting of Stockholders, to be filed pursuant to Regulation 14A with the Securities and Exchange Commission within 120 days of December 31, 2018. Such information is incorporated herein by reference.

Item 13. CERTAIN RELATIONSHIPS AND RELATED TRANSACTIONS, AND DIRECTOR INDEPENDENCE

Information required by this item will be contained in our Definitive Proxy Statement for our 2019 Annual Meeting of Stockholders, to be filed pursuant to Regulation 14A with the Securities and Exchange Commission within 120 days of December 31, 2018. Such information is incorporated herein by reference.

Item 14. PRINCIPAL ACCOUNTING FEES AND SERVICES

Information required by this item will be contained in our Definitive Proxy Statement for our 2019 Annual Meeting of Stockholders, to be filed pursuant to Regulation 14A with the Securities and Exchange Commission within 120 days of December 31, 2018. Such information is incorporated herein by reference.

PART IV

ITEM 15. EXHIBITS AND FINANCIAL STATEMENT SCHEDULES

(a) The financial statements at page F-1 are incorporated by reference to a part of this Annual Report on Form 10-K.

Financial statement schedules are omitted because they are not applicable or the required information is shown in the financial statements or notes thereto.

(b) Exhibits: The exhibits to this report are listed in the exhibit index below.

Exhibit No.	Description
2.1	Asset Purchase Agreement, dated May 22, 2015, by and between Retrophin, Inc. and Sanofi (incorporated by reference to Exhibit 2.1 to the Company's Current report on Form 8-K filed with the SEC on May 27, 2015).
3.1	Certificate of Incorporation of the Company (incorporated by reference to Exhibit 3.1 to Amendment No. 2 to the Company's General Form for Registration of Securities on Form 10-12G, filed with the SEC on October 28, 2010).
3.2	Certificate of Amendment of Certificate of Incorporation of the Company (incorporated by reference to Exhibit 3.1 to the Company's Current Report on Form 8-K, filed with the SEC on June 11, 2015).
3.3	Amended and Restated Bylaws of the Company (incorporated by reference to Exhibit 3.2 to the Company's Current Report on Form 8-K, filed with the SEC on June 11, 2015).
4.1	Form of Note Purchase Agreement for principal senior convertible notes with an interest rate of 4.50% due 2019 ("2019 Notes"), dated May 29, 2014, by and among the Company and the investors identified therein (incorporated by reference to Exhibit 10.1 to the Company's Current Report on Form 8-K, filed with the SEC on June 4, 2014).
4.2	Form of Indenture for 2019 Notes, dated May 30, 2014 (incorporated by reference to Exhibit 10.2 to the Company's Current Report on Form 8-K, filed with the SEC on June 4, 2014).
4.3	Form of Note for 2019 Notes, dated May 30, 2014 (incorporated by reference to Exhibit 10.3 to the Company's Current Report on Form 8-K, filed with the SEC on May 29, 2014).
4.4	Base Indenture, dated September 10, 2018, between the Company and U.S. Bank National Association, as Trustee (incorporated by reference to Exhibit 4.1 to the Company's Current Report on Form 8-K, filed with the SEC on September 10, 2018).
4.5	First Supplemental Indenture, dated September 10, 2018, between the Company and U.S. Bank National Association, as Trustee (including the form of 2.50% Convertible Senior Note due 2025) (incorporated by reference to Exhibit 4.2 to the Company's Current Report on Form 8-K, filed with the SEC on September 10, 2018).
10.1	Trademark License and Supply Agreement, dated May 29, 2014, by and between Retrophin, Inc. and Mission Pharmacal Company (incorporated by reference to Exhibit 10.1 to the Company's Current Report on Form 8-K, filed with the SEC on June 4, 2014).
10.2	First Amendment to Trademark License and Supply Agreement, effective as of July 28, 2014, by and between Mission Pharmacal Company and Retrophin, Inc. (incorporated by reference to Exhibit 10.1 to the Company's Current Report on Form 8-K, filed with the SEC on July 29, 2014).
10.3	International Rights Purchase Agreement, dated as of March 26, 2014, by and between Manchester Pharmaceuticals LLC and Retrophin Therapeutics International, LLC (incorporated by reference to Exhibit 10.1 to the Company's Current Report on Form 8-K, filed with the SEC on March 31, 2014).
10.4+	Sublicense Agreement, dated February 16, 2012, by and among Ligand Pharmaceuticals Incorporated, a Delaware corporation, Pharmacopeia, Inc., a Delaware limited liability company, and Retrophin, LLC, a Delaware limited liability company (incorporated by reference to Exhibit 10.1 to the Company's Current Report on Form 8-K, filed with the SEC on December 19, 2012).
10.5†	Employment Agreement, dated March 2, 2015, by and between Retrophin, Inc. and Laura M. Clague (incorporated by reference to Exhibit 10.25 to the Company's Annual Report on Form 10-K, filed with the SEC on March 11, 2015).
10.6	Retrophin, Inc. 2014 Incentive Compensation Plan as amended (incorporated by reference to Exhibit 99.1 to the Company's Current Report on Form 8-K, filed with the SEC on February 9, 2015).
10.7+	Amendment No. 4 to Sublicense Agreement dated as of September 17, 2015, between Retrophin, Inc. and Ligand Pharmaceuticals Incorporated (incorporated by reference to Exhibit 10.1 to the Company's Quarterly Report on Form 10-Q/A, filed with the SEC on December 22, 2015).
10.10	Addendum to Trademark License and Supply Agreement, dated October 19, 2015, by and between to Company and Mission Pharmacal (incorporated by reference to Exhibit 10.2 to the Company's Quarterly Report on Form 10-Q, filed with the SEC on November 6, 2015).
10.11	Asset Purchase Agreement dated as of January 9, 2015, between Retrophin, Inc. and Turing Pharmaceuticals AG (incorporated by reference to Exhibit 10.1 to the Company's Quarterly Report on Form 10-Q, filed with the SEC on May 11, 2015).
10.12	Asset Purchase Agreement dated as of February 12, 2015, among Retrophin, Inc., Manchester Pharmaceuticals LLC and Turing Pharmaceuticals AG (incorporated by reference to Exhibit 10.2 to the Company's Quarterly Report on Form 10-Q, filed with the SEC on May 11, 2015).
10.13+	Amendment No. 3 to Sublicense Agreement dated as of February 27, 2015, between Retrophin, Inc. and Ligand Pharmaceuticals Incorporated (incorporated by reference to Exhibit 10.5 to the Company's Quarterly Report on Form 10-Q, filed with the SEC on May 11, 2015).
10.14+	Asset Purchase Agreement dated January 10, 2015 by and between Retrophin, Inc. and Asklepios Pharmaceuticals, LLC (incorporated by reference to Exhibit 10.6 to the Company's Quarterly Report on Form 10-Q, filed with the SEC on May 11, 2015).

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10.15†	Employment Agreement, dated August 15, 2016, by and between Retrophin, Inc. and Neil McFarlane (incorporated by reference to Exhibit 10.1 to the Company's Quarterly Report on Form 10-Q, filed with the SEC on November 4, 2016).
10.16+	Amendment One to the Third Amendment to Trademark License and Supply Agreement, dated September 12, 2016, by and between the Company and Mission Pharmacal Company (incorporated by reference to Exhibit 10.2 to the Company's Quarterly Report on Form 10-Q, filed with the SEC on November 4, 2016).
10.17+	Amendment Two to the Third Amendment to Trademark License and Supply Agreement, dated November 3, 2017, by and between the Company and Mission Pharmacal Company.
10.18+	Third Amendment to Trademark License and Supply Agreement dated as of March 17, 2016, between Retrophin Inc. and Mission Pharmacal. (incorporated by reference to Exhibit 10.2 to the Company's Quarterly Report on Form 10-Q, filed with the SEC on May 5, 2016).
10.19	Fourth Amendment to Trademark License and Supply Agreement dated as of November 28, 2018, between Retrophin Inc. and Mission Pharmacal.
10.20+	Asset Purchase Agreement dated as of June 9, 2016 between Retrophin, Inc. and Asklepios Pharmaceuticals, LLC (incorporated by reference to Exhibit 10.1 to the Company's Quarterly Report on Form 10-Q, filed with the SEC on August 4, 2016).
10.21	Retrophin, Inc. 2015 Equity Incentive Plan, as amended (incorporated by reference to Exhibit 99.1 to the Company's current report on Form 8-K, filed with the SEC on May 18, 2017).
10.22†	Amendment to Employment Agreement, entered into as of April 11, 2017 and modifies the Employment Agreement dated March 2, 2015, by and between Retrophin, Inc. and Laura M. Clague (incorporated by reference to Exhibit 10.2 to the Company's Quarterly Report on Form 10-Q, filed with the SEC on May 5, 2017).
10.23†	Amendment to Employment Agreement, entered into as of April 11, 2017 and modifies the Employment Agreement dated August 15, 2016, by and between Retrophin, Inc. and Neil McFarlane (incorporated by reference to Exhibit 10.3 to the Company's Quarterly Report on Form 10-Q, filed with the SEC on May 5, 2017).
10.24	Retrophin, Inc. 2017 Employee Stock Purchase Plan (incorporated by reference to Exhibit 99.2 to the Company's current report on Form 8-K, filed with the SEC on May 18, 2017)
10.25	Form of Stock Option Grant Notice, Option Agreement and Notice of Exercise for Inducement Grant Outside of 2015 Equity Incentive Plan (incorporated by reference to Exhibit 99.3 to the Company's current report on Form S-8, filed with the SEC on June 8, 2017).
10.26	Form of Restricted Stock Unit Grant Notice and Restricted Stock Unit Award Agreement for Inducement Grant Outside of 2015 Equity Incentive Plan(incorporated by reference to Exhibit 99.4 to the Company's current report on Form S-8, filed with the SEC on June 8, 2017).
10.27	Retrophin, Inc. 2018 Equity Incentive Plan, Form of Stock Option Grant Notice, Option Agreement and Exercise Notice, Form of Restricted Stock Unit Award Agreement for use thereunder. (incorporated by reference to Exhibit 99.1 to the Company's Current Report on Form 8-K, filed with the SEC on May 10, 2018)
10.28	Form of Indemnity Agreement (incorporated by reference to Exhibit 10.1 to the Company's Quarterly Report on Form 10-Q, filed with the SEC on May 1, 2018)
10.29	Amendment No. 5 to Sublicense Agreement dated as of March 20, 2018, between the Company and Ligand Pharmaceuticals Incorporated (incorporated by reference to Exhibit 10.2 to the Company's Quarterly Report on Form 10-Q, filed with the SEC on May 1, 2018)
10.3	Employment Agreement, dated February 13, 2017, and Amendment to Employment Agreement, dated April 11, 2017, by and between the Company and William Rote (incorporated by reference to Exhibit 10.3 to the Company's Quarterly Report on Form 10-Q, filed with the SEC on May 1, 2018)
10.31	Employment Agreement, dated February 6, 2017, and Amendment to Employment Agreement, dated April 11, 2017, by and between the Company and Elizabeth E. Reed (incorporated by reference to Exhibit 10.4 to the Company's Quarterly Report on Form 10-Q, filed with the SEC on May 1, 2018)
10.32†	2019 Retrophin, Inc. Executive Officer Annual Bonus Plan.
10.33†	Retirement Agreement, dated January 4, 2019, by and between the Company and Stephan Aselage.
10.34†	Employment Agreement, dated January 4, 2019, by and between the Company and Eric M. Dube.
10.35†	Employment Agreement, dated July 26, 2018, by and between the Company and Noah Rosenberg, M.D.
21.1	List of subsidiaries of the Company.
23.1	Consent of BDO USA, LLP.
24.1	Power of Attorney (see signature page hereto).
31.1	Chief Executive Officer's Certification pursuant to Section 302 of the Sarbanes-Oxley Act of 2002.
31.2	Chief Financial Officer's Certification pursuant to Section 302 of the Sarbanes-Oxley Act of 2002.
32.1	Chief Executive Officer's Certification pursuant to Section 906 of Sarbanes Oxley Act of 2002.
32.2	Chief Financial Officer's Certification pursuant to Section 906 of Sarbanes Oxley Act of 2002.
101.INS	XBRL Instance Document.
101.SCH	XBRL Taxonomy Extension Schema Document.
101.CAL	XBRL Taxonomy Extension Calculation Linkbase Document.
101.DEF	XBRL Taxonomy Extension Definition Linkbase Document.
101.LAB	XBRL Taxonomy Extension Label Linkbase Document.
101.PRE	Taxonomy Extension Presentation Linkbase Document.

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- + We have received confidential treatment of certain portions of this agreement, which have been omitted and filed separately with the SEC pursuant to Rule 406 under the Securities Act of 1933, as amended, or Rule 24b-2 of the Securities Exchange Act of 1934, as amended.
- † Indicates management contract or compensatory plan.

ITEM 16. FORM 10-K SUMMARY

None.

SIGNATURES

Pursuant to the requirements of Section 13 or 15(d) 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.

Date: February 26, 2019

RETROPHIN, INC.

By: /s/ Eric M. Dube
Name: Eric M. Dube
Title: Chief Executive Officer

By: /s/ Laura Clague
Name: Laura Clague
Title: Chief Financial Officer

POWER OF ATTORNEY

Know all persons by these presents, that each person whose signature appears below constitutes and appoints Eric Dube and Laura Clague, and each of them, as his attorneys-in-fact and agents, each with power of substitution in any and all capacities, to sign any 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 Securities and Exchange Commission, hereby ratifying and confirming all that the attorney-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:

<u>Signature</u>	<u>Title</u>	<u>Date</u>
<u>/s/ Eric M. Dube</u> Eric M. Dube	Chief Executive Officer and Director (Principal Executive Officer)	February 26, 2019
<u>/s/ Laura Clague</u> Laura Clague	Chief Financial Officer (Principal Financial Officer and Principal Accounting Officer)	February 26, 2019
<u>/s/ Stephen Aselage</u> Stephen Aselage	Director	February 26, 2019
<u>/s/ Roy D. Baynes</u> Roy D. Baynes	Director	February 26, 2019
<u>/s/ Timothy Coughlin</u> Timothy Coughlin	Director	February 26, 2019
<u>/s/ John Kozarich</u> John Kozarich	Director	February 26, 2019
<u>/s/ Gary Lyons</u> Gary Lyons	Director	February 26, 2019
<u>/s/ Jeffrey A. Meckler</u> Jeffrey A. Meckler	Director	February 26, 2019
<u>/s/ John A. Orwin</u> John A. Orwin	Director	February 26, 2019
<u>/s/ Ron Squarer</u> Ron Squarer	Director	February 26, 2019

RETROPHIN, INC. AND SUBSIDIARIES

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Report of Independent Registered Public Accounting Firm

Shareholders and Board of Directors

Retrophin, Inc.

San Diego, California

Opinion on the Consolidated Financial Statements

We have audited the accompanying consolidated balance sheets of Retrophin, Inc (the "Company") and subsidiaries as of December 31, 2018 and 2017, the related consolidated statements of operations and comprehensive income (loss), stockholders' equity (deficit), and cash flows for each of the three years in the period ended December 31, 2018, and the related notes (collectively referred to as the "consolidated financial statements"). In our opinion, the consolidated financial statements present fairly, in all material respects, the financial position of the Company and subsidiaries at December 31, 2018 and 2017, and the results of their operations and their cash flows for each of the three years in the period ended December 31, 2018, in conformity with accounting principles generally accepted in the United States of America.

We also have 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, 2018, based on criteria established in *Internal Control - Integrated Framework (2013)* issued by the Committee of Sponsoring Organizations of the Treadway Commission ("COSO") and our report dated February 26, 2019 expressed an unqualified opinion thereon.

Change in Accounting Method Related to Derivative Financial Instruments, Warrants

As discussed in Note 2 to the consolidated financial statements, the Company has changed its method for accounting for certain financial instruments with down round features in 2018 due to the adoption of ASU 2017-11, Earnings Per Share (Topic 260); Distinguishing Liabilities from Equity (Topic 480); Derivatives and Hedging (Topic 815): (Part I) Accounting for Certain Financial Instruments with Down Round Features.

Basis for Opinion

These consolidated financial statements are the responsibility of the Company's management. Our responsibility is to express an opinion on the Company's consolidated financial statements based on our audits. We are a public accounting firm registered with the Public Company Accounting Oversight Board (United States) ("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 audits 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 consolidated financial statements are free of material misstatement, whether due to error or fraud.

Our audits included performing procedures to assess the risks of material misstatement of the consolidated 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 consolidated financial statements. Our audits also included evaluating the accounting principles used and significant estimates made by management, as well as evaluating the overall presentation of the consolidated financial statements. We believe that our audits provide a reasonable basis for our opinion.

/s/ BDO USA, LLP

We have served as the Company's auditor since 2014.

New York, New York

February 26, 2019

RETROPHIN, INC. AND SUBSIDIARIES
CONSOLIDATED BALANCE SHEETS
(In thousands, except share and per share amounts)

	December 31, 2018	December 31, 2017
Assets		
Current assets:		
Cash and cash equivalents	\$ 102,873	\$ 99,394
Marketable securities	368,668	201,236
Accounts receivable, net	14,490	13,872
Inventory, net	5,619	5,351
Prepaid expenses and other current assets	2,312	3,112
Prepaid taxes	1,716	2,842
Total current assets	495,678	325,807
Property and equipment, net	3,146	3,230
Other assets	7,709	5,556
Investment-equity	15,000	—
Intangible assets, net	186,691	184,817
Goodwill	936	936
Total assets	\$ 709,160	\$ 520,346
Liabilities and Stockholders' Equity		
Current liabilities:		
Accounts payable	\$ 6,954	\$ 18,938
Accrued expenses	49,695	36,018
Guaranteed minimum royalty, short term	2,100	2,000
Other current liabilities	4,065	3,902
Business combination-related contingent consideration	19,350	9,100
Convertible debt	22,457	—
Derivative financial instruments, warrants	—	15,710
Total current liabilities	104,621	85,668
Convertible debt	195,091	45,077
Other noncurrent liabilities	4,496	2,472
Guaranteed minimum royalty, long term	13,049	13,095
Business combination-related contingent consideration, less current portion	73,650	80,900
Total liabilities	390,907	227,212
Stockholders' Equity:		
Preferred stock \$0.0001 par value; 20,000,000 shares authorized; 0 issued and outstanding as of December 31, 2018 and 2017, respectively	—	—
Common stock \$0.0001 par value; 100,000,000 shares authorized; 41,389,524 and 39,373,745 issued and outstanding as of December 31, 2018 and 2017, respectively	4	4
Additional paid-in capital	589,795	471,800
Accumulated deficit	(270,017)	(177,655)
Accumulated other comprehensive loss	(1,529)	(1,015)
Total stockholders' equity	318,253	293,134
Total liabilities and stockholders' equity	\$ 709,160	\$ 520,346

The accompanying notes are an integral part of these consolidated financial statements.

RETROPHIN, INC. AND SUBSIDIARIES
CONSOLIDATED STATEMENTS OF OPERATIONS AND COMPREHENSIVE INCOME (LOSS)
(In thousands, except share and per share amounts)

	Years Ended December 31,		
	2018	2017	2016
Net product sales	\$ 164,246	\$ 154,937	\$ 133,591
Operating expenses:			
Cost of goods sold	5,527	3,605	4,554
Research and development	123,757	78,168	70,822
Selling, general and administrative	103,654	101,333	91,941
Change in fair value of contingent consideration	11,590	19,389	18,383
Restructuring	(242)	3,608	893
Legal fee settlement	—	2,625	5,212
Total operating expenses	<u>244,286</u>	<u>208,728</u>	<u>191,805</u>
Operating loss	(80,040)	(53,791)	(58,214)
Other Income (expense), net:			
Other income (expense), net	(474)	1,107	(264)
Interest income	5,499	3,234	3,975
Interest expense	(9,810)	(4,422)	(4,734)
Loss on extinguishment of debt	(17,042)	—	—
Change in fair value of derivative instruments	—	(4,491)	1,655
Total other income (expense), net	<u>(21,827)</u>	<u>(4,572)</u>	<u>632</u>
Loss before benefit (provision) for income taxes	(101,867)	(58,363)	(57,582)
Income tax benefit (provision)	(811)	(1,368)	9,679
Net loss	<u>\$ (102,678)</u>	<u>\$ (59,731)</u>	<u>\$ (47,903)</u>
Net loss per common share:			
Basic	<u>\$ (2.54)</u>	<u>\$ (1.54)</u>	<u>\$ (1.29)</u>
Diluted	<u>\$ (2.54)</u>	<u>\$ (1.54)</u>	<u>\$ (1.29)</u>
Weighted average common shares outstanding:			
Basic	<u>40,433,171</u>	<u>38,769,816</u>	<u>36,997,865</u>
Diluted	<u>40,433,171</u>	<u>38,769,816</u>	<u>38,288,012</u>
Comprehensive loss:			
Net loss	\$ (102,678)	\$ (59,731)	\$ (47,903)
Foreign currency translation gain (loss)	39	(339)	93
Unrealized gain (loss) on marketable securities	(553)	(186)	99
Comprehensive loss	<u>\$ (103,192)</u>	<u>\$ (60,256)</u>	<u>\$ (47,711)</u>

The accompanying notes are an integral part of these consolidated financial statements.

RETROPHIN, INC. AND SUBSIDIARIES
CONSOLIDATED STATEMENTS OF STOCKHOLDERS' EQUITY (DEFICIT)
(In thousands, except share amounts)

	Common Stock		Additional Paid in Capital	Accumulated Other Comprehensive Income (Loss)	Accumulated Deficit	Total Stockholders' Equity (Deficit)
	Shares	Amount				
BALANCE - DECEMBER 31, 2015	36,465,853	\$ 4	\$ 365,802	\$ (682)	\$ (65,153)	\$ 299,971
Share based compensation	—	—	29,102	—	—	29,102
Legal fee settlement-short swing profit recovery	—	—	2,025	—	—	2,025
Exercise of warrants and reclassification of derivative liability	898,633	—	20,720	—	—	20,720
Unrealized gain/(loss) on marketable securities	—	—	—	99	—	99
Foreign currency translation adjustments	—	—	3	93	—	96
Issuance of common shares under the equity incentive plan	542,183	—	4,016	—	—	4,016
Tax shortfall from stock option exercises	—	—	(359)	—	—	(359)
Net loss	—	—	—	—	(47,903)	(47,903)
BALANCE - DECEMBER 31, 2016	37,906,669	4	421,309	(490)	(113,056)	307,767
Adoption of ASU 2016-16 required de-recognition of intra-company deferred tax assets	—	—	—	—	(4,868)	(4,868)
Share based compensation	—	—	26,645	—	—	26,645
Exercise of warrants and reclassification of derivative liability	607,481	—	14,866	—	—	14,866
Unrealized gain/(loss) on marketable securities	—	—	—	(186)	—	(186)
Foreign currency translation adjustments	—	—	—	(339)	—	(339)
Issuance of common shares under the equity incentive plan and proceeds from exercise.	819,573	—	8,087	—	—	8,087
ESPP stock purchase and expense	40,022	—	893	—	—	893
Net loss	—	—	—	—	(59,731)	(59,731)
BALANCE - DECEMBER 31, 2017	39,373,745	4	471,800	(1,015)	(177,655)	\$ 293,134
Adoption of ASU 2017-11 - reclassification of derivative liability of warrants with down round provisions	—	—	5,394	—	10,316	15,710
Share based compensation	—	—	19,494	—	—	19,494
Exercise of warrants	1,036,054	—	5,305	—	—	5,305
Unrealized gain/(loss) on marketable securities	—	—	—	(553)	—	(553)
Foreign currency translation adjustments	—	—	—	39	—	39
Issuance of common shares under the equity incentive plan and proceeds from exercise.	892,713	—	10,588	—	—	10,588
ESPP stock purchase and expense	87,012	—	2,269	—	—	2,269
Convertible debt issue	—	—	74,945	—	—	74,945
Net loss	—	—	—	—	(102,678)	(102,678)
BALANCE - DECEMBER 31, 2018	41,389,524	\$ 4	\$ 589,795	\$ (1,529)	\$ (270,017)	\$ 318,253

The accompanying notes are an integral part of these consolidated financial statements

RETROPHIN, INC. AND SUBSIDIARIES
CONSOLIDATED STATEMENTS OF CASH FLOWS
(In thousands)

	For the year ended December 31,		
	2018	2017	2016
Cash Flows from Operating Activities:			
Net loss	\$ (102,678)	\$ (59,731)	\$ (47,903)
Adjustments to reconcile net loss to net cash provided by (used in) operating activities:			
Depreciation and amortization	18,668	17,804	16,135
Deferred income tax	—	(6,425)	(22,661)
Settlement expense	—	2,625	5,212
Loss on extinguishment of debt	17,042	—	—
Loss on allowance for inventory	2,457	609	262
Accretion on notes receivable	—	(651)	(1,927)
Accretion on contingent consideration	1,357	1,723	1,976
Amortization of debt discount and deferred financing costs	3,398	656	656
Amortization of premiums on investments	382	1,338	1,097
Share based compensation	19,774	26,874	29,102
Legal accrual reversal	—	—	(2,967)
Change in estimated fair value of contingent consideration	11,590	19,389	18,383
Payments from change in fair value of contingent consideration	(8,085)	(3,949)	(4,416)
Change in estimated fair value of liability classified warrants	—	4,491	(1,655)
Foreign currency transaction gain	464	(1,081)	—
Other operating activities	(396)	(83)	54
Changes in operating assets and liabilities, net of business acquisitions:			
Accounts receivable	152	4,945	(6,090)
Inventory	(2,773)	(1,706)	(568)
Prepaid expenses and other current assets	(1,358)	(2,702)	(2,447)
Prepaid income taxes	1,126	621	4,644
Accounts payable	(2,708)	2,060	(2,916)
Accrued expenses and other current liabilities	16,630	596	12,588
Net cash provided by (used in) operating activities	<u>(24,958)</u>	<u>7,403</u>	<u>(3,441)</u>
Cash Flows from Investing Activities:			
Purchase of fixed assets	(727)	(887)	(1,428)
Purchase of intangible assets	(18,974)	(13,122)	(10,496)
Investment - Equity	(15,000)	—	—
Proceeds from the sale/maturity of marketable securities	162,755	114,526	159,520
Purchase of marketable securities	(331,345)	(102,415)	(184,111)
Proceeds from the maturity of notes receivable	—	47,500	47,500
Cash paid upon acquisition, net of cash acquired	—	—	(615)
Net cash provided by (used in) investing activities	<u>(203,291)</u>	<u>45,602</u>	<u>10,370</u>
Cash Flows from Financing Activities:			
Payment of acquisition-related contingent consideration	(9,721)	(4,099)	(10,511)
Payment of other liability	(1,000)	(852)	(1,000)
Payment of guaranteed minimum royalty	(2,000)	(2,000)	(2,000)
Proceeds from exercise of warrants	5,305	3,645	6,005
Proceeds from exercise of stock options	10,588	8,087	4,016
Proceeds from issuance of 2025 convertible senior notes	276,000	—	—
Repurchase of 2019 convertible senior notes including premium	(40,203)	—	—
Payment of debt issuance and financing costs	(8,820)	—	—
Other financing activities	1,714	664	(359)
Net cash provided by (used in) financing activities	<u>231,863</u>	<u>5,445</u>	<u>(3,849)</u>
Effect of exchange rate changes on cash	(135)	(58)	117
Net increase in cash and cash equivalents	3,479	58,392	3,197
Cash and cash equivalents, beginning of year	99,394	41,002	37,805
Cash and cash equivalents, end of year	<u><u>\$ 102,873</u></u>	<u><u>\$ 99,394</u></u>	<u><u>\$ 41,002</u></u>

Supplemental Disclosure of Cash Flow Information:			
Cash paid for interest	\$ 1,880	\$ 2,070	\$ 2,070
Cash paid for income taxes	\$ 218	\$ 7,172	\$ 7,933
Non-cash Investing and financing activities:			
Short swing profit judgment offset with settlement expense accrual	\$ —	\$ —	\$ 2,025
Reclassification of derivative liability to equity due to exercise of warrants	\$ —	\$ 11,221	\$ 14,715

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Accrued royalty in excess of minimum payable to the sellers of Thiola	\$ 14,572	\$ 13,247	\$ 11,206
Term extension of current Thiola agreement	\$ —	\$ 5,885	\$ —
Present value of contingent consideration payable upon acquisition related to L-UDCA	\$ —	\$ —	\$ 25,000
Adoption of ASU 2017-11 - reclassification of derivative liability of warrants with down round provisions	\$ 15,710	\$ —	\$ —

The accompanying notes are an integral part of these consolidated financial statements.

RETROPHIN, INC. AND SUBSIDIARIES

NOTES TO CONSOLIDATED FINANCIAL STATEMENTS

NOTE 1. DESCRIPTION OF BUSINESS

Organization and Description of Business

Retrophin, Inc. ("we", "our", "us", "Retrophin" and the "Company") refers to Retrophin, Inc., a Delaware corporation, as well as our direct and indirect subsidiaries. Retrophin is a fully integrated biopharmaceutical company headquartered in San Diego, California focused on identifying, developing and delivering life-changing therapies to people with rare diseases. We regularly evaluate and, where appropriate, act on opportunities to expand our product pipeline through licenses and acquisitions of products in areas that will serve patients with serious or rare diseases and that we believe offer attractive growth characteristics.

The Company is developing the following pipeline products:

The Company is developing fosmetpantotenate (RE-024), a novel small molecule, as a potential treatment for PKAN. PKAN is a genetic neurodegenerative disorder that is typically diagnosed in the first decade of life.

Sparsentan, also known as RE-021, is an investigational product candidate with a dual mechanism of action, a potent angiotensin receptor blocker ("ARB") and selective endothelin receptor antagonist ("ERA"), with in vitro selectivity toward endothelin receptor type A. Sparsentan is currently being evaluated in two pivotal Phase 3 clinical studies in the following indications:

- **Focal segmental glomerulosclerosis ("FSGS")** is a rare kidney disease characterized by proteinuria where the glomeruli become progressively scarred. FSGS is a leading cause of end-stage renal disease.
- **Immunoglobulin A nephropathy ("IgAN")** is an immune-complex-mediated glomerulonephritis characterized by hematuria, proteinuria, and variable rates of progressive renal failure. IgAN is the most common primary glomerular disease.

The Company is a party to a joint development agreement with Censa Pharmaceuticals Inc., a privately held biotechnology company focused on developing therapies for orphan metabolic diseases, to evaluate sepiapterin ("CNSA-001") for the treatment of phenylketonuria (PKU).

In September 2017, the Company entered a three-way Cooperative Research and Development Agreement ("CRADA") with the National Institutes of Health's National Center for Advancing Translational Sciences (NCATS) and patient advocacy foundation NGLY1.org to collaborate on research efforts aimed at the identification of potential small molecule therapeutics for NGLY1 deficiency.

Liquid ursodeoxycholic acid ("L-UDCA") is a liquid formulation of ursodeoxycholic acid being developed for the treatment of a rare liver disease called primary biliary cholangitis ("PBC"). The Company obtained the rights to L-UDCA in 2016 with the intention of making L-UDCA commercially available to the subset of PBC patients who have difficulty swallowing.

The Company sells the following three products:

- **Chenodal (chenodiol tablets)** is approved in the United States for the treatment of patients suffering from gallstones in whom surgery poses an unacceptable health risk due to disease or advanced age. Chenodal has been the standard of care for cerebrotendinous xanthomatosis ("CTX") patients for more than three decades and the Company is currently pursuing adding this indication to the label.
- **Cholbam (cholic acid capsules)** is approved in the United States for the treatment of bile acid synthesis disorders due to single enzyme defects and is further indicated for adjunctive treatment of patients with peroxisomal disorders.
- **Thiola (tiopronin tablets)** is approved in the United States for the prevention of cystine (kidney) stone formation in patients with severe homozygous cystinuria.

NOTE 2. SUMMARY OF SIGNIFICANT ACCOUNTING POLICIES

A summary of the significant accounting policies applied in the preparation of the accompanying consolidated financial statements follows:

Principles of Consolidation

The consolidated financial statements represent the consolidation of the accounts of the Company and its subsidiaries in conformity with accounting principles generally accepted in the United States ("U.S. GAAP"). All intercompany accounts and transactions have been eliminated in consolidation.

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Use of Estimates

In preparing financial statements in conformity with U.S. GAAP, management is required to make estimates and assumptions that affect the reported amounts of assets and liabilities and disclosure of contingent assets and liabilities at the date of the financial statements, as well as the reported amounts of expenses during the reporting period. Due to inherent uncertainty involved in making estimates, actual results reported in future periods may be affected by changes in these estimates. On an ongoing basis, the Company evaluates its estimates and assumptions. These estimates and assumptions include revenue recognition, valuing equity securities in share-based payments, estimating expenses of contracted research organizations, estimating fair value of equity instruments recorded as derivative liabilities, estimating the fair value of net assets acquired in business combinations, estimating the useful lives of depreciable and amortizable assets, goodwill impairment, estimating the fair value of contingent consideration, estimating of valuation allowances and uncertain tax positions, and estimates associated with the assessment of impairment for long lived assets.

Revenue Recognition

Product sales for the years ended December 31, 2018, 2017 and 2016 consisted of sales of Chenodal, Cholbam and Thiola. Effective January 1, 2018, the Company adopted Accounting Standards Codification ("ASC"), Topic 606, Revenue from Contracts with Customers, using the full retrospective transition method. This standard applies to all contracts with customers, except for contracts that are within the scope of other standards, such as leases, insurance, collaboration arrangements and financial instruments. Under Topic 606, an entity recognizes revenue when its customer obtains control of promised goods or services, in an amount that reflects the consideration which the entity expects to receive in exchange for those goods or services. To determine revenue recognition for arrangements that an entity determines are within the scope of Topic 606, the entity performs the following five steps: (i) identify the contract(s) with a customer; (ii) identify the performance obligations in the contract; (iii) determine the transaction price; (iv) allocate the transaction price to the performance obligations in the contract; and (v) recognize revenue when (or as) the entity satisfies a performance obligation. The Company only applies the five-step model to contracts when it is probable that the entity will collect substantially all the consideration it is entitled to in exchange for the goods or services it transfers to the customer.

Revenue from product sales is recorded at delivery, net of applicable provisions for rebates under government (including medicaid) programs, commercial rebates, prompt pay discounts, and other sales-related deductions. We review our estimates of rebates and other applicable provisions each period and record any necessary adjustments in the current period.

See Note 3 for further discussion.

Research and Development Costs

Research and development includes expenses related to sparsentan, fosmetpantotenate and our other pipeline programs. We expense all research and development costs as they are incurred. Our research and development costs are comprised of salaries and bonuses, benefits, non-cash share-based compensation, license fees, milestones under license agreements, costs paid to third-party contractors to perform research, conduct clinical trials, and develop drug materials and delivery devices, and associated overhead expenses and facilities costs. We charge direct internal and external program costs to the respective development programs. We also incur indirect costs that are not allocated to specific programs because such costs benefit multiple development programs and allow us to increase our pharmaceutical development capabilities. These consist of internal shared resources related to the development and maintenance of systems and processes applicable to all of our programs.

Clinical Trial Expenses

We record expenses in connection with our clinical trials under contracts with contract research organizations (CROs) that support conducting and managing clinical trials. The financial terms and activities of these agreements vary from contract to contract and may result in uneven expense levels. Generally, these agreements set forth activities that drive the recording of expenses such as start-up and initiation activities, enrollment and treatment of patients, or the completion of other clinical trial activities.

Expenses related to clinical trials are accrued based on our estimates and/or representations from service providers regarding work performed, including actual level of patient enrollment, completion of patient studies and progress of the clinical trials. Other incidental costs related to patient enrollment or treatment are accrued when reasonably certain. If the amounts we are obligated to pay under our clinical trial agreements are modified (for instance, as a result of changes in the clinical trial protocol or scope of work to be performed), we adjust our accruals accordingly on a prospective basis. Revisions to our contractual payment obligations are charged to expense in the period in which the facts that give rise to the revision become reasonably certain.

We currently have three Phase 3 clinical trials in process that are in varying stages of activity, with ongoing non-clinical support trials. As such, clinical trial expenses will vary depending on the all the factors set forth above and may fluctuate significantly from quarter to quarter.

Employee Stock-Based Compensation

The Company recognizes all employee share-based compensation as a cost in the financial statements. Equity-classified awards principally related to stock options, restricted stock units ("RSUs") and performance stock units ("PSUs"), are measured at the grant date fair value of the award. The Company determines grant date fair value of stock option awards using the Black-Scholes option-pricing model. The fair value of RSUs and PSUs are determined using the closing price of the Company's common stock on the grant date. For service based vesting grants, expense is recognized over the requisite service period based on the number of options or shares expected to ultimately vest. For PSUs, expense is recognized over the implicit service period, assuming vesting is probable. No expense is recognized for PSUs if it is not probable the vesting criteria will be satisfied. Forfeitures are accounted for as they occur.

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	Initial Vesting Term
Stock Options	3 to 4 years
Restricted Stock Units	2 to 4 years

Earnings (Loss) Per Share

We calculate our basic earnings per share by dividing net income by the weighted average number of shares outstanding during the period. The diluted earnings per share computation includes the effect, if any, of shares that would be issuable upon the exercise of outstanding stock options, derivative liability, convertible debt and RSUs, reduced by the number of shares which are assumed to be purchased by the Company from the resulting proceeds at the average market price during the year, when such amounts are dilutive to the earnings per share calculation.

Cash and Cash Equivalents

We consider all highly liquid marketable securities with an original maturity of three months or less to be cash equivalents. Due to the short-term maturity of such investments, the carrying amounts are a reasonable estimate of fair value.

Marketable Securities

The Company accounts for marketable securities held as "available-for-sale" in accordance with ASC 320, "Investments Debt and Equity Securities" ("ASC 320"). The Company classifies these investments as current assets and carries them at fair value. Unrealized gains and losses are recorded as a separate component of stockholders' equity as accumulated other comprehensive loss. Realized gains or losses on marketable security transactions are reported in the Consolidated Statements of Operations and Comprehensive Income (Loss). Marketable securities are maintained at one financial institution and are governed by the Company's investment policy as approved by the Company's Board of Directors.

Trade and Notes Receivable

Trade Receivables, Net

Trade accounts receivable are recorded net of allowances for prompt payment and doubtful accounts. Estimates for allowances for doubtful accounts are determined based on existing contractual obligations, historical payment patterns and individual customer circumstances. The allowance for doubtful accounts was zero and \$0.2 million at December 31, 2018 and 2017, respectively. For the years ended December 31, 2018, 2017 and 2016, bad debt expense recorded in the Statement of Operations and Comprehensive Income (Loss) was approximately zero, \$0.2 million and \$0.2 million, respectively.

Notes Receivable

Notes receivable arose from the sale of a pediatric priority review voucher (the "PRV"). On July 2, 2015, the Company sold and transferred the PRV to Sanofi for \$245.0 million. \$150.0 million was received upon closing, and \$47.5 million was due on each of the first and second anniversaries of the closing. In accordance with U.S. GAAP, the Company recorded the future short term and long term notes receivable at their present value of \$46.2 million and \$44.9 million, respectively, at the date of the sale using a discount rate of 2.8%. The accretion on the notes receivables totaled \$0.7 million and \$1.9 million for 2017 and 2016, respectively, and is recorded in interest expense, net, in the Consolidated Statements of Operations and Comprehensive Income (Loss). The first and second annual payments were received on July 1, 2016 and June 30, 2017 in accordance with the terms of the sale agreement. As of December 31, 2018, there are no outstanding notes receivable.

Inventory and Related Reserves

Inventory, which is recorded at the lower of cost or net realizable value, includes materials, labor, and other direct and indirect costs and are valued using the first-in, first-out method. The Company periodically analyzes its inventory levels to identify inventory that may expire prior to expected sale or has a cost basis in excess of its estimated realizable value, and writes down such inventory as appropriate. In addition, the Company's products are subject to strict quality control and monitoring which the Company's manufacturers perform throughout their manufacturing process. The Company does not directly manufacture any product. The Company has single suppliers for products Chenodal and Thiola, and prospectively arranges for manufacture from contract service providers for its product Cholbam. The inventory reserve was \$1.8 million and \$0.7 million at December 31, 2018 and 2017, respectively.

Inventory, net of reserve, consisted of the following at December 31, 2018 and 2017 (*in thousands*):

	December 31, 2018	December 31, 2017
Raw material	\$ 4,689	\$ 3,435
Finished goods	930	1,916
Total inventory	\$ 5,619	\$ 5,351

Segment Information

The Company currently operates in one business segment focused on the development and commercialization of innovative therapies for people with serious and life threatening rare diseases and medical conditions. The Company is not organized by market and is managed and operated as one business. A single management team reports to the chief operating decision maker who comprehensively manages the entire business. The Company does not operate any separate lines of business or separate business entities with respect to its products. Accordingly, the Company does

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not accumulate discrete financial information with respect to separate products, other than revenues, and does not have separately reportable segments.

Property and Equipment, net

Property and equipment are stated at cost, net of accumulated depreciation. Depreciation is computed using the straight-line method over the related estimated useful lives as presented in the table below. Significant additions and improvements are capitalized, while repairs and maintenance are charged to expense as incurred. Property and equipment purchased for specific research and development projects with no alternative use is expensed as incurred.

The major classifications of property and equipment, including their respective expected useful lives, consists of the following:

Computers and equipment	3 years
Furniture and fixtures	7 years
Leasehold improvements	Shorter of length of lease or life of the asset

Intangible Assets, Net

Our intangible assets consist of licenses, purchased technology and acquired in-process research and development (IPR&D). Intangible assets with definite lives are amortized on a straight-line basis over their estimated useful lives and are reviewed periodically for impairment.

Intangible assets related to IPR&D projects are considered to be indefinite-lived until the completion or abandonment of the associated research and development efforts. During the period the assets are considered indefinite-lived, they will not be amortized but will be tested for impairment. If and when development is complete, which generally occurs when regulatory approval to market a product is obtained, the associated assets are deemed finite-lived and are amortized over a period that best reflects the economic benefits provided by these assets.

Goodwill

Goodwill represents the excess of purchase price over fair value of net assets acquired in a business combination and is not amortized. Goodwill is subject to impairment testing at least annually or when a triggering event occurs that could indicate a potential impairment. The Company has one segment and one reporting unit and as such reviews goodwill for impairment at the consolidated level.

For the years ended December 31, 2018, 2017 and 2016 there were no impairments to goodwill.

Impairment of Long-Lived Assets

Our long-lived assets are primarily comprised of intangible assets and property and equipment. We evaluate our finite-lived intangible assets, other than goodwill and property and equipment, for impairment whenever events or changes in circumstances indicate the carrying value of an asset or group of assets may not be recoverable. If these circumstances exist, recoverability of assets to be held and used is measured by a comparison of the carrying amount of an asset group to future undiscounted net cash flows expected to be generated by the use and eventual disposition of the asset group. If such assets are considered to be impaired, the impairment to be recognized is measured by the amount by which the carrying amount of the assets exceeds the fair value of the assets.

In addition, indefinite-lived intangible assets, comprised of IPR&D, are reviewed for impairment annually and whenever events or changes in circumstances indicate that it is more likely than not that the asset is impaired by comparing the fair value to the carrying value of the asset. To determine the fair value of the asset, the Company used the multi-period excess earnings method of the income approach. The more significant assumptions inherent in the application of this method include: the amount and timing of projected future cash flows (including revenue, cost of sales, research and development costs, and sales and marketing expenses), and the discount rate selected to measure the risks inherent in the future cash flows.

There were no impairments related to intangible assets in the years ended December 31, 2018, 2017 and 2016.

Contingent Consideration

We record contingent consideration resulting from a business combination at its fair value on the acquisition date. On a quarterly basis, we revalue these obligations and record increases or decreases from their fair value as an adjustment to the consolidated statement of operations. Changes to contingent consideration obligations can result from changes to discount rates, accretion of the liability due to the passage of time, changes in revenue forecasts and changes in our estimates of the likelihood or timing of achieving commercial milestones.

Income Taxes

The Company follows ASC 740, Income Taxes, which requires recognition of deferred tax assets and liabilities for the expected future tax consequences of events that have been included in the financial statements or tax returns. Under this method, deferred tax assets and liabilities are based on the differences between the financial statement and tax basis of assets and liabilities using enacted tax rates in effect for the year in which the differences are expected to reverse. Deferred tax assets are reduced by a valuation allowance to the extent management concludes it is more likely than not that the asset will not be realized.

The standard addresses the determination of whether tax benefits claimed or expected to be claimed on a tax return should be recorded in the financial statements. Under ASC 740, the Company may recognize the tax benefit from an uncertain tax position only if it is more likely than not that the tax position will be sustained on examination by the tax authorities, based on the technical merits of the position. The tax benefits recognized in

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the financial statements from such a position should be measured based on the largest benefit that has a greater than fifty percent likelihood of being realized upon ultimate settlement. ASC 740 also provides guidance on de-recognition, classification, interest and penalties on income taxes, accounting in interim periods and requires increased disclosures. The Company's policy is to record estimated interest and penalty related to the underpayment of income taxes or unrecognized tax benefits as a component of its income tax provision.

Reclassifications

Certain reclassifications have been made to the prior year financial statements in order to conform to the current year's presentation.

Patents

The Company expenses external costs, such as filing fees and associated attorney fees, incurred to obtain issued patents and patent applications pending. The Company also expenses costs associated with maintaining and defending patents subsequent to their issuance in the period incurred.

Derivative Financial Instruments, Warrants

The Company does not use derivative instruments to hedge exposures to cash flow, market or foreign currency risks. However, since 2013, the Company has issued five tranches of common stock purchase warrants to secure financing, remediate covenant violations and provide consideration for amendments with respect to a credit facility extinguished in 2015.

Historically, the Company accounted for these instruments, which did not have fixed settlement provisions, as derivative instruments in accordance with FASB ASC 815-40, *Derivative and Hedging - Contracts in Entity's Own Equity*. This was due to an anti-dilution provision for the warrants that provided for a reduction to the exercise price if the Company issued equity or equity linked instruments in the future at an effective price per share less than the exercise price then in effect for the warrant ("down round provision"). As such, the warrants were re-measured at each balance sheet date based on estimated fair value. Changes in estimated fair value were recorded as non-cash adjustments within other income (expenses), net, in the Company's Consolidated Statements of Operations and Comprehensive Income (Loss).

As of January 1, 2018, the Company early adopted ASU 2017-11, which revised the guidance for instruments with down round provisions. As such the Company treats outstanding warrants as free-standing equity linked instruments that will be recorded to equity in the Consolidated Balance Sheet.

The fair value of the derivative liability balance as of December 31, 2017 of \$15.7 million was reclassified by means of a cumulative-effect adjustment to equity as of January 1, 2018.

Recently Adopted Accounting Pronouncements

In May 2014, the FASB issued ASU No. 2014-09, Revenue from Contracts with Customers. Under the new standard, revenue is recognized at the time a good or service is transferred to a customer for the amount of consideration for which the entity expects to be entitled for that specific good or service. Entities may use a full retrospective approach or report the cumulative effect as of the date of adoption. The Company adopted the new standard on January 1, 2018 using the full retrospective approach and there was no impact on timing or recognition of revenue. See Note 3 for further discussion.

In October 2016, the FASB issued ASU No. 2016-16, Income Taxes (Topic 740): Intra-Entity Transfers of Assets Other Than Inventory. The new guidance changes the accounting for income tax effects of intra-entity transfers of assets other than inventory. Under the new guidance, the selling (transferring) entity is required to recognize a current tax expense or benefit upon transfer of the asset. Similarly, the purchasing (receiving) entity is required to recognize a deferred tax asset or deferred tax liability, as well as the related deferred tax benefit or expense, upon receipt of the asset. As of January 1, 2017, the Company reversed the balance of \$4.9 million in its prepaid tax asset account as a charge to retained earnings.

In July 2017, the FASB issued ASU No. 2017-11, Earnings Per Share (Topic 260); Distinguishing Liabilities from Equity (Topic 480); Derivatives and Hedging (Topic 815): (Part I) Accounting for Certain Financial Instruments with Down Round Features. These amendments simplify the accounting for certain financial instruments with down round features. The amendments require companies to disregard the down round feature when assessing whether the instrument is indexed to its own stock, for purposes of determining liability or equity classification. See Note 15 for further discussion.

In January 2017, the FASB issued ASU 2017-01, Business Combinations (Topic 805): Clarifying the Definition of a Business. The new guidance dictates that, when substantially all of the fair value of the gross assets acquired (or disposed of) is concentrated in a single identifiable asset or a group of similar identifiable assets, it should be treated as an acquisition or disposal of an asset. The guidance was adopted as of January 1, 2018.

Recently Issued Accounting Pronouncements

From time to time, new accounting pronouncements are issued by the FASB or other standard setting bodies. Unless otherwise discussed, the Company believes that the impact of recently issued standards that are not yet effective will not have a material impact on its consolidated financial position or results of operations upon adoption.

In February 2016, the FASB issued ASU No. 2016-02, Leases. The new standard establishes a right-of-use ("ROU") model that requires a lessee to record a ROU asset and a lease liability on the balance sheet for all leases with terms longer than 12 months. Leases will be classified as either finance or operating, with classification affecting the pattern of expense recognition in the income statement. Subsequently, the FASB issued ASU

No. 2018-10, Codification Improvements to Topic 842, ASU No. 2018-11, Targeted Improvements, and ASU No. 2018-20, Narrow-Scope Improvements for Lessors, to clarify and amend the guidance in ASU No. 2016-02. The ASUs are effective for interim and annual periods beginning after December 15, 2018, with early adoption permitted. We will adopt the ASUs on January 1, 2019 on a modified retrospective basis through a cumulative adjustment to our beginning accumulated deficit balance. Prior comparative periods will not be restated under this method, and we will adopt all available practical expedients, as applicable. The Company has finished its search for leases and reviewed the related contacts and determined its impact to the consolidated balance sheet is less than 5% of total assets and liabilities as of January 1, 2019. No material cumulative-effect adjustment to equity is expected.

In June 2016, the FASB issued ASU No. 2016-13, Financial Instruments - Credit Losses (Topic 326): Measurement of Credit Losses on Financial Instruments. Topic 326 amends guidance on reporting credit losses for assets held at amortized cost basis and available for sale debt securities. For assets held at amortized cost basis, Topic 326 eliminates the probable initial recognition threshold in current GAAP and, instead, requires an entity to reflect its current estimate of all expected credit losses. The allowance for credit losses is a valuation account that is deducted from the amortized cost basis of the financial assets to present the net amount expected to be collected. For available for sale debt securities, credit losses should be measured in a manner similar to current GAAP, however Topic 326 will require that credit losses be presented as an allowance rather than as a write-down. This ASU update affects entities holding financial assets and net investment in leases that are not accounted for at fair value through net income. The amendments affect loans, debt securities, trade receivables, net investments in leases, off balance sheet credit exposures, reinsurance receivables, and any other financial assets not excluded from the scope that have the contractual right to receive cash. This update is effective for fiscal years beginning after December 15, 2019, including interim periods within those fiscal years. The Company is currently evaluating whether the adoption of the new standard will have a material effect on its consolidated financial statements and related disclosures.

NOTE 3. REVENUE RECOGNITION

Product Revenue, Net

Product sales for the years ended December 31, 2018, 2017 and 2016 consisted of sales of Chenodal, Cholbam and Thiola. Effective January 1, 2018, the Company adopted Accounting Standards Codification ("ASC"), Topic 606, Revenue from Contracts with Customers, using the full retrospective transition method. This standard applies to all contracts with customers, except for contracts that are within the scope of other standards, such as leases, insurance, collaboration arrangements and financial instruments. Under Topic 606, an entity recognizes revenue when its customer obtains control of promised goods or services, in an amount that reflects the consideration which the entity expects to receive in exchange for those goods or services. To determine revenue recognition for arrangements that an entity determines are within the scope of Topic 606, the entity performs the following five steps: (i) identify the contract(s) with a customer; (ii) identify the performance obligations in the contract; (iii) determine the transaction price; (iv) allocate the transaction price to the performance obligations in the contract; and (v) recognize revenue when (or as) the entity satisfies a performance obligation. The Company only applies the five-step model to contracts when it is probable that the entity will collect substantially all the consideration it is entitled to in exchange for the goods or services it transfers to the customer.

The Company sells Chenodal and Cholbam (Kolbam), which are aggregated as bile acid products, and Thiola through direct-to-patient distributors. The Company sells its products worldwide, with more than 95% of the revenue generated in North America.

Revenues from product sales are recognized when the customer obtains control of the Company's product, which occurs upon delivery to the customer. The Company receives payments from its product sales based on terms that generally are within 60 days of delivery of product to the patient.

Deductions from Revenue

Revenues from product sales are recorded at the net sales price, which includes provisions resulting from discounts, rebates and co-pay assistance that is offered to its customers, health care providers, payors and other indirect customers relating to the Company's sales of its products. These provisions are based on the amounts earned or to be claimed on the related sales and are classified as a reduction of accounts receivable (if the amount is payable to the customer) or as a current liability (if the amount is payable to a party other than a customer). Where appropriate, these reserves take into consideration the Company's historical experience, current contractual and statutory requirements and specific known market events and trends. Overall, these reserves reflect the Company's best estimates of the amount of consideration to which it is entitled based on the terms of the contract. If actual results in the future vary from the Company's provisions, the Company will adjust the provision, which would affect net product revenue and earnings in the period such variances become known. Our historical experience is that such adjustments have been immaterial.

Government Rebates: We calculate the rebates that we will be obligated to provide to government programs and deduct these estimated amounts from our gross product sales at the time the revenues are recognized. Allowances for government rebates and discounts are established based on actual payer information, which is reasonably estimated at the time of delivery, and the government-mandated discounts applicable to government-funded programs. Rebate discounts are included in other current liabilities in the accompanying consolidated balance sheets.

Commercial Rebates: We calculate the rebates that we incur due to contracts with certain commercial payors and deduct these amounts from our gross product sales at the time the revenues are recognized. Allowances for commercial rebates are established based on actual payer information, which is reasonably estimated at the time of delivery. Rebate discounts are included in other current liabilities in the accompanying consolidated balance sheets.

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Prompt Pay Discounts: We offer discounts to certain customers for prompt payments. We accrue for the calculated prompt pay discount based on the gross amount of each invoice for those customers at the time of sale.

Product Returns: Consistent with industry practice, we offer our customers a limited right to return product purchased directly from the Company, which is principally based upon the product's expiration date. Generally, shipments are only made upon a patient prescription thus returns are minimal.

Co-pay Assistance: We offer a co-pay assistance program, which is intended to provide financial assistance to qualified commercially insured patients with prescription drug co-payments required by payors. The calculation of the accrual for co-pay assistance is based on an identification of claims and the cost per claim associated with product that has been recognized as revenue.

The following table summarizes net product revenues for the twelve months ended December 31, 2018, 2017 and 2016 (*in thousands*):

	Twelve Months Ended December 31,		
	2018	2017	2016
Thiola	89,176	82,311	71,199
Bile acid products	75,070	72,626	62,392
Total net product revenue	164,246	154,937	133,591

NOTE 4. FUTURE ACQUISITION RIGHT AND JOINT DEVELOPMENT AGREEMENT

Censa Pharmaceuticals Inc.

In December 2017, the Company entered into a Future Acquisition Right and Joint Development Agreement (the "Option Agreement") with Censa, which became effective in January 2018. The Company has agreed to fund certain development activities of Censa's CNSA-001 program, in an aggregate amount expected to be approximately \$17 million through proof of concept, and has the right, but not the obligation, to acquire Censa (the "Option") on the terms and subject to the conditions set forth in a separate Agreement and Plan of Merger. In exchange for the Option, the Company paid \$10 million, and an additional \$5 million upon Censa's completion of a specified development milestone set forth in the Option Agreement.

If the Company exercises the Option, the Company will acquire Censa for \$65 million in upfront consideration, subject to certain adjustments, paid as a combination of 20% in cash and 80% in shares of the Company's common stock, valued at a fixed price of \$21.40 per share; provided, however, that Censa may elect on behalf of its equity holders to receive the upfront consideration in 100% cash if the average price per share of the Company's common stock for the ten trading days ending on the date the Company provides a notice of interest to exercise the Option is less than \$19.26. In addition, if the Company exercises the Option and acquires Censa, the Company would be required to make further cash payments to Censa's equity holders of up to an aggregate of \$25 million if the CNSA-001 program achieves specified development and commercial milestones.

The Company determined that Censa is a variable interest entity ("VIE") and concluded that the Company is not the primary beneficiary of the VIE. As such, the Company did not consolidate Censa's results into its consolidated financial statements. The Company will continue to monitor facts and circumstances for changes that could potentially result in the Company becoming the primary beneficiary.

Through December 31, 2018, the Company has paid Censa \$10.0 million as an upfront payment, \$16.8 million in development funding, and \$5.0 million related to a development milestone. The Company capitalized the upfront and milestone payments and expensed the development funding paid to research and development expense in the Consolidated Statement of Operations and Comprehensive Income (Loss). The Company is treating the upfront payment and milestone payment, both of which are compensation for the Option, as a cost-method investment with a total carrying value of \$15.0 million as of December 31, 2018.

NOTE 5. BUSINESS COMBINATION AND ASSET TRANSACTIONS

Amendment to Trademark License and Supply Agreement

In November 2017, the Company amended their agreement with the manufacturer of Thiola to extend the term of the current exclusive U.S. and Canada licensing agreement by an additional five years, to 2029. The royalty rate and guaranteed minimum payment were also extended through the new agreement term. Upon execution of the amendment, the Company capitalized an additional \$5.9 million in intangible assets and recorded a guaranteed minimum liability for the same amount.

In November 2018, the license agreement was amended to extend the territorial rights beyond the United States and Canada. As consideration for the expanded territory the Company paid an up-front fee of \$0.3 million and will pay guaranteed minimum royalties equaling the greater of \$0.1 million or 20% of our Thiola net sales generated from outside of the United States during each calendar year. Upon execution of the amendment, the Company capitalized an additional \$1.0 million in intangible assets and recorded a guaranteed minimum liability of \$0.7 million related to this amendment.

Acquisition of Liquid Ursodeoxycholic Acid (L-UDCA)

On June 20, 2016, the Company signed a definitive agreement to purchase the rights, titles, licenses and ownership of L-UDCA from Askleion Pharmaceuticals, LLC ("Askleion").

The acquisition was accounted for under the acquisition method of accounting in accordance with Accounting Standard Codification ("ASC") 805. The fair value of assets acquired and liabilities assumed was based upon an independent third-party valuation and the Company's estimates. Critical estimates in valuing certain intangible assets include but are not limited to future expected cash flows from acquired product rights for L-UDCA, licenses, trade names and developed technologies, present value and discount rates. Management's estimates of fair value are based upon assumptions believed to be reasonable, but which are inherently uncertain and unpredictable and, as a result, actual results may differ from estimates.

The purchase included \$25.5 million for an intangible asset with a definite life related to product rights in the U.S. The useful life related to the acquired product rights is expected to be approximately 17 years once the NDA is approved by the FDA. Until approval, the asset is considered IPR&D with an indefinite life and is not amortized.

The contingent consideration of \$25.0 million (present value) recorded during the period ended June 30, 2016, is related to an agreement to pay an additional cash amount in the form of milestones and sales royalties through 2035. The accrued contingent consideration was recorded as a liability at acquisition-date fair value using the income approach with an assumed discount rate of 12.0% over the applicable term. The undiscounted amount the Company could pay as contingent consideration under the agreement is up to \$70.3 million.

The purchase price allocation of \$25.5 million as of the acquisition completion date of June 16, 2016 was as follows (*in thousands*):

Cash paid upon consummation	\$	500
Present value of contingent consideration		25,000
Total purchase price	\$	25,500
Fair Value of Assets Acquired and Liabilities Assumed		
Acquired product rights: L-UDCA (intangible asset)	\$	25,500
Total purchase price	\$	25,500

Unaudited pro forma information for the transaction is not presented, because the effects of such transaction are considered immaterial to the Company.

Divestiture of Assets:

Sale of Assets to Sanofi

The FDA granted Askleion a Pediatric PRV, awarded to encourage development of new drugs and biologics for the prevention and treatment of rare pediatric diseases. A Pediatric PRV is transferable and provides the bearer with FDA priority review classification for a new drug application. The Pediatric PRV was transferred to the Company under the terms of the asset purchase agreement between the Company and Askleion dated January 12, 2015, pursuant to which the Company acquired Cholbam.

On July 2, 2015, the Company sold and transferred the Pediatric PRV to Sanofi for \$245.0 million. \$150.0 million was received upon closing, and \$47.5 million was due on each of the first and second anniversaries of the closing. In accordance with U.S. GAAP, the Company recorded the future short term and long term notes receivable at their present value of \$46.2 million and \$44.9 million, respectively, at the date of the sale using a discount rate of 2.8%. The gain from the sale of the asset was approximately \$140.0 million, net of \$4.9 million in fees contractually due as part of the Cholbam acquisition. The first and second annual payments were received on July 1, 2016 and June 30, 2017 in accordance with the terms of the sale agreement.

NOTE 6. MARKETABLE SECURITIES

The Company's marketable securities as of December 31, 2018 and 2017 were comprised of available-for-sale marketable securities which are carried at fair value, with the unrealized gains and losses reported in accumulated other comprehensive income (loss). The amortized cost of debt securities in this category are adjusted for amortization of premiums and accretion of discounts to maturity. Such amortization and accretion is included in interest income (loss). Realized gains and losses and declines in value judged to be other-than-temporary, if any, on available-for-sale securities are included in other income or expense. The cost of securities sold is based on the specific identification method. Interest and dividends on securities classified as available-for-sale are included in interest income. All available-for-sale securities are classified as current assets, even if the maturity when acquired by the Company is greater than one year due to the ability to liquidate within the next 12 months.

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Marketable securities consist of the following (*in thousands*):

	As of December 31,	
	2018	2017
Marketable Securities:		
Commercial paper	59,255	6,897
Corporate debt securities	299,413	164,297
Securities of government sponsored entities	10,000	30,042
Total Marketable Securities:	\$ 368,668	\$ 201,236

The following is a summary of short-term marketable securities classified as available-for-sale as of December 31, 2018 (*in thousands*):

	Contractual Maturity (in years)	Amortized Cost	Unrealized Gains	Unrealized Losses	Aggregate Estimated Fair Value
Marketable Securities:					
Commercial paper	Less than 1	\$ 59,313	\$ —	\$ (58)	\$ 59,255
Corporate debt securities	Less than 1	149,824	—	(604)	149,220
Total maturity less than 1 year		209,137	—	(662)	208,475
Corporate debt securities	1 to 2	150,813	18	(638)	150,193
Securities of government-sponsored entities	1 to 2	9,997	4	(1)	10,000
Total maturity 1 to 2 years		160,810	22	(639)	160,193
Total available-for-sale securities		\$ 369,947	\$ 22	\$ (1,301)	\$ 368,668

The following is a summary of short-term marketable securities classified as available-for-sale as of December 31, 2017 (*in thousands*):

	Contractual Maturity (in years)	Amortized Cost	Unrealized Losses	Aggregate Estimated Fair Value
Marketable Securities:				
Commercial paper	Less than 1	\$ 6,911	\$ (14)	\$ 6,897
Corporate debt securities	Less than 1	86,531	(198)	86,333
Securities of government-sponsored entities		30,132	(90)	30,042
Total maturity less than 1 year		123,574	(302)	123,272
Corporate debt securities	1 to 2	78,388	(424)	77,964
Total maturity 1 to 2 years		78,388	(424)	77,964
Total available-for-sale securities		\$ 201,962	\$ (726)	\$ 201,236

During 2018 and 2017, the Company had no realized gains or losses on marketable securities. During 2016, the Company realized a gain of less than \$0.1 million on marketable securities. The Company received proceeds from the sale or maturity of marketable securities of \$162.8 million, \$114.5 million and \$159.5 million for 2018, 2017 and 2016, respectively.

The primary objective of the Company's investment portfolio is to enhance overall returns while preserving capital and liquidity. The Company's investment policy limits interest-bearing security investments to certain types of instruments issued by institutions with primarily investment grade credit ratings and places restrictions on maturities and concentration by asset class and issuer.

The Company reviews the available-for-sale investments for other-than-temporary declines in fair value below cost basis each quarter and whenever events or changes in circumstances indicate that the cost basis of an asset may not be recoverable. This evaluation is based on a number of factors, including the length of time and the extent to which the fair value has been below the cost basis and adverse conditions related specifically to the security, including any changes to the credit rating of the security, and the intent to sell, or whether the Company will more likely than not be required to sell the security before recovery of its amortized cost basis. The assessment of whether a security is other-than-temporarily impaired could change in the future due to new developments or changes in assumptions related to any particular security. As of December 31, 2018 and 2017, the Company believed the cost basis for available-for-sale investments were recoverable in all material respects. For both December 31, 2018 and 2017, any investments in an unrealized loss position for longer than 12 months were immaterial.

NOTE 7. NOTES PAYABLE

Convertible Senior Notes Due 2025

On September 10, 2018, the Company completed its registered underwritten public offering of \$276 million aggregate principal amount of 2.50% Convertible Senior Notes due 2025 ("2025 Notes") and entered into a base indenture and supplemental indenture agreement ("2025 Indenture") with

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respect to the 2025 Notes. The 2025 Notes will mature on September 15, 2025 ("Maturity Date"), unless earlier repurchased, redeemed, or converted. The 2025 Notes are senior unsecured obligations of the Company and bear interest at an annual rate of 2.50%, payable semi-annually in arrears on March 15 and September 15 of each year, beginning on March 15, 2019.

The composition of the Company's 2025 Notes are as follows (*in thousands*):

	December 31, 2018	December 31, 2017
2.50% convertible senior notes due 2025	\$ 276,000	\$ —
Unamortized debt discount	(74,836)	—
Unamortized debt issuance costs	(6,073)	—
Total 2025 Notes, net of unamortized debt discount and debt issuance costs	\$ 195,091	\$ —

The net proceeds from the issuance of the 2025 Notes were approximately \$267.2 million, after deducting commissions and the offering expenses payable by the Company. A portion of the net proceeds from the 2025 Notes were used by the Company to repurchase \$23.4 million aggregate principal amount of its 4.5% senior convertible notes due in 2019 in privately-negotiated transactions.

Holders may convert their 2025 Notes at their option only in the following circumstances: (1) during any calendar quarter commencing after the calendar quarter ending on December 31, 2018 (and only during such calendar quarter), if the last reported sale price per share of the Company's common stock for each of at least 20 trading days, whether or not consecutive, during the period of 30 consecutive trading days ending on, and including, the last trading day of the immediately preceding calendar quarter exceeds 130% of the conversion price on the applicable trading day; (2) during the five consecutive business days immediately after any 10 consecutive trading day period ("measurement period") if the trading price per \$1,000 principal amount of 2025 Notes for each trading day of the measurement period was less than 98% of the product of the last reported sale price per share of the Company's common stock on such trading day and the conversion rate on such trading day; (3) upon the occurrence of certain corporate events or distributions on the Company's common stock; (4) if the Company calls the 2025 Notes for redemption; and (5) at any time from, and including, May 15, 2025 until the close of business on the scheduled trading day immediately before the Maturity Date. The Company will settle conversions by paying or delivering, as applicable, cash, shares of the Company's common stock, or a combination of cash and shares of the Company's common stock, at the Company's election, based on the applicable conversion rate.

The initial conversion rate for the 2025 Notes is 25.7739 shares of the Company's common stock per \$1,000 principal amount of 2025 Notes, which represents an initial conversion price of approximately \$38.80 per share. If a "make-whole fundamental change" (as defined in the 2025 Indenture) occurs, then the company will, in certain circumstances, increase the conversion rate for a specified period of time.

The 2025 Notes will be redeemable, in whole or in part, at the Company's option at any time, and from time to time, on or after September 15, 2022 and, in the case of any partial redemption, on or before the 40th scheduled trading day before the Maturity Date, at a cash redemption price equal to the principal amount of the 2025 Notes to be redeemed, plus accrued and unpaid interest, if any, to, but excluding, the redemption date, but only if the last reported sale price per share of the Company's common stock exceeds 130% of the conversion price on each of at least 20 trading days during the 30 consecutive trading days ending on, and including, the trading day immediately before the date the Company sends the related redemption notice. If a fundamental change (as defined in the 2025 Indenture) occurs, then, subject to certain exceptions, holders may require the Company to repurchase their 2025 Notes at a cash repurchase price equal to the principal amount of the 2025 Notes to be repurchased, plus accrued and unpaid interest, if any, to, but excluding, the fundamental change repurchase date.

As of December 31, 2018, the 2025 Notes had a market price of \$897 per \$1,000 or \$247.7 million principal amount. In the event of conversion, holders would forgo all future interest payments, any unpaid accrued interest and the possibility of further stock price appreciation. Upon the receipt of conversion requests, the settlement of the 2025 Notes will be paid pursuant to the terms of the 2025 Indenture. In the event that all of the 2025 Notes are converted, the Company would be required to repay the \$276.0 million in principal value and any conversion premium in any combination of cash and shares of its common stock at the Company's option. In addition, calling the 2025 Notes for redemption will constitute a "make whole fundamental change."

The 2025 Notes are the Company's general unsecured obligations that rank senior in right of payment to all of its indebtedness that is expressly subordinated in right of payment to the 2025 Notes, and equal in right of payment to the Company's unsecured indebtedness.

The 2025 Notes are currently classified on the Company's consolidated balance sheet at December 31, 2018 as long-term debt.

Under ASC 470-20, Debt with Conversion and Other Options, an entity must separately account for the liability and equity components of convertible debt instruments (such as the 2025 Notes) that may be settled entirely or partially in cash upon conversion, in a manner that reflects the issuer's economic interest cost. The liability component of the instrument is valued in a manner that reflects the market interest rate for a similar nonconvertible instrument at the date of issuance. The initial carrying value of the liability component was \$198.6 million. The equity component of \$77.4 million, representing the conversion option, was determined by deducting the fair value of the liability component from the par value of the 2025 Notes and is recorded in additional paid-in capital on the consolidated balance sheet at the issuance date. That equity component is treated as a discount on the liability component of the 2025 Notes, which is amortized over the seven year term of the 2025 Notes using the effective interest rate method. The equity component is not re-measured as long as it continues to meet the conditions for equity classification. The Company allocated the total transaction costs of approximately \$8.8 million related to the issuance of the 2025 Notes to the liability and equity components of the 2025 Notes based on their relative values. Transaction costs attributable to the liability component are amortized to interest expense over the

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seven-year term of the 2025 Notes, and transaction costs attributable to the equity component are netted with the equity component in stockholders' equity.

The effective interest rate on the liability components of the 2025 Notes for the period from the date of issuance through December 31, 2018 was 7.7%. The following table sets forth total interest expense recognized related to the 2025 Notes (*in thousands*):

	Twelve Months Ended December 31,	
	2018	2017
Contractual interest expense	\$ 2,108	\$ —
Amortization of debt discount	2,582	—
Amortization of debt issuance costs	273	—
Total interest expense for the 2025 Notes	\$ 4,963	\$ —

The 2025 Notes do not contain any financial or operating covenants or any restrictions on the payment of dividends, the issuance of other indebtedness or the issuance or repurchase of securities by the Company. The 2025 Indenture contains customary events of default with respect to the 2025 Notes, including that upon certain events of default, 100% of the principal and accrued and unpaid interest on the 2025 Notes will automatically become due and payable.

Convertible Senior Notes Due 2019

On May 29, 2014, the Company entered into a Note Purchase Agreement relating to a private placement by the Company of \$46 million aggregate principal senior convertible notes due 2019 (the "2019 Notes") which are convertible into shares of the Company's common stock at an initial conversion price of \$17.41 per share. The conversion price is subject to customary anti-dilution protection. The 2019 Notes bear interest at a rate of 4.5% per annum, payable semiannually in arrears on May 15 and November 15 of each year, beginning on November 15, 2014. The 2019 Notes mature on May 30, 2019 unless earlier converted or repurchased in accordance with the terms. The aggregate carrying value of the 2019 Notes on their issuance was \$43 million, which was net of the \$3 million debt discount.

In September 2018, the Company used part of the net proceeds from the issuance of the 2025 Notes discussed above to repurchase \$23.4 million aggregate principal value of the 2019 Notes in privately-negotiated transactions for approximately \$40.2 million in cash. The partial repurchase of the 2019 Notes resulted in a \$17.0 million loss on early extinguishment of debt.

As of December 31, 2018 the fair value of a share of common stock was \$22.63, exceeding the initial conversion price per share of the 2019 Notes. If the debt holders were to convert the Company would be required to issue 1,297,530 shares of common stock assuming that no fundamental change in the Company had occurred. The Company has reserved sufficient shares of its common stock to satisfy the conversion requirements related to the 2019 Notes. As of December 31, 2018, the convert value exceeded the carrying value by approximately \$6.8 million.

In estimating the fair value of the Company's convertible debt, the Company performed an analysis on the straight-debt portion and the conversion feature. To estimate the fair value of conversion feature, the Company used the Monte Carlo Simulation as of December 31, 2018. To estimate the fair value of straight-debt portion, excluding the conversion feature, the Company discounted to present value the scheduled coupon payments and principal repayment, using an appropriate fair market yield. As of December 31, 2018 the fair value of the debt was estimated at \$30.0 million using level 2 inputs.

The net carrying amount of the Notes consists of the following (*in thousands*):

	December 31,	
	2018	2017
Aggregate principle amount of Notes	\$ 22,590	\$ 46,000
Unamortized debt discount and debt issuance costs	(133)	(923)
	\$ 22,457	\$ 45,077

Interest Expense

Total interest expense recognized for the years ended December 31, 2018, 2017 and 2016 was \$9.8 million, \$4.4 million and \$4.7 million, respectively.

NOTE 8. FAIR VALUE MEASUREMENTS

The Company accounts for financial instruments in accordance with ASC 820, "Fair Value Measurements and Disclosures" ("ASC 820"). ASC 820 establishes a fair value hierarchy that prioritizes the inputs to valuation techniques used to measure fair value. The hierarchy gives the highest priority to unadjusted quoted prices in active markets for identical assets or liabilities (Level 1 measurements) and the lowest priority to unobservable inputs (Level 3 measurements). The three levels of the fair value hierarchy under ASC 820 are described below:

Level 1 – Unadjusted quoted prices in active markets that are accessible at the measurement date for identical, unrestricted assets or liabilities;

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Level 2 – Quoted prices in markets that are not active or financial instruments for which all significant inputs are observable, either directly or indirectly; and

Level 3 – Prices or valuations that require inputs that are both significant to the fair value measurement and unobservable.

In estimating the fair value of the Company's contingent consideration, the Company used the Monte Carlo Simulation model as of December 31, 2018, a probability-based expected method as of December 31, 2017 and the comparable uncontrolled transaction ("CUT") method in 2016 for royalty payments based on projected revenues. Based on the fair value hierarchy, the Company classified contingent consideration within Level 3 because valuation inputs are based on projected revenues discounted to a present value.

Financial instruments with carrying values approximating fair value include cash and cash equivalents, accounts receivable, notes receivable, deposits on lease agreements, and accounts payable, due to their short term nature.

The following table presents the Company's asset and liabilities that are measured and recognized at fair value on a recurring basis, classified under the appropriate level of the fair value hierarchy as of December 31, 2018 (in thousands):

	As of December, 2018	Fair Value Hierarchy at December 31, 2018		
	Total carrying and estimated fair value	Quoted prices in active markets (Level 1)	Significant other observable inputs (Level 2)	Significant unobservable inputs (Level 3)
Asset:				
Cash and Cash Equivalents	\$ 102,873	\$ 62,978	\$ 39,895	\$ —
Marketable securities, available-for-sale	368,668	—	368,668	—
Total	\$ 471,541	\$ 62,978	\$ 408,563	\$ —
Liabilities:				
Business combination-related contingent consideration	93,000	—	—	93,000
Total	\$ 93,000	\$ —	\$ —	\$ 93,000

The following table presents the Company's assets and liabilities that are measured and recognized at fair value on a recurring basis, classified under the appropriate level of the fair value hierarchy as of December 31, 2017 (in thousands):

	As of December, 2017	Fair Value Hierarchy at December 31, 2017		
	Total carrying and estimated fair value	Quoted prices in active markets (Level 1)	Significant other observable inputs (Level 2)	Significant unobservable inputs (Level 3)
Asset:				
Cash and Cash Equivalents	\$ 99,394	\$ 92,726	\$ 6,668	\$ —
Marketable securities, available-for-sale	201,236	—	201,236	—
Total	\$ 300,630	\$ 92,726	\$ 207,904	\$ —
Liabilities:				
Derivative liability related to warrants	\$ 15,710	\$ —	\$ —	\$ 15,710
Business combination-related contingent consideration	90,000	—	—	90,000
Total	\$ 105,710	\$ —	\$ —	\$ 105,710

The following table sets forth a summary of changes in the estimated fair value of the Company's Level 3 derivative liability for the year ended December 31, 2017 (in thousands):

	Fair Value Measurements of Common Stock Warrants (Level 3)
	2017
Balance at January 1,	\$ 22,440
Reclassification of derivative liability to equity upon exercise of warrants	(11,221)
Change in estimated fair value of liability classified warrants	4,491
Balance at December 31,	\$ 15,710

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The following table sets forth a summary of changes in the estimated fair value of the Company's Level 3 business combination-related contingent consideration for the years ended December 31, 2018 and 2017 (in thousands):

	Fair Value Measurements of Acquisition-Related Contingent Consideration (Level 3)	
	2018	2017
Balance at January 1,	\$ 90,000	\$ 87,478
Increase from revaluation of contingent consideration	11,590	19,389
Contractual Payments	(6,373)	(6,006)
Contractual Payments accrued at December 31	(2,171)	(11,012)
Foreign currency impact	(46)	151
Balance at December 31,	\$ 93,000	\$ 90,000

NOTE 9. INTANGIBLE ASSETS

Ligand License Agreement

In 2013, the Company entered into a \$2.5 million agreement with Ligand for a worldwide sublicense to develop, manufacture and commercialize a drug technology compound including sparsentan (the "Ligand License Agreement"). The cost of the Ligand License Agreement, which is presented net of amortization in the accompanying Consolidated Balance Sheet in intangible assets, net, is being amortized to research and development on a straight-line basis through September 30, 2023. As consideration for the license, we are required to make substantial payments upon the achievement of certain milestones, totaling up to \$114.1 million. Through 2018, we have made milestone payments to Ligand of \$7.2 million under the terms of the Ligand License Agreement. Should we commercialize sparsentan or any products containing related compounds, we will be obligated to pay to Ligand an escalating annual royalty between 15% and 17% of net sales of all such products.

In September 2015, the Ligand License Agreement was amended to facilitate sub-licensing in Asia-Pacific. As consideration for the amendment the Company paid \$1.0 million.

In March 2018, the Ligand License Agreement was amended to update certain development milestones set forth in the Sublicense Agreement to comport with the current development timeline for sparsentan. As consideration, the Company paid Ligand \$4.6 million, which replaced the amount that would have been due upon initiation of the first Phase 3 trial for sparsentan.

Manchester Pharmaceuticals LLC

In 2014, the Company acquired intangible assets with finite lives related to the Chenodal product rights, trade names, and customer relationships with the values of \$67.8 million, \$0.2 million, and \$0.4 million, respectively. The useful lives related to the acquired product rights, trade names, and customer relationships are expected to be approximately 16, 1 and, 10 years, respectively. Amortization of product rights, trade names and customer relationships are being recorded in selling, general and administrative expense over their respective lives.

Thiola License Agreement

The Company entered into a license agreement with Mission Pharmacal in 2014, in which the Company obtained an exclusive, royalty-bearing license to market, sell and commercialize Thiola (tiopronin) in the United States and Canada, and a non-exclusive license to use know-how relating to Thiola to the extent necessary to market Thiola. The initial term of the license is 10 years and will automatically renew thereafter for periods of one year.

The Company paid Mission an up-front license fee of \$3 million and will pay guaranteed minimum royalties during each calendar year the greater of \$2 million or twenty percent (20%) of the Company's net sales of Thiola through May 28, 2024.

In November 2017, the Company amended its agreement with Mission to extend the term of the current exclusive U.S. and Canada licensing agreement by an additional five years, to 2029. The royalty rate and guaranteed minimum payment were also extended through the new agreement term. Upon execution of the amendment, the Company capitalized an additional \$5.9 million in intangible assets and recorded a guaranteed minimum liability for the same amount.

In November 2018, the Company amended its agreement with Mission to extend the territorial rights beyond the United States and Canada. As consideration for the expanded territory the Company paid an up-front fee of \$0.3 million and will pay guaranteed minimum royalties equaling the greater of \$0.1 million or 20% of our Thiola net sales generated from outside of the United States during each calendar year. Upon execution of the amendment, the Company capitalized an additional \$1.0 million in intangible assets and recorded a guaranteed minimum liability of \$0.7 million related to this amendment.

The present value of guaranteed minimum royalties payable using a discount rate of ranging from approximately 7% to 11% based on the Company's then borrowing rate is \$15.2 million and \$15.1 million as of December 31, 2018 and 2017, respectively. As of December 31, 2018, the guaranteed minimum royalty current and long term liability was approximately \$2.1 million and \$13.1 million, respectively, and is recorded as guaranteed minimum royalty in the Consolidated Balance Sheet. As of December 31, 2017, the guaranteed minimum royalty current and long term liability was approximately \$2.0 million and \$13.1 million, respectively, and is recorded as guaranteed minimum royalty in the Consolidated Balance Sheet. The Company has

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capitalized \$70.0 million related to the Thiola intangible asset which consists of the up-front license fee, professional fees, present value of the guaranteed minimum royalties and any additional payments through 2018 in excess of minimum royalties.

There are 10.4 years remaining in the term of the license agreement.

Cholbam (Kolbam) Asset Purchase

On March 31, 2015, the Company completed its acquisition from Asklepiion of all worldwide rights, titles and ownership of Cholbam, including all related contracts, data assets, intellectual property, regulatory assets and the PRV. The Company capitalized \$75.9 million and \$7.3 million for the U.S. and international economic interest, respectively.

L-UDCA

On June 20, 2016, the Company signed a definitive agreement to purchase the rights, titles, and ownership of L-UDCA from Asklepiion. The purchase included \$25.5 million for an intangible asset with a definite life related to product rights for the U.S. The useful life related to the acquired product rights is expected to be approximately 17 years once the NDA is approved by the FDA. Until approval, the asset is considered IPR&D with an indefinite life and is not amortized.

Amortizable intangible assets as of December 31, 2018 (*in thousands*):

	Useful Life	Gross Carrying Amount	Accumulated Amortization	Net Book Value
Chenodal Product Rights	16	\$ 67,849	\$ (20,213)	\$ 47,636
Thiola License	15	70,009	(14,523)	55,486
Economic Interest - U.S. revenue Cholbam	10	75,900	(28,487)	47,413
Economic Interest - International revenue Cholbam	10	7,700	(2,890)	4,810
Economic Interest - L-UDCA (acquired IPR&D)	Indefinite	25,500	—	25,500
Ligand License	11	7,900	(2,397)	5,503
Manchester Customer Relationships	10	403	(192)	211
Manchester Trade Name	1	175	(175)	—
Internal use software	5	207	(75)	132
Total		\$ 255,643	\$ (68,952)	\$ 186,691

Amortizable intangible assets as of December 31, 2017 (*in thousands*):

	Useful Life	Gross Carrying Amount	Accumulated Amortization	Net Book Value
Chenodal Product Rights	16	\$ 67,849	\$ (15,976)	\$ 51,873
Thiola License	10	54,471	(10,168)	44,303
Economic Interest - U.S. revenue Cholbam	10	75,900	(20,903)	54,997
Economic Interest - International revenue Cholbam	10	8,058	(2,219)	5,839
Economic Interest - L-UDCA (acquired IPR&D)	Indefinite	25,500	—	25,500
Ligand License	11	3,300	(1,420)	1,880
Manchester Customer Relationships	10	403	(152)	251
Manchester Trade Name	1	175	(175)	—
Internal use software	5	207	(33)	174
Total		\$ 235,863	\$ (51,046)	\$ 184,817

The following table summarizes amortization expense for the twelve months ended December 31, 2018, 2017 and 2016 (*in thousands*):

	2018	2017	2016
Research and development	\$ 976	\$ 327	\$ 328
Selling, general and administrative	17,052	17,004	15,665
Total amortization expense	\$ 18,028	\$ 17,331	\$ 15,993

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As of December 31, 2018, amortization expense (excluding infinite lived IPR&D) for the next five years is expected to be as follows (*in thousands*):

2019	\$	19,166
2020		19,210
2021		19,158
2022		19,125
2023		18,825
Thereafter		65,707
Total	\$	<u>161,191</u>

NOTE 10. ACCRUED EXPENSES

Accrued expenses consist of the following at December 31, 2018 and 2017 (*in thousands*):

	2018	2017
Compensation related costs	\$ 10,446	\$ 7,749
Research and development	16,515	6,989
Government rebate reserves	8,464	5,883
Selling, general and administrative	2,990	3,896
Royalty/contingent consideration	6,805	6,429
Restructuring expenses	—	3,549
Miscellaneous accrued expenses	4,475	1,523
Total accrued expenses	\$ 49,695	\$ 36,018

NOTE 11. COMMITMENTS AND CONTINGENCIES

Leases

Facilities	Base Rent	Lease Expiration
Corporate Headquarters San Diego, CA	\$2.3 million	July 2024

In July 2016, the Company entered into an agreement to lease 23,107 square feet of office space, which commenced in December 2016, for a term of 7 years and 7 months. Under the terms of the lease, the Company will pay base annual rent (subject to an annual fixed percentage increase), plus property taxes, and other normal and necessary expenses, such as utilities, repairs, security and maintenance. Certain incentives were included in the lease, including approximately \$1.5 million in tenant improvement allowances and seven months of rent abatement. The Company has the right to extend the lease for five years.

In July 2017, the Company amended the office lease to add an additional 22,339 square feet of office space in an adjacent building.

Following is a schedule of the future minimum rental commitments for our operating lease as of December 31, 2018 (*in thousands*):

	Rental Payments
2019	\$ 2,343
2020	2,414
2021	2,486
2022	2,560
2023	2,637
Thereafter	1,585
	\$ <u>14,025</u>

Legal Proceedings

In August 2017, Martin Shkreli, the Company's former Chief Executive Officer, was convicted on securities fraud charges following investigations by the U.S. Attorney for the Eastern District of New York and the U.S. Securities and Exchange Commission. The Company was not a target of these investigations and cooperated with them fully. Mr. Shkreli has appealed his conviction to the United States Court of Appeals for the Second Circuit, and the appeal will likely not be decided until later in 2019. In connection with the trial and pending appeal proceedings, Mr. Shkreli sought advancement of his legal fees from the Company, and the Company has advanced a total of \$5.4 million in legal fees, of which \$3.8 million has been

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reimbursed by its directors' and officers' insurance carriers. Pending the outcome of Mr. Shkreli's appeal, the insurance carriers have reserved their rights to assert that certain of the advanced funds pertain to claims excluded from coverage under the relevant insurance policy and are therefore recoverable by the carriers. As a result, the final amount of the reimbursement from the insurance carriers is not currently estimable. In addition, a portion of these and the other legal fees the Company has advanced to Mr. Shkreli will be subject to reimbursement by Mr. Shkreli under Delaware law in the event it is ultimately determined that Mr. Shkreli is not entitled to be indemnified by the Company in these proceedings.

In August 2015, the Company filed a lawsuit in federal district court for the Southern District of New York against Mr. Shkreli, asserting that he breached his fiduciary duty of loyalty during his tenure as the Company's Chief Executive Officer and a member of its Board of Directors. Mr. Shkreli served a demand for JAMS arbitration on Retrophin, claiming that Retrophin had breached his December 2013 employment agreement. In response to Mr. Shkreli's arbitration demand, the Company asserted counterclaims in the arbitration that are substantially similar to the claims it previously asserted in the federal lawsuit against Mr. Shkreli. In October 2018, after the arbitration panel determined that Retrophin's counterclaims were arbitrable, the Company voluntarily dismissed the federal action without prejudice. The Company does not expect the claims and counterclaims in the arbitration to be heard by the arbitration panel before mid-2019. In connection with these proceedings, Mr. Shkreli sought advancement of his legal fees from the Company relating to his defense of the Company's claims against him. The Company has advanced, and expects to continue to advance, certain of these legal fees to Mr. Shkreli.

For the years ended December 31, 2018 and 2017, the Company recorded zero and \$2.6 million in expenses and paid zero and \$3.6 million related to advancements for Mr. Shkreli, respectively. The Company received zero and \$2.6 million in reimbursement from its directors' and officers' insurance carriers during the year ended December 31, 2018 and 2017, respectively. The reimbursement in 2017 is recorded as a liability on the Consolidated Balance Sheet pending the outcome of an appeal, if any.

From time to time the Company is involved in legal proceedings arising in the ordinary course of business. On October 23, 2018, Spring Pharmaceuticals, LLC (Spring) filed a lawsuit against the Company, Martin Shkreli, Mission Pharmacal Company and Alamo Pharma Services, Inc. in the United States District Court for the Eastern District of Pennsylvania alleging that the Company violated various federal and state antitrust and unfair competition laws by allegedly refusing to sell samples of the Thiola® brand drug so that Spring can conduct the bioequivalence testing needed to submit an ANDA to the FDA for approval to market a generic version of the product. Spring is seeking injunctive relief and damages. The Company intends to vigorously defend against Spring's claims. On January 15, 2019, the Company filed a motion to dismiss the lawsuit, which is in the process of being briefed.

The Company is not aware of any other proceedings or claims that could have, individually or in the aggregate, a material adverse effect on its results of operations or financial condition.

NOTE 12. STOCKHOLDERS' EQUITY / DEFICIT

Common Stock

The Company is currently authorized to issue up to 100,000,000 shares of \$0.0001 par value common stock. All issued shares of common stock are entitled to vote on a 1 share/1 vote basis.

Preferred Stock

The Company is currently authorized to issue up to 20,000,000 shares of \$0.0001 par value preferred stock, of which 1,000 shares are designated Class "A" Preferred shares, \$0.001 par value. Class A Preferred Shares are not entitled to interest, have certain liquidation preferences, special voting rights and other provisions. No preferred stock has been issued to date.

2015 Equity Incentive Plan

On June 8, 2015, the Company's stockholders approved the 2015 Equity Incentive Plan (the "2015 Plan"). The 2015 Plan is intended as the successor to and continuation of the Company's 2014 Incentive Compensation Plan. Stockholders approved 1.4 million new shares to be issued under the 2015 Plan, in addition to 0.6 million unallocated shares remaining available for issuance under the 2014 Incentive Compensation Plan that were added to the 2015 Plan.

On May 18, 2016, the Company's stockholders approved an amendment to the 2015 Plan (the "Amended 2015 Plan"). The amendment provides for an additional 1.6 million new shares to be issued under the Amended 2015 Plan, in addition to 0.7 million unallocated shares remaining available for issuance. The amendment also includes a provision that on or after March 21, 2016, the number of shares available for issuance under the Amended 2015 Plan will be reduced by one share for each share subject to a stock option or stock appreciation right and by 2.0 shares for each share subject to any other type of stock award issued pursuant to the Amended 2015 Plan, and any such shares will return to the share reserve at the same rates upon cancellation or other forfeiture of such awards or shares.

On May 17, 2017, the Company's stockholders approved an amendment to the Amended 2015 Plan. The amendment provides for an additional 1.8 million new shares to be issued under the Amended 2015 Plan.

2018 Equity Incentive Plan

On May 9, 2018, the Company's stockholders approved the 2018 Equity Incentive Plan (the "2018 Plan"). The 2018 Plan is intended as the successor to and continuation of the Amended 2015 Plan. Stockholders approved 1.8 million new shares to be issued under the 2018 Plan, in addition to 1.6 million unallocated shares remaining available for issuance under the Amended 2015 Plan that were added to the 2018 Plan.

2017 Employee Stock Purchase Plan

The 2017 Employee Stock Purchase Plan ("2017 ESPP") originated with 380,000 shares of common stock available for issuance. Beginning on January 1, 2018, and ending on (and including) January 1, 2026, the number of shares of common stock available for issuance under the 2017 ESPP shall increase by an amount equal to the lesser of (i) one percent (1%) of the total number of shares of common stock outstanding on December 31st of the preceding calendar year and (ii) 300,000 shares of common stock.

Substantially all employees are eligible to participate and, through payroll deductions, can purchase shares on established dates semi-annually. The purchase price per share sold pursuant to the 2017 ESPP will be the lower of (i) 85% of the fair market value of common stock on the first day of the offering period or (ii) 85% of the fair market value on the purchase date. Each offering period will span up to six months. Purchases may be up to 15% of qualified compensation, with an annual limit of \$25,000. The 2017 ESPP is intended to qualify as an "employee stock purchase plan" under Section 423 of the Internal Revenue Code.

As of December 31, 2018, there were approximately 680,000 shares authorized and 552,966 shares reserved for future issuance under the 2017 ESPP.

Stock Options

The fair values of stock option grants during the year ended December 31, 2018, 2017 and 2016 were calculated on the date of grant using the Black-Scholes option pricing model. Compensation expense is recognized over the period of service, generally the vesting period. During the year ended December 31, 2018, 1,349,250 stock options were granted by the Company. The following weighted average assumptions were used in the Black-Scholes options pricing model to estimate the fair value of stock options for the specified reporting periods:

	Twelve Months Ended December 31,		
	2018	2017	2016
Risk free rate	2.80%	2.10%	1.20%
Expected volatility	68%	70%	68%
Expected life (in years)	6.2	6.1	5.8
Expected dividend yield	—	—	—

The risk-free interest rate was based on rates established by the Federal Reserve. The Company's expected volatility was based on analysis of the Company's volatility, as well as the volatilities of guideline companies. The expected life of the Company's options was determined using the simplified method as a result of limited historical data regarding the Company's exercise activity. The dividend yield is based upon the fact that the Company has not historically paid dividends, and does not expect to pay dividends in the foreseeable future.

The following table summarizes our stock option activity and related information for the years ended December 31, 2018:

	Shares Underlying Options	Weighted Average		Aggregate Intrinsic Value (in thousands)
		Exercise Price	Remaining Contractual Term (in years)	
Outstanding at December 31, 2017	7,153,668	\$ 17.16	6.95	\$ 39,010
Granted	1,349,250	25.56	—	—
Forfeited and expired	(476,369)	24.50	—	—
Exercised	(749,212)	14.13	—	9,325
Outstanding at December 31, 2018	7,277,337	\$ 18.55	6.94	\$ 40,650

The following table summarizes our stock options exercisable at December 31, 2018, 2017 and 2016:

	Shares Underlying Options	Weighted Average		Aggregate Intrinsic Value (in thousands)
		Exercise Price	Remaining Contractual Term (in years)	
2016	3,793,017	\$ 14.94	6.82	\$ 23,358
2017	4,610,233	\$ 15.97	5.85	\$ 31,991
2018	4,834,781	\$ 16.81	5.98	\$ 35,387

The weighted average grant date fair value of options granted was \$16.21, \$11.77, and \$10.09 during the years ended December 31, 2018, 2017 and 2016, respectively. The aggregate intrinsic value for outstanding options is calculated as the difference between the exercise price of the underlying awards and the closing price of the Company's common stock of \$22.63, \$21.07 and \$18.93 as of December 31, 2018, 2017 and 2016,

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respectively. Unrecognized compensation cost associated with unvested stock options amounts to \$30.0 million as of December 31, 2018, which will be expensed over a weighted average remaining vesting period of 2.7 years.

Restricted Stock Units

As of December 31, 2018, there was approximately \$7.9 million of unrecognized compensation cost related to restricted stock units ("RSUs") granted. This amount is expected to be recognized over a weighted average period of 2.9 years.

The following table summarizes our restricted stock unit activity for the year ended December 31, 2018:

	Number of RSUs	Weighted Average Grant Date Fair Value
Unvested December 31, 2017	94,832	\$ 20.19
Granted	395,311	25.40
Vested	(58,251)	20.08
Forfeited/cancelled	(31,466)	25.20
Unvested December 31, 2018	400,426	\$ 24.95

Performance-based Stock Units

As of December 31, 2018, there was approximately \$1.6 million of unrecognized compensation cost related to performance-based stock units ("PSUs") granted. This amount is expected to be recognized over a weighted average period of 1.3 years.

The following table summarizes our performance-based stock unit activity for the year ended December 31, 2018:

	Number of PSUs	Weighted Average Grant Date Fair Value
Unvested December 31, 2017	250,500	\$ 20.63
Granted	66,500	25.75
Vested	(85,250)	22.33
Forfeited/cancelled	(5,000)	18.46
Unvested December 31, 2018	226,750	\$ 21.54

Share Based Compensation

Total non-cash stock-based compensation expense consisted of the following for the years ended December 31, 2018, 2017 and 2016 (*in thousands*):

	Twelve Months Ended December 31,		
	2018	2017	2016
Selling, general and administrative expenses	\$ 13,550	\$ 17,924	\$ 18,614
Research and development expenses	6,224	8,950	10,488
Total	\$ 19,774	\$ 26,874	\$ 29,102

Exercise of Warrants

During the twelve months ended December 31, 2018, 2017 and 2016, the Company issued the following shares of common stock upon the exercise of warrants for cash received by the Company: (*in thousands except share amounts*)

	Shares Issued	Cash Received	Derivative Liability Reclassified as Equity	Change in Fair Value Expense
2016	898,633	\$ 6,005	\$ 14,715	\$ 2,909
2017	607,481	\$ 3,645	\$ 11,221	\$ 3,033
2018	1,036,054	\$ 5,305	n/a	n/a

As of December 31, 2018 there are no warrants for common shares outstanding. See Note 15 for further discussion.

NOTE 13. EARNINGS (LOSS) PER SHARE

Basic earnings (loss) per share ("EPS") represents net income (loss) attributable to common shareholders divided by the weighted average number of common shares outstanding during the measurement period. Diluted EPS represents net income attributable to common shareholders divided by the weighted average number of common shares outstanding during the measurement period while also giving effect to all potentially dilutive common shares that were outstanding during the period using the treasury stock method.

Basic and diluted EPS is calculated as follows (*net income amounts are stated in thousands*):

	For the year ended December 31,								
	2018			2017			2016		
	Shares	Net loss	EPS	Shares	Net loss	EPS	Shares	Net Income	EPS
Basic Earnings per Share	40,433,171	\$ (102,678)	\$ (2.54)	38,769,816	\$ (59,731)	\$ (1.54)	36,997,865	\$ (47,903)	\$ (1.29)
Dilutive shares related to warrants	—	—	—	—	—	—	1,290,147	—	—
Change in fair value of derivative instruments	—	—	—	—	—	—	—	(1,655)	—
Dilutive Earnings per Share	40,433,171	\$ (102,678)	\$ (2.54)	38,769,816	\$ (59,731)	\$ (1.54)	38,288,012	\$ (49,558)	\$ (1.29)

For the years ended December 31, 2018, 2017 and 2016, the following shares were excluded because they were anti-dilutive:

	For the year ended December 31,		
	2018	2017	2016
Convertible Debt	8,410,932	2,642,160	2,642,160
Restricted Stock	395,034	157,319	444,942
Options	7,210,576	7,080,998	6,286,584
Warrants	282,807	1,159,424	—
Total Anti-Dilutive Shares	16,299,349	11,039,901	9,373,686

NOTE 14. INCOME TAXES

For financial reporting purposes, net income (loss) before income taxes includes the following components (*in thousands*):

	Year Ended December 31,		
	2018	2017	2016
United States	\$ (87,573)	\$ (55,611)	\$ (52,750)
Foreign	(14,294)	(2,752)	(4,832)
Total	\$ (101,867)	\$ (58,363)	\$ (57,582)

The components of the provision (benefit) for income taxes, in the Consolidated Statement of Operations are as follows (*in thousands*):

	2018			2017			2016		
Current									
Federal	\$	698	\$	6,991	\$	13,137			
State		113		802		(155)			
		811		7,793		12,982			
Deferred									
Federal		—		(7,965)		(18,814)			
State		—		1,540		(3,847)			
		—		(6,425)		(22,661)			
Total tax provision (benefit)	\$	811	\$	1,368	\$	(9,679)			

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The following is a reconciliation of the statutory federal income tax rate to the Company's effective tax rate expressed as a percentage of income (loss) before income taxes:

	2018	2017	2016
Statutory rate - federal	(21.00)%	(35.00)%	(35.00)%
State taxes, net of federal benefit	(4.44)%	(3.30)%	(3.16)%
Change in FV of derivative liability (warrants)	— %	2.82 %	1.10 %
Change in federal tax rate	— %	23.29 %	— %
Convertible Debt	21.77 %	— %	— %
Loss on extinguishment of debt	4.09 %	— %	— %
Other permanent differences	0.10 %	1.04 %	2.05 %
Tax credits	(11.86)%	(5.79)%	(1.58)%
Return to provision adjustments and other true-ups	1.42 %	(3.48)%	(1.15)%
Other	1.06 %	1.25 %	3.09 %
Change in valuation allowance	9.79 %	21.62 %	16.30 %
Income tax provision (benefit)	0.93 %	2.45 %	(18.35)%

The significant components of the Company's deferred tax assets and liabilities as of December 31, 2018 and 2017 are as follows (*in thousands*):

	2018	2017
Deferred Tax Assets:		
Net operating loss	\$ 9,700	\$ 1,099
Research and development and other tax credits	14,715	1,599
Contingent consideration	23,459	23,080
Other accrued expenses	3,710	2,603
Stock based compensation	16,761	15,695
Other	555	358
	68,900	44,434
Deferred Tax Liabilities:		
Intangible assets	(14,288)	(16,810)
Convertible Debt	(18,419)	—
Tax basis depreciation less than book depreciation	—	—
	(32,707)	(16,810)
Net deferred tax assets (liabilities) before valuation allowance	36,194	27,624
Valuation allowance	(36,194)	(27,624)
Total deferred tax liability	\$ —	\$ —

The Company has established a full valuation allowance against its U.S. federal and state deferred tax assets due to the uncertainty surrounding the realization of such assets in future periods. The ultimate realization of deferred tax assets is dependent upon the generation of future taxable income during the periods in which temporary differences become deductible. Management considers the scheduled reversal of deferred liabilities and tax planning strategies in making this assessment and evaluates the recoverability of the deferred tax assets as of each reporting date. At such time as it is determined that it is more likely than not that deferred assets are realizable, the valuation allowance will be reduced accordingly and recorded as a tax benefit.

The Company has recorded a valuation allowance of \$36.2 million as of December 31, 2018 to reflect the estimated amount of deferred tax assets that may not be realized. The Company increased its valuation allowance by \$8.6 million for the year ended December 31, 2018.

At December 31, 2018, the Company had available unused U.S. federal and state net operating loss ("NOL") carryforwards of \$36.5 million and \$33.1 million, respectively, all of which are fully offset by a valuation allowance. The U.S. federal NOL carryforwards generated for tax years ending on or prior to December 31, 2017 and the state tax loss carryforwards will begin to expire in 2030 and 2022, respectively. In addition, at December 31, 2018, the Company had federal orphan drug tax credit carryforwards of \$12.8 million that begin to expire in 2037 unless utilized, federal research and development tax credit carryforwards of \$0.5 million that begin to expire in 2038 unless utilized and California Competes tax credit carryforwards of \$2.0 million that begin to expire in 2022. The Company has international subsidiaries whose operations are not material for the year ended December 31, 2018.

The Company accounts for uncertain tax benefits in accordance with the provisions of ASC 740-10 of the *Accounting for Uncertainty in Income Taxes*. As of December 31, 2018 the Company had no unrecognized tax benefits.

The Company does not anticipate that the amount of unrecognized tax benefits as of December 31, 2018 will change materially within the following 12 months.

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A reconciliation of the Company's unrecognized tax benefits for the years 2018 and 2017 is provided in the following table (*in thousands*):

	2018	2017
Balance as of January 1:	\$ —	\$ 1,500
Increase in current period positions	—	—
Decrease in prior period positions	—	(1,500)
Increase in prior period positions	—	—
Balance as of December 31:	\$ —	\$ —

The Company files income tax returns in the U.S. federal jurisdiction, various state and local, and foreign jurisdictions. The Company's income tax returns are open to examination by federal, state and foreign tax authorities, generally for the years ended December 31, 2015 and later.

The Company's policy is to record estimated interest and penalties related to the underpayment of income taxes or unrecognized tax benefits as a component of its income tax provision. During the years ended 2018, 2017 and 2016, the Company did not recognize any interest or penalties in its Consolidated Statements of Operations and Comprehensive Income (Loss) and there were no accruals recorded for interest or penalties at December 31, 2018 and 2017.

U.S. Tax Reform

The Tax Act of 2017 was enacted on December 22, 2017. The Tax Act of 2017 reduces the US federal corporate tax rate from 35% to 21%, as well as making several other significant changes to the tax law, effective January 1, 2018. Pursuant to the Securities and Exchange Commission Staff Accounting Bulletin No. 118, Income Tax Accounting Implications of the Tax Cuts and Jobs Act (SAB 118), given the amount and complexity of the changes in tax law resulting from the Tax Act of 2017, the Company had not finalized the accounting for the income tax effects of the Tax Act of 2017 as of December 31, 2017.

We completed our accounting for Tax Reform on December 22, 2018 and as of December 31, 2018, the Company's accounting for the following elements of the Tax Act of 2017 were completed and there were no changes to the provisional amounts previously recorded.

Revaluation of deferred tax assets and liabilities

We have remeasured our deferred tax assets and liabilities based on the rates at which they are expected to reverse in the future, which is generally 21% plus state and local tax. The Company recorded a decrease related to our deferred tax assets and liabilities of \$13.0 million as a result of the tax rate decrease, with a corresponding adjustment to our valuation allowance for the year ended December 31, 2017.

Valuation allowances

The Company must assess whether its valuation allowance analyses for deferred tax assets are affected by various aspects of the Tax Act of 2017 (e.g., deemed repatriation of deferred foreign income, future GILTI inclusions, new categories of foreign tax credits). At December 31, 2017, the Company increased its valuation allowance by \$12.0 million as a result of the Tax Act of 2017 and its effects on the realizability of our deferred tax assets.

NOTE 15. DERIVATIVE FINANCIAL INSTRUMENTS

Since 2013, the Company has issued 5 tranches of common stock purchase warrants to secure financing, remediate covenant violations related to a credit facility and provide consideration for credit facility amendments.

Historically, the Company accounted for these instruments, which do not have fixed settlement provisions, as derivative instruments in accordance with FASB ASC 815-40, *Derivative and Hedging - Contracts in Entity's Own Equity*. This was due to an anti-dilution provision for the warrants that provides for a reduction to the exercise price if the Company issues equity or equity linked instruments in the future at an effective price per share less than the exercise price then in effect for the warrant ("down round provision"). As such, the warrants were re-measured at each balance sheet date based on estimated fair value. Changes in estimated fair value were recorded as non-cash adjustments within other income (expenses), net, in the Company's

As of January 1, 2018, the Company early adopted ASU 2017-11, which revised the guidance for instruments with down round provisions. As such the Company treats outstanding warrants as free-standing equity linked instruments that will be recorded to equity in the Consolidated Balance Sheet.

The fair value of the derivative liability balance as of December 31, 2017 of \$15.7 million was reclassified by means of a cumulative-effect adjustment to equity as of January 1, 2018.

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The following table presents the Company's derivative warrant issuances and balances outstanding during the years ended December 31, 2018 and 2017:

	Warrants	Weighted Average	
		Exercise Price	Grant Date Fair Value
Outstanding at December 31, 2016	1,766,905	\$ 7.23	\$ 3.87
Issued	—	—	—
Canceled	—	—	—
Exercised	607,481	6.00	3.33
Outstanding at December 31, 2017	1,159,424	\$ 7.86	\$ 4.15
Issued	—	—	—
Canceled	554	5.99	3.33
Exercised	1,158,870	7.88	4.15
Outstanding at December 31, 2018	—	—	—

As of December 31, 2018 there are no outstanding warrants.

NOTE 16. RETIREMENT PLAN

The Company has a 401(k) defined contribution savings plan for the benefit of all eligible employees. Employer matching contributions were \$0.9 million, \$0.6 million, and \$0.5 million for the years ended December 31, 2018, 2017 and 2016, respectively.

NOTE 17. RESTRUCTURING

On March 7, 2017, the Company initiated a plan to consolidate its operations to its corporate headquarters in San Diego, California. The Company adjusted employee related separation charges by \$0.2 million in the current year as a result of this consolidation.

The following table presents a reconciliation of the restructuring liability recorded within accrued expenses on the Company's Condensed Consolidated Balance Sheets (*in thousands*):

	Twelve Months Ended December 31,	
	2018	2017
Liability, beginning of period	\$ 3,549	\$ 893
Restructuring expenses	—	3,608
Cash settlements	(3,307)	(897)
Adjustments to previous estimates	(242)	(55)
Liability, end of period	\$ —	\$ 3,549

NOTE 18. PROPERTY AND EQUIPMENT

Property, plant and equipment, net consisted of the following (*in thousands*):

	December 31,	
	2018	2017
Computers and equipment	\$ 506	\$ 436
Furniture and fixtures	1,150	945
Leasehold improvements	2,628	2,071
Construction-in-progress	—	363
	4,284	3,815
Less: Accumulated depreciation	(1,138)	(585)
Total property and equipment, net	\$ 3,146	\$ 3,230

The construction-in-process balance consists of costs related to the Company's leasehold improvements at its facilities in San Diego, California.

Depreciation expense for the years ended December 31, 2018, 2017 and 2016 was \$0.6 million, \$0.5 million and \$0.1 million, respectively.

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The Company has not capitalized interest related to the property and equipment purchases.

NOTE 19. QUARTERLY FINANCIAL INFORMATION (UNAUDITED)

The following table presents selected consolidated statements of operations data for each quarter for the fiscal years ended December 31, 2018 and 2017 (*unaudited, in thousands, except for per share data*):

	Fourth Quarter	Third Quarter	Second Quarter	First Quarter
For the year ended December 31, 2018:				
Net product sales	\$ 43,771	\$ 40,706	\$ 41,337	\$ 38,432
Total operating expenses	48,756	76,289	62,897	56,344
Operating loss	(4,985)	(35,583)	(21,560)	(17,912)
Total other income (expense), net	(2,470)	(18,518) ¹	(602)	(237)
Loss before provision for income taxes	(7,455)	(54,101)	(22,162)	(18,149)
Income tax benefit (provision)	—	(415)	(167)	(229)
Net income (loss)	\$ (7,455)	\$ (54,516)	\$ (22,329)	\$ (18,378)
Net Loss per common share				
Basic	\$ (0.18)	\$ (1.34)	\$ (0.56)	\$ (0.46)
Diluted	\$ (0.18)	\$ (1.34)	\$ (0.56)	\$ (0.46)
For the year ended December 31, 2017:				
Net product sales	\$ 42,177	\$ 40,340	\$ 38,800	\$ 33,620
Total operating expenses	57,354	50,948	52,398	48,028
Operating loss	(15,177)	(10,608)	(13,598)	(14,408)
Total other income (expense), net	4,139	(8,409)	(1,556)	1,254
Income (loss) before provision for income taxes	(11,038)	(19,017)	(15,154)	(13,154)
Income tax benefit (provision)	(6,580)	1,223	1,925	2,064
Net income (loss)	\$ (17,618)	\$ (17,794)	\$ (13,229)	\$ (11,090)
Net income (loss) per common share				
Basic	\$ (0.45)	\$ (0.46)	\$ (0.34)	\$ (0.29)
Diluted	\$ (0.55)	\$ (0.46)	\$ (0.34)	\$ (0.32)

¹ In September 2018, the Company executed a partial repurchase of the 2019 Notes that resulted in a \$17.0 million loss on early extinguishment of debt. See Note 7 for further discussion.

FOURTH AMENDMENT TO TRADEMARK LICENSE AND SUPPLY AGREEMENT

This Fourth Amendment to Trademark License and Supply Agreement (“**Fourth Amendment**”), made effective November 28, 2018 (the “**Fourth Amendment Effective Date**”), amends the Trademark License and Supply Agreement dated May 28, 2014 by and between Mission Pharmacal Company (“**Mission**”) and Retrophin, Inc. (together with its affiliates, “**Retrophin**”) (such agreement as previously amended by the amendments listed in Exhibit A, the “**Agreement**”).

WHEREAS, Mission and Retrophin have mutually agreed to expand the “Territory” exclusively licensed to Retrophin under the Agreement to become worldwide, and have agreed to associated economic terms, and wish to set forth all of the foregoing terms in this Fourth Amendment.

NOW THEREFORE, for good and valuable consideration, the receipt and adequacy of which is hereby acknowledged, it is agreed as follows:

1. **Terminology.** All references in this Fourth Amendment to “Sections” and Articles are to articles and sections of the Agreement. All initially capitalized terms used and not defined in this Fourth Amendment, but defined in the Agreement, have the meanings given in the Agreement. For clarity, all initially capitalized terms defined in this Fourth Amendment, and used in an amended Section of the Agreement provided below, have the meanings in the Agreement that are given in this Fourth Amendment.
2. **License Fee for Expanded Territory.** Within ten (10) days of the signing of this Amendment by the later-to-sign of Mission and Retrophin, Retrophin shall pay Mission a license fee equal to two hundred fifty thousand dollars (\$250,000) by wire transfer of immediately available funds. Such license fee shall be non-refundable. Such license fee is in respect of the expansion of the Territory under the Agreement to become worldwide.
3. **Amendments.**
 - a. Section 2.6 of the Agreement is deleted and replaced with the following:

2.6 “**Territory**” means worldwide.
 - b. Article 7 (including Sections 7.0 and 7.1) of the Agreement is deleted and replaced with the following:

7.0 **Trademark Royalty.** Retrophin shall pay Mission a quarterly trademark royalty (“**Trademark Royalty**”) equal to twenty percent (20%) of Retrophin’s Net Sales of the Product. The Trademark Royalty will be paid in accordance with the provisions of Section 11.2 below. For clarity, this applies to all Net Sales under this Agreement, including, after the Fourth Amendment Effective Date, Net Sales worldwide.

7.1 **Minimum U.S./Canada Royalty.** If and only if the total sum of all Trademark Royalties paid during a calendar year with respect to Net Sales in the United States and Canada is less than two million dollars (\$2,000,000.00), then Retrophin shall pay Mission an additional royalty payment for the difference such that the total sum of all royalties paid during such calendar year with respect to Net Sales in the United States and Canada will be, at a minimum, two million dollars (\$2,000,000.00), such amount to be prorated for calendar year 2014 and the last calendar year of this Agreement.

7.2 **Minimum ROW Royalty.** This Section shall apply from and after the calendar year in which the Fourth Amendment Effective Date occurs. If and only if the total sum of all Trademark Royalties paid during a calendar year from and after the Fourth Amendment Effective Date with respect to Net Sales outside the United States and Canada is less than one hundred thousand dollars (\$100,000), then Retrophin shall pay Mission an additional royalty payment for the difference such that the total sum of all royalties paid during such calendar year with respect to Net Sales outside the United States and Canada will be, at a minimum, one hundred thousand dollars (\$100,000), such amount to be prorated for the calendar year in which the Fourth Amendment Effective Date occurs and the last calendar year of this Agreement.
4. **Exclusive Supply.** For the avoidance of doubt, all Product sold under the license of the Agreement must be supplied by Mission or its designated supplier.
5. **Agreement In Full Force and Effect.** Except as set forth above, the Agreement shall remain in full force and effect.
6. **Entire Agreement.** All noted Amendments to the Agreement, including this Amendment and the Agreement, together constitute the entire agreement of the parties hereto with respect to the topics addressed therein and supersedes any and all prior agreements,

whether oral or in writing between the parties hereto with respect to subject matter hereof. This Amendment may not be amended, modified or supplemented except by written agreement of the parties hereto. This Amendment may be executed in one or more counterparts, each of which shall be deemed and original, but all of which together shall constitute one and the same instrument.

IN WITNESS HEREOF, the undersigned have executed this Amendment on the day and year first above written.

[SIGNATURE PAGE FOLLOWS]

RETROPHIN, INC.

MISSION PHARMACAL COMPANY

By: /s/ Stephen Aselage _____ By: /s/ Thomas J. Dooley
Name: Stephen Aselage Name: Thomas J. Dooley
Title: Chief Executive Officer Title: Chief Financial Officer

Exhibit A to Fourth Amendment - Prior Amendments to Agreement

1. First Amendment to Trademark License and Supply Agreement, dated July 28, 2014.
2. Second Amendment to Trademark License and Supply Agreement, dated September 24, 2015.
3. Third Amendment to Trademark License and Supply Agreement, dated March 17, 2016.
 - a. Amendment One to the Third Amendment to Trademark License and Supply Agreement, dated September 12, 2016.
 - b. Amendment Two to the Third Amendment to Trademark License and Supply Agreement, dated November 3, 2017.

2019 Retrophin, Inc. Executive Officer Annual Bonus Plan

Plan Objective

The purpose of the Retrophin, Inc. Executive Officer Bonus Plan (the “Plan”) is to provide incentives to and reward executive officers of Retrophin, Inc. (the “Company”) (each a “Participant,” as defined below) to achieve corporate performance goals and to work together to achieve outstanding results in all aspects of the Company’s business, thus benefiting themselves, Company shareholders and the people who benefit from the Company’s services.

Eligibility

- All regular full-time executive officers of Retrophin are eligible to receive a bonus under this Plan (“Participant”).
- Participants must be employed as a regular full-time employee by the Company prior to October 1 of the bonus plan year.
- In order to be eligible to receive a bonus for a particular Bonus Plan Year (if any is earned), a Participant must be actively employed, and in good standing, as of the date the bonus checks are distributed for that year or as otherwise approved by the Board.
- Temporary executive officers and consultants (regardless of their roles or responsibilities) are not eligible to participate.
- Participation in the “Retrophin, Inc. Executive Officer Bonus Plan” is approved on an annual basis. Criteria for participation may be subject to change at the commencement of the Bonus Plan Year, and eligibility to participate in any Bonus Plan Year does not guarantee eligibility to participate in any subsequent Bonus Plan Year.
- Participants whose individual performance is deemed to not be meeting expectations by the Compensation Committee are ineligible.

Definitions

- “Bonus Plan Year” means the twelve-month period beginning on each January 1 and ending on each December 31.
- The “Board” means the Board of Directors of the Company.
- The “Compensation Committee” means the Compensation Committee of the Board, as constituted from time to time.
- The “Base Pay” is a Participant’s annual rate of base salary in effect as of December 31st of the applicable Bonus Plan Year.
- The “Company Target Performance Measures” shall be determined at the sole discretion of the Compensation Committee or the Board and shall be set forth in writing, and may include, but shall not be limited to, a combination of financial, research and development and/or operational goals.
- The “Company Modifier” is determined at the sole discretion of the Compensation Committee or the Board and is designed to reflect performance against Company results. For illustration purposes only, if the Company performance significantly exceeds the Company Target Performance Measures, the Company Modifier could exceed 100%, but in no case more than 150%. Similarly, if Company performance fails to meet the Company Target Performance Measures, the Company Modifier could be less than 100%. There is a minimum Corporate Performance required of 40% for any payment under the Plan to be considered. No Participant will have any entitlement to or earn a right to receive a bonus under this Plan until the date on which such bonus is paid. The Board and/or Compensation Committee reserve the right, at any time, regardless of corporate performance to approve or not approve the payment of a Bonus during any Plan Year.

under section 409A of the Code or successor guidance thereto. In the administration and interpretation of the Plan, such intention is to govern.

- It is intended that this Plan be exempt from regulation under the Employee Retirement Income Security Act of 1974, as amended, as a “payroll practice” and a “bonus program”, as described in U.S. Department of Labor Regulations 2510.3-1(b) and 2510.3-2(c), respectively.
- Any bonuses paid under the Bonus Plan shall be subject to the provisions of any claw-back policy implemented by the Company, including, without limitation, any claw-back policy adopted to comply with the requirements of the Dodd-Frank Wall Street Reform and Consumer Protection Act and any rules, regulations or interpretations thereunder.
- This Plan shall be subject to and construed in accordance with the laws of the State of California without regard to conflicts of laws.
- The Compensation Committee possesses sole discretion and authority to construe and interpret the terms and provisions of the Plan and to resolve any issue arising out of, relating to, or resulting from its administration and operation. Any disagreement or dispute by any person claiming a benefit under the Plan regarding any aspect of the Plan or its administration must be promptly presented in writing to the Compensation Committee for determination. Payments shall be made under the Plan only if the Compensation Committee determines in its sole discretion that the claimant is entitled to them. Any determinations the Compensation Committee makes in relation to the Plan will be final, conclusive, and binding on all persons, entities and parties claiming any interest under the Plan and will be entitled to the maximum possible deference allowed by law.
- Except as explicitly provided by law, this Plan is provided at the Company's sole discretion, and the Company reserves the power at any time and from time to time, to modify, amend or terminate (in whole or in part) any or all of the provisions of the Plan at any time, prospectively or retroactively, without prior notice or obligation. Any amendment to the Plan shall be adopted by formal action of the Board.
- The Plan will be operated as an unfunded arrangement, and nothing in this document will be construed to require the Company to fund any awards or to establish a trust or purchase an insurance policy or other product for such purpose. The Company may make such arrangements as it desires to provide for the payment of bonuses under the Plan.
- Any payments made pursuant to the Plan shall not be counted as compensation for purposes of any other employee benefit plan, program or agreement sponsored, maintained or contributed to by the Company unless expressly provided for in such employee benefit plan, program, agreement, or arrangement.

Retrophin, Inc.
3721 Valley Centre Drive, Suite 200
San Diego, CA 92130

January 4, 2019

Stephen Aselage

Re: Retirement Agreement

Dear Steve:

This letter agreement (the "**Agreement**") sets forth our mutual understanding regarding your retirement as an employee of Retrophin, Inc., a Delaware corporation (the "**Company**").

1. **Retirement.** Effective as of the date of this Agreement (the "**Retirement Date**"), you hereby resign as the Company's Chief Executive Officer, and from all other offices and positions held by you at the Company or at any subsidiary of the Company (with the exception of your position as a member of the Company's Board of Directors (the "**Board**"), as addressed in paragraph 2 below).

2. **Continued Board Service.** Following the Retirement Date, you will continue serving as a member of the Board, to serve until your successor is duly elected and qualified.

3. **Transition Assistance.** In order to assist with an orderly transition of your CEO duties, you agree to provide transition support to the Company on an as-needed basis.

4. **Retirement Benefits.**

(a) Effective as of the Retirement Date, you will be entitled to receive the benefits set forth in Section 6.5(b)(i), Section 6.5(b)(ii) and Section 6.5(c)(i) of your employment agreement with the Company dated March 2, 2015, as amended on April 11, 2017 (the "**Employment Agreement**").

(b) Notwithstanding the terms set forth in Section 3.2 of the Employment Agreement, you will remain eligible to receive your annual incentive bonus payment for 2018, in an amount to be determined by the Board or the Compensation Committee of the Board in their sole discretion, which the Company anticipates paying on or before March 15, 2019.

(c) In the event that your service as a member of the Board terminates prior to the 18-month anniversary of the Retirement Date, the vesting of all outstanding Stock Awards (as defined in the Employment Agreement) with time-based vesting that were held by you on the Retirement Date shall be accelerated such that the amount of shares vested under such time-based Stock Awards shall equal that number of shares that would have been vested if you had continued to render continuous service to the Company for the 18 months immediately following the Retirement Date.

(d) In the event that your service as a member of the Board terminates prior to the 18-month anniversary of the Retirement Date, all outstanding Stock Awards with performance-based vesting held by you for which the relevant performance period ends within the 18-month period following the Retirement Date shall remain eligible for vesting during such 18-month period as though you had continued to render continuous service to the Company throughout such period, and such Stock Awards with performance-based vesting shall vest (if applicable) based on actual performance during such performance period. For the avoidance of doubt, you acknowledge and agree that, on the 18-month anniversary of the Retirement Date, all unvested Stock Awards with performance-based vesting then-held by you shall automatically expire and terminate.

5. **Release.** As a condition to the benefits provided in this Agreement to which you would not otherwise be entitled, you agree, on the Retirement Date, to execute and return to the Company the General Release attached as Exhibit A to the Employment Agreement (the "**Release**"), and to allow the Release to become effective.

6. **General.** This Agreement, including its exhibits, and the Employment Agreement constitute the complete, final and exclusive embodiment of the entire agreement between you and the Company with regard to this subject matter. This Agreement may not be modified or amended except in a writing signed by both you and a duly authorized officer of the Company. This Agreement will be deemed to have been entered into and will be construed and enforced in accordance with the laws of the State of California as applied to contracts made and to be performed entirely within California. Any ambiguity in this Agreement shall not be construed against either party as the drafter. Any waiver of a breach of this Agreement, or

rights hereunder, shall be in writing and shall not be deemed to be a waiver of any successive breach or rights hereunder. This Agreement may be executed in counterparts which shall be deemed to be part of one original, and facsimile signatures and signatures transmitted by PDF shall be equivalent to original signatures. The terms of any payments or benefits to be provided pursuant to this Agreement will be construed to the greatest extent possible so as to be exempt from or compliant with the provisions of Section 409A of the Internal Revenue Code and the regulations promulgated thereunder.

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If this Agreement is acceptable to you, please sign below and return the original to me.

Sincerely,

Retrophin, Inc.

By: /s/ Gary Lyons

Name: Gary Lyons

Title: Chairman of the Board

Accepted and Agreed:

/s/ Stephen Aselage

Stephen Aselage

January 4, 2019

Date

EMPLOYMENT AGREEMENT

THIS EMPLOYMENT AGREEMENT is effective as of the last date signed by the parties hereto (the "Effective Date") and is entered into by and between **Retrophin, Inc.**, a Delaware corporation (hereinafter the "Company"), and Eric Dube, Ph.D. (hereinafter "Executive").

RECITALS

WHEREAS, the Company and Executive wish to set forth in this Agreement the terms and conditions under which Executive will be employed by the Company on and after the Effective Date hereof;

NOW, THEREFORE, the Company and Executive, in consideration of the mutual promises set forth herein, agree as follows:

1.

NATURE OF EMPLOYMENT

1.1. **Effect of Agreement.** This Agreement shall govern the terms of Executive's employment with the Company on and after the Effective Date until it is terminated by either the Company or Executive pursuant to the terms set forth in Article 6. Executive shall report directly to the Board of Directors of the Company.

1.2. **At-Will Employment.** Executive shall continue to be employed on an at-will basis by the Company and therefore either Executive or the Company may terminate the employment relationship and this Agreement at any time, with or without Cause (as defined herein) and with or without advance notice, subject to the provisions of Article 6.

1.3. **Board of Directors.** Executive shall serve on the Board of Directors of the Company for so long as Executive remains the Company's Chief Executive Officer. Upon termination of Executive's employment for any reason, or in the event Executive ceases to remain the Company's Chief Executive Officer for any other reason, Executive shall immediately resign from the Board of Directors of the Company unless otherwise unanimously requested by all the other members of the Board of Directors of the Company.

2.

EMPLOYMENT DUTIES

2.1. **Title/Responsibilities.** Executive agrees to serve the Company in the position of President and Chief Executive Officer. Executive shall have the powers and duties commensurate with such position.

2.2. **Full Time Attention.** Executive shall devote his best efforts and his full business time and attention to the performance of the services customarily incident to such office and to such other services as the Board of Directors may reasonably request.

2.3. **Other Activities.** Except upon the prior written consent of the Board of Directors, Executive shall not during the period of employment engage, directly or indirectly, in any other business activity (whether or not pursued for pecuniary advantage) that is or may be competitive with, or that might place him in a competing position to that of the Company or any other corporation or entity that directly or indirectly controls, is controlled by, or is under common control with the Company, provided that Executive may own less than two percent (2%) of the outstanding securities of any such publicly traded competing corporation.

3.

COMPENSATION

3.1. **Base Salary.** Executive shall receive a Base Salary at an annual rate of \$625,000, payable semi-monthly in equal installments in accordance with the Company's normal payroll practices. The Board of Directors or the Compensation Committee of the Board of Directors (the "Compensation Committee") shall provide Executive with annual performance reviews, and, thereafter, Executive shall be entitled to such increase in Base Salary as the Compensation Committee may from time to time establish in its sole discretion.

3.2. **Incentive Bonus.** In addition to any other bonus Executive shall be awarded by the Compensation Committee, Executive shall be eligible to receive an annual incentive bonus as determined by the Compensation Committee based upon the achievement by the Company of annual corporate goals established by the Board of Directors or the Compensation Committee and Executive's individual performance during the applicable year. Executive's annual incentive bonus at target will be 60% of Executive's Base Salary (the "Target Annual Bonus"). The Compensation Committee in consultation with the independent members of the Board of Directors shall, in its sole discretion, determine whether the annual corporate goals have been attained. Any annual incentive bonus shall be considered earned only if Executive is employed by the Company on the date that the determination is made as to whether annual corporate goals have been met. This determination generally will be made within the first quarter following the end of the Company's fiscal year. Except as provided in Article 6 herein, no pro-rata bonus will be considered earned if Executive leaves the Company for any reason prior to the foregoing determination date. Any annual incentive bonus that is earned shall be paid no later than the fifteenth day of the third month following the end of the Company's fiscal year for which such bonus was earned.

3.3. **Equity.**

(a) Subject to approval by the Board of Directors or Compensation Committee, the Company will grant Executive the following equity awards (collectively, the "Initial Equity Awards"): (i) a stock option to purchase 400,000 shares of Company common stock (the "Option"), (ii) a performance-based restricted stock unit award covering 50,000 shares of Company common stock, and (iii) a time-based restricted stock unit award covering 50,000 shares of Company common stock (the "Time-Based RSU"). The stock option is a non-qualified stock option, has a 10-year term and will vest over four years, with one-fourth vesting on the one-year anniversary of the grant date and the remaining three-fourths vesting over the following three years in 36 equal monthly installments. The performance-based restricted stock unit award will vest upon the Company's achievement of performance based milestones specified in the applicable equity award agreement; provided, however, that no portion of the performance-based restricted stock unit award will vest prior to the one-year anniversary of the grant date, and provided further that the performance-based restricted stock unit award will expire on May 9, 2023 to the extent the specified performance based milestones are not achieved by such date. The time-based restricted stock unit award will vest over four years, with one-fourth vesting on each anniversary of the grant date. The vesting of each Initial Equity Award is subject to Executive's continued employment through the applicable vesting dates, and is subject to accelerated vesting in certain circumstances pursuant to Article 6 below. Each of the Initial Equity Awards is intended to be a material inducement to Executive's acceptance of the Company's offer of employment with the Company, and will be granted outside the Company's 2018 Equity Incentive Plan (the "2018 Plan") but pursuant to the terms of the 2018 Plan as if such awards were granted under the 2018 Plan.

(b) Subject to approval by the Compensation Committee, in consultation with the independent members of the Board of Directors, Executive will be eligible to receive additional Stock Awards on terms to be determined by the Compensation Committee at the time of any such grant. The determination whether to grant any additional Stock Award to Executive is in the sole discretion of the Compensation Committee, in consultation with the independent members of the Board of Directors. For all purposes of this Agreement, "Stock Awards" shall mean any rights granted by the Company to Executive with respect to the common stock of the Company, including, without limitation, the Initial Equity Awards and other stock options, stock appreciation rights, restricted stock, stock bonuses and restricted stock units.

3.4. **Relocation.** Executive's primary office location shall be the Company's office located in San Diego, California, and as a condition of employment, Executive shall relocate to the San Diego area. Executive shall be eligible for reimbursement of certain out-of-pocket expenses incurred as a result of Executive's permanent relocation to the San Diego area in accordance with the Company's Relocation Policy Guidelines (such reimbursed amounts, if any, the "Relocation Payments"). Should Executive's employment terminate within 12 months after the Effective Date pursuant to a Voluntary Resignation (as set forth in Section 6.7 herein), Executive shall be required to repay to the Company 100% of any amounts previously paid to him for the Relocation Payments, and hereby authorizes the Company to withhold any such amount from Executive's final paycheck or other earned compensation. Should Executive's employment terminate after 12 months but within 24 months after the Effective Date pursuant to a Voluntary Resignation (as set forth in Section 6.7 herein), Executive shall be required to repay to the Company 50% of any amounts previously paid to him for the Relocation Payments, and hereby authorizes the Company to withhold any such amount from Executive's final paycheck or other earned compensation.

3.5. **Inducement Advance.** Executive shall receive a one-time cash inducement advance (the "Inducement Advance") in the total amount of \$100,000.00, subject to applicable withholding, which shall be deemed earned when Executive successfully completes two full years of employment from the Effective Date. The Inducement Advance shall be paid within thirty days following the Effective Date. Should Executive's employment terminate within 12 months after the Effective Date pursuant to a Voluntary Resignation (as set forth in Section 6.7 herein), Executive shall be required to repay to the Company 100% of any amounts actually received by him for the Inducement Advance, and hereby authorizes the Company to withhold any such amount from Executive's final paycheck or other earned compensation. Should Executive's employment terminate after 12 months but within 24 months after the Effective Date pursuant to a Voluntary Resignation (as set forth in Section 6.7 herein), Executive shall be required to repay to the Company 50% of any amounts actually received by him for the Inducement Advance, and hereby authorizes the Company to withhold any such amount from Executive's final paycheck or other earned compensation.

3.6. **Withholdings.** All compensation and benefits payable to Executive under this Agreement shall be subject to all federal, state, local taxes and other withholdings and similar taxes and payments required by applicable law.

4.

EXPENSE ALLOWANCES AND FRINGE BENEFITS

4.1. **Vacation.** Executive shall be eligible for all paid holidays recognized by the Company, and 20 days of paid vacation per annum, accrued at the rate of 1.67 days per month. Vacation days for the first calendar year of employment may be prorated. The Company provides for rollover of vacation from year to year up to an allowable maximum. Executive shall also be eligible for personal days and sick days consistent with the Company's policy.

4.2. **Benefits.** During Executive's employment hereunder, the Company shall also provide Executive with the health insurance benefits it generally provides to its other senior management employees. As Executive becomes eligible in accordance with applicable criteria adopted by the Company, the Company shall provide Executive with the right to participate in and to receive benefit from life, accident, disability, medical, and savings plans and similar benefits made available generally to employees of the Company as such plans and benefits may be adopted by the Company. With respect to long-term disability insurance coverage, Executive will pay all premiums for such coverage with after-tax dollars, and the Company will reimburse Executive for the premium costs so paid by Executive, which reimbursement benefit shall be taxable income, subject to withholding. The amount and extent of benefits to which Executive is entitled shall be governed by the specific benefit plan as it may be amended from time to time.

4.3. **Business Expense Reimbursement.** During the term of this Agreement, Executive shall be entitled to receive proper reimbursement for all reasonable out-of-pocket expenses incurred by him (in accordance with the policies and procedures established by the Company for its senior executive officers) in performing services hereunder. Executive agrees to furnish to the Company adequate records and other documentary evidence of such expense for which Executive seeks reimbursement. Such expenses shall be reimbursed and accounted for under the policies and procedures established by the Company, and such reimbursement shall be made promptly, but in no event later than December 31 of the calendar year following the year in which such expenses were incurred by Executive.

5.

CONFIDENTIALITY

5.1. **Confidential Information.** Executive represents and warrants that he has previously executed and delivered to the Company the Company's standard Confidentiality Agreement.

5.2. **Return of Property.** All documents, records, apparatus, equipment and other physical property which is furnished to or obtained by Executive in the course of his employment with the Company shall be and remain the sole property of the Company. Executive agrees that, upon the termination of his employment, he shall return all such property (whether or not it pertains to Confidential Information as defined in the Confidentiality Agreement), and agrees not to make or retain copies, reproductions or summaries of any such property.

5.3. **No Use of Prior Confidential Information.** Executive will not intentionally disclose to the Company or use on its behalf any confidential information belonging to any of his former employers or any other third party.

6.

TERMINATION

6.1. **General.** As set forth in Section 1.2 herein, Executive shall be employed on an at-will basis by the Company. Notwithstanding the foregoing, Executive's employment and this Agreement may be terminated in one of six ways as set forth in this Article 6: (a) Executive's Death (Section 6.2); (b) Executive's Disability (Section 6.3); (c) Termination by the Company for Cause (Section 6.4); (d) Termination by the Company without Cause (Section 6.5); (e) Termination by Executive due to a Constructive Termination (Section 6.6); or (f) Voluntary Resignation (Section 6.7).

6.2. **By Death.** Executive's employment and this Agreement shall terminate automatically upon the death of Executive. In such event:

(a) **Stock Awards.** The vesting of the Option and Time-Based RSU (to the extent then unvested) shall be accelerated (to the extent applicable) so that the amount of shares vested under such Option and Time-Based RSU shall equal 1/48th of the total number of shares subject to the Option and Time-Based RSU, respectively, multiplied by the number of full months that elapsed between the grant date and Executive's termination of employment.

(b) **Bonus.** The Company shall pay to Executive's beneficiaries or his estate, as the case may be, a lump sum amount equal to Executive's Target Annual Bonus (as defined in Section 3.2) for the Company's fiscal year in which Executive's death occurs multiplied by a fraction, the numerator of which is the number of full months of employment by Executive in such fiscal year and the denominator of which is 12. Such amount shall be paid as soon as administratively practicable, but in no event later than March 15 following the year in which Executive's death occurred.

(c) **Accrued Compensation.** The Company shall pay to Executive's beneficiaries or his estate, as the case may be, any accrued Base Salary, any vested deferred compensation (other than pension plan or profit-sharing plan benefits that will be paid in accordance with the applicable plan), any benefits under any plans of the Company (other than pension and profit-sharing plans) in which Executive is a participant to the full extent of Executive's rights under such plans, any accrued vacation pay and any appropriate business expenses incurred by Executive in connection with his duties hereunder, all to the date of termination (collectively "Accrued Compensation").

(d) **No Severance Compensation.** The compensation and benefits set forth in Sections 6.2(a) through (c) herein shall be the only compensation and benefits provided by the Company in the event of Executive's death and no other severance compensation or benefits shall be provided.

6.3. **By Disability.** If Executive is prevented from performing his duties hereunder by reason of any physical or mental incapacity that results in Executive's satisfaction of all requirements necessary to receive benefits under the Company's long-term disability plan due to a total disability, then, to the extent permitted by law, the Company may terminate the employment of Executive and this Agreement at or after such time. In such event, and if Executive signs the General Release set forth as **Exhibit A** or such other form of release as the Company may require (the "Release") on or within the time period set forth therein, but in no event later than forty-five (45) days after the termination date and allows such Release to become effective (the "Release Effective Date"), then:

(a) **Accrued Compensation.** The Company shall pay to Executive all Accrued Compensation (as defined in Section 6.2(c) herein).

(b) **Base Salary Continuation.** The Company shall continue to pay Executive's Base Salary, less required withholdings, for a period of 18 months (the "Disability Base Salary Payments") following Executive's separation from service; provided that the Disability Base Salary Payments shall be reduced by any insurance or other payments to Executive under policies and plans sponsored by the Company, even if premiums are paid by Executive. Subject to the provisions of Section 6.11, the Disability Base Salary Payments shall be paid in accordance with the Company's standard payroll practices; provided, however, that any amounts that would otherwise be scheduled to be paid prior to the Release Effective Date shall instead accrue and be paid during the first payroll period following the Release Effective Date, and all other payments shall be made as originally scheduled.

(c) **Bonus.** The Company shall pay to Executive a lump sum amount equal to Executive's Target Annual Bonus (as defined in Section 3.2) for the Company's then-current fiscal year multiplied by a fraction, the numerator of which is the number of full months of employment by Executive in the current fiscal year and the denominator of which is 12. Such payment shall be made within ten (10) days following the Release Effective Date.

(d) **Stock Awards.** The vesting of all outstanding Stock Awards held by Executive shall be accelerated such that the amount of shares vested under such Stock Awards shall equal that number of shares that would have been vested if Executive had continued to render services to the Company for 18 continuous months after the date of Executive's termination of employment.

(e) **Health Insurance Benefits.** To the extent provided by the federal COBRA law or, if applicable, state insurance laws, and by the Company's current group health insurance policies, Executive will be eligible to continue Executive's group health insurance benefits at Executive's own expense. If Executive timely elects continued coverage under COBRA, the Company shall pay Executive's COBRA premiums, and any applicable Company COBRA premiums, necessary to continue Executive's then-current coverage for a period of 18 months after the date of Executive's termination of employment; *provided, however*, that any such payments will cease if Executive voluntarily enrolls in a health insurance plan offered by another employer or entity during the period in which the Company is paying such premiums. Executive agrees to immediately notify the Company in writing of any such enrollment.

Notwithstanding the foregoing, if the Company determines, in its sole discretion, that it cannot provide the foregoing benefit without potentially incurring financial costs or penalties under applicable law (including, without limitation, Section 2716 of the Public Health Service Act), the Company shall in lieu thereof provide to Executive a taxable monthly amount to continue his group health insurance coverage in effect on the date of separation from service (which amount shall be based on the premium for the first month of COBRA coverage), which payments shall be made regardless of whether Executive elects COBRA continuation coverage and shall commence in the month following the month in which Executive incurs a separation from service and shall end on the earlier of (x) the date on which Executive voluntarily enrolls in a health insurance plan offered by another employer or entity during the period in which the Company is paying such amounts and (y) 18 months after the date of Executive's separation from service.

(f) **Disability Plans.** Nothing in this Section 6.3 shall affect Executive's rights under any disability plan in which Executive is a participant.

6.4. Termination by the Company for Cause.

(a) **No Liability.** The Company may terminate Executive's employment and this Agreement for Cause (as defined below) without liability at any time. In such event, the Company shall pay Executive all Accrued Compensation (as defined in Section 6.2(c) herein), but no other compensation or reimbursement of any kind, including without limitation, any severance compensation or benefits shall be paid, and thereafter the Company's obligations hereunder shall terminate. For clarity, the foregoing sentence shall not have any impact on any awards or other benefits that are vested as of the date of such termination.

(b) **Definition of "Cause."** For purposes of this Agreement, "Cause" shall mean one or more of the following:

(i) Executive's intentional commission of an act, or intentional failure to act, that materially injures the business of the Company; *provided, however,* that in no event shall any business judgment made in good faith by Executive and within Executive's defined scope of authority constitute a basis for termination for Cause under this Agreement;

(ii) Executive's intentional refusal or intentional failure to act in accordance with any lawful and proper direction or order of the Board of Directors;

(iii) Executive's material breach of Executive's fiduciary, statutory, contractual, or common law duties to the Company (including any material breach of this Agreement, the Confidentiality Agreement, or the Company's written policies);

(iv) Executive's conviction of, or plea of guilty or nolo contendere to, any felony or any crime involving dishonesty; or

(v) Executive's participation in any fraud or other act of willful misconduct against the Company;

provided, however, that in the event that any of the foregoing events is reasonably capable of being cured, the Company shall provide written notice to Executive describing the nature of such event and Executive shall thereafter have ten (10) business days to cure such event.

6.5. Termination by the Company without Cause.

(a) **The Company's Right.** The Company may terminate Executive's employment and this Agreement without Cause (as defined in Section 6.4(b) herein) at any time by giving thirty (30) days advance written notice to Executive.

(b) **Severance Benefits.** If the Company terminates Executive's employment without Cause, and if Executive signs the Release on or within the time period set forth therein (but in no event later than forty-five (45) days after the termination date) and allows such Release to become effective, then:

(i) **Accrued Compensation.** The Company shall pay to Executive all Accrued Compensation (as defined in Section 6.2(c) herein).

(ii) **Cash Compensation Amount Payments.** The Company shall pay Executive an amount equal to (A) Executive's annual Base Salary plus Executive's Target Annual Bonus (as defined in Section 3.2 herein) multiplied by (B) 1.5 (the "Cash Compensation Amount"). Subject to the provisions of Section 6.11, the Cash Compensation Amount will be paid in equal installments on the Company's standard payroll dates over a period of 18 months following Executive's separation from service; provided, however, that any amounts that would otherwise be scheduled to be paid prior to the Release Effective Date shall instead accrue and be paid during the first payroll period following the Release Effective Date, and all other payments shall be made as originally scheduled.

(iii) **Stock Awards.** The vesting of all outstanding Stock Awards held by Executive shall be accelerated such that the amount of shares vested under such Stock Awards shall equal that number of shares that would have been vested if Executive had continued to render services to the Company for 18 continuous months after the date of Executive's termination of employment.

(iv) **Health Insurance Benefits.** To the extent provided by the federal COBRA law or, if applicable, state insurance laws, and by the Company's current group health insurance policies, Executive will be eligible to continue Executive's group health insurance benefits at Executive's own expense. If Executive timely elects continued coverage

under COBRA, the Company shall pay Executive's COBRA premiums, and any applicable Company COBRA premiums, necessary to continue Executive's then-current coverage for a period of 18 months after the date of Executive's termination of employment; *provided, however*, that any such payments will cease if Executive voluntarily enrolls in a health insurance plan offered by another employer or entity during the period in which the Company is paying such premiums. Executive agrees to immediately notify the Company in writing of any such enrollment.

Notwithstanding the foregoing, if the Company determines, in its sole discretion, that it cannot provide the foregoing benefit without potentially incurring financial costs or penalties under applicable law (including, without limitation, Section 2716 of the Public Health Service Act), the Company shall in lieu thereof provide to Executive a taxable monthly amount to continue his group health insurance coverage in effect on the date of separation from service (which amount shall be based on the premium for the first month of COBRA coverage), which payments shall be made regardless of whether Executive elects COBRA continuation coverage and shall commence in the month following the month in which Executive incurs a separation from service and shall end on the earlier of (x) the date on which Executive voluntarily enrolls in a health insurance plan offered by another employer or entity during the period in which the Company is paying such amounts and (y) 18 months after the date of Executive's separation from service.

6.6. Termination by Executive due to a Constructive Termination.

(a) **Executive's Right.** Executive may resign his employment and terminate this Agreement at any time as a result of a Constructive Termination (as defined in Section 6.6(c) herein).

(b) **Severance Benefits.** If Executive resigns his employment and terminates this Agreement as a result of a Constructive Termination, and if Executive signs the Release on or within the time period set forth therein (but in no event later than forty-five (45) days after the termination date) and allows such Release to become effective, then Executive shall receive all of the severance benefits set forth in Section 6.5(b) herein.

(c) **Definition of "Constructive Termination."** For purposes of this Agreement, "Constructive Termination" shall mean a resignation of employment and termination of this Agreement by Executive for one or more of the following reasons:

(i) Assignment to, or withdrawal from, Executive of any duties or responsibilities that results in a material diminution in such Executive's authority, duties or responsibilities as in effect immediately prior to such change;

(ii) A material diminution in the authority, duties or responsibilities of the supervisor to whom Executive is required to report, including a requirement that Executive report to a corporate officer or employee instead of reporting directly to the Board of Directors;

(iii) A material reduction by the Company of Executive's annual Base Salary;

(iv) A relocation of Executive or the Company's principal executive offices if Executive's principal office is at such offices, to a location more than forty (40) miles from the location at which Executive is then performing his duties, except for an opportunity to relocate which is accepted by Executive in writing; or

(v) A material breach by the Company of any provision of this Agreement or any other enforceable written agreement between Executive and the Company;

provided however, that Executive must first provide the Company with written notice specifying the condition giving rise to a Constructive Termination within ninety (90) days following the initial existence of such condition; and Executive's notice must specify that Executive intends to terminate his employment no earlier than thirty (30) days after providing such notice, and the Company must be given an opportunity to cure such condition within thirty (30) days following its receipt of such notice and avoid paying benefits.

6.7. **Voluntary Resignation.** Executive may resign his employment and terminate this Agreement at any time for any reason other than due to a Constructive Termination (as defined in Section 6.6(c) herein). In such event the Company shall pay Executive all Accrued Compensation (as defined in Section 6.2(c) herein), but no other compensation or reimbursement of any kind, including without limitation, any severance compensation or benefits shall be paid, and thereafter the Company's obligations hereunder shall terminate.

6.8. Change in Control.

(a) **Severance Benefits.** If (i) within three (3) months prior to, or on or within twelve (12) months after, the consummation of a Change in Control (as defined in Section 6.8(b) herein), (1) the Company terminates Executive's employment and this Agreement without Cause pursuant to Section 6.5 herein or (2) Executive resigns his employment and terminates this Agreement as a result of a Constructive Termination pursuant to Section 6.6 herein, and (ii) in either event (1) or (2), Executive signs the Release on or within the time period set forth therein, but in no event later than forty-

five (45) days after the termination date and allows such Release to become effective, then Executive shall receive the following severance benefits in lieu of any severance benefits set forth in Section 6.5(b) or Section 6.6(b) herein:

(i) **Accrued Compensation.** The Company shall pay to Executive all Accrued Compensation (as defined in Section 6.2(c) herein).

(ii) **CIC Cash Compensation Amount Payment.** The Company shall pay Executive an amount equal to (A) Executive's annual Base Salary plus Executive's Target Annual Bonus (as defined in Section 3.2 herein) multiplied by (B) 2.0 (collectively, the "CIC Cash Compensation Amount"). The CIC Cash Compensation Amount will be paid in one lump sum within ten (10) days following the Release Effective Date.

(iii) **Stock Awards.** The vesting of all outstanding Stock Awards held by Executive shall be accelerated in full, effective as of the Release Effective Date.

(iv) **Health Insurance Benefits.** To the extent provided by the federal COBRA law or, if applicable, state insurance laws, and by the Company's current group health insurance policies, Executive will be eligible to continue Executive's group health insurance benefits at Executive's own expense. If Executive timely elects continued coverage under COBRA, the Company shall pay Executive's COBRA premiums, and any applicable Company COBRA premiums, necessary to continue Executive's then-current coverage for a period of 24 months after the date of Executive's termination of employment; *provided, however*, that any such payments will cease if Executive voluntarily enrolls in a health insurance plan offered by another employer or entity during the period in which the Company is paying such premiums. Executive agrees to immediately notify the Company in writing of any such enrollment.

Notwithstanding the foregoing, if the Company determines, in its sole discretion, that it cannot provide the foregoing benefit without potentially incurring financial costs or penalties under applicable law (including, without limitation, Section 2716 of the Public Health Service Act), the Company shall in lieu thereof provide to Executive a taxable monthly amount to continue his group health insurance coverage in effect on the date of separation from service (which amount shall be based on the premium for the first month of COBRA coverage), which payments shall be made regardless of whether Executive elects COBRA continuation coverage and shall commence in the month following the month in which Executive incurs a separation from service and shall end on the earlier of (x) the date on which Executive voluntarily enrolls in a health insurance plan offered by another employer or entity during the period in which the Company is paying such amounts and (y) 24 months after the date of Executive's separation from service.

(b) For purposes of this Agreement, a "Change in Control" shall have occurred if at any time following the Effective Date, any of the following events shall occur:

(i) The Company is merged, or consolidated, or reorganized into or with another corporation or other legal person, and as a result of such merger, consolidation or reorganization less than 50% of the combined voting power of the then-outstanding securities of such corporation or person immediately after such transaction are held in the aggregate by the holders of voting securities of the Company immediately prior to such transaction;

(ii) The Company sells all or substantially all of its assets or any other corporation or other legal person and thereafter, less than 50% of the combined voting power of the then-outstanding voting securities of the acquiring or consolidated entity are held in the aggregate by the holders of voting securities of the Company immediately prior to such sale;

(iii) There is a report filed after the date of this Agreement on Schedule 13D or Schedule 14D-1 (or any successor schedule, form or report), each as promulgated pursuant to the Securities Exchange Act of 1934 (the "Exchange Act") disclosing that any person (as the term "person" is used in Section 13(d)(3) or Section 14(d)(2) of the Exchange Act) has become the beneficial owner (as the term beneficial owner is defined under Rule 13d-3 or any successor rule or regulation promulgated under the Exchange Act) representing 50% or more of the combined voting power of the then-outstanding voting securities of the Company; or

(iv) During any period of two (2) consecutive years following the Effective Date, individuals who at the beginning of any such period constitute the directors of the Company cease for any reason to constitute at least a majority thereof unless the election to the nomination for election by the Company's shareholders of each director of the Company first elected during such period was approved by a vote of at least two-thirds of the directors of the Company then still in office who were directors of the Company at the beginning of such period.

6.9. **Mitigation.** Except as otherwise specifically provided herein, Executive shall not be required to mitigate the amount of any payment provided under this Agreement by seeking other employment or self-employment, nor shall the amount of any payment provided for under this Agreement be reduced by any compensation earned by Executive as a result of employment by another employer or through self-employment or by retirement benefits after the date of Executive's termination of employment from the Company, except as provided herein.

6.10. **Coordination.** If upon termination of employment, Executive becomes entitled to rights under other plans, contracts or arrangements entered into by the Company, this Agreement shall be coordinated with such other arrangements so that Executive's rights under this Agreement are not reduced, and that any payments under this Agreement offset the same types of payments otherwise provided under such other arrangements, but do not otherwise reduce any payments or benefits under such other arrangements to which Executive becomes entitled.

6.11. **Application of Section 409A.** Notwithstanding anything to the contrary herein, the following provisions apply to the extent severance benefits provided herein are subject to Section 409A of the Code and the regulations and other guidance thereunder and any state law of similar effect (collectively "Section 409A"). Severance benefits shall not commence until Executive has a "separation from service" for purposes of Section 409A. If Executive is a "specified employee" within the meaning of 409A(a)(2)(B)(i) of the Code, any installment payments of Disability Base Salary Payments pursuant to Section 6.3(b) or Cash Compensation Amounts pursuant to Section 6.5(b) or 6.6(b) that are triggered by a separation from service shall be accelerated to the minimum extent necessary so that (a) the lesser of (y) the total cash severance payment amount, or (z) six (6) months of such installment payments are paid no later than March 15 of the calendar year following such termination, and (b) all amounts paid pursuant to the foregoing clause (a) will constitute separate payments for purposes of Section 1.409A-2(b)(2) of the Treasury Regulations and thus will be payable pursuant to the "short-term deferral" rule set forth in Section 1.409A-1(b)(4) of the Treasury Regulations. It is intended that if Executive is a "specified employee" within the meaning of Section 409A(a)(2)(B)(i) of the Code at the time of such separation from service the foregoing provision shall result in compliance with the requirements of Section 409A(a)(2)(B)(i) of the Code because payments to Executive will either be payable pursuant to the "short-term deferral" rule set forth in Section 1.409A-1(b)(4) of the Treasury Regulations or will not be paid until at least 6 months after separation from service. The severance benefits are intended to qualify for an exemption from application of Section 409A or comply with its requirements to the extent necessary to avoid adverse personal tax consequences under Section 409A, and any ambiguities herein shall be interpreted accordingly.

6.12. **Parachute Payments.**

(a) If any payment or benefit (including payments or benefits pursuant to this Agreement) that Executive would receive in connection with a Change in Control or otherwise ("Payment") would (1) constitute a "parachute payment" within the meaning of Section 280G of the Code, and (2) but for this sentence, be subject to the excise tax imposed by Section 4999 of the Code (the "Excise Tax"), then such Payment shall be equal to the Reduced Amount. The "Reduced Amount" shall be either (x) the largest portion of the Payment that would result in no portion of the Payment being subject to the Excise Tax or (y) the largest portion, up to and including the total, of the Payment, whichever amount, after taking into account all applicable federal, state and local employment taxes, income taxes, and the Excise Tax (all computed at the highest applicable marginal rate), results in Executive's receipt, on an after-tax basis, of the greater economic benefit notwithstanding that all or some portion of the Payment may be subject to the Excise Tax. If a reduction in payments or benefits constituting "parachute payments" is necessary so that the Payment equals the Reduced Amount, Executive shall have no rights to any additional payments and/or benefits, and reduction shall occur in the manner that results in the greatest economic benefit for Executive. If more than one method of reduction will result in the same economic benefit, the items so reduced will be reduced pro rata.

(b) In the event it is subsequently determined by the Internal Revenue Service that some portion of the Reduced Amount as determined pursuant to clause (x) in the preceding paragraph is subject to the Excise Tax, Executive agrees to promptly return to the Company a sufficient amount of the Payment so that no portion of the Reduced Amount is subject to the Excise Tax. For the avoidance of doubt, if the Reduced Amount is determined pursuant to clause (y) in the preceding paragraph, Executive will have no obligation to return any portion of the Payment pursuant to the preceding sentence.

(c) The independent registered public accounting firm engaged by the Company for general audit purposes as of the day prior to the effective date of the event described in Section 280G(b)(2)(A)(i) of the Code will perform the foregoing calculations. If the independent registered public accounting firm so engaged by the Company is serving as accountant or auditor for the individual, entity or group effecting such Change in Control or similar transaction, the Company will appoint a nationally recognized independent registered public accounting firm to make the determinations required hereunder. The Company will bear all expenses with respect to the determinations by such independent registered public accounting firm required to be made hereunder. Any good faith determinations of the independent registered public accounting firm made hereunder will be final, binding and conclusive upon the Company and you.

7.

GENERAL PROVISIONS

7.1. **Governing Law.** The validity, interpretation, construction and performance of this Agreement and the rights of the parties thereunder shall be interpreted and enforced under California law without reference to principles of conflicts of laws.

7.2. Assignment; Successors; Binding Agreement.

(a) **No Assignment.** Executive may not assign, pledge or encumber his interest in this Agreement or any part thereof.

(b) **Assumption by Successor.** The Company will require any successor (whether direct or indirect, by purchase, merger, consolidation or otherwise) to all or substantially all of the business and/or assets of the Company, by operation of law or by agreement in form and substance reasonably satisfactory to Executive, to assume and agree to perform this Agreement in the same manner and to the same extent that the Company would be required to perform it if no such succession had taken place.

(c) **Binding Agreement.** This Agreement shall inure to the benefit of and be enforceable by Executive's personal or legal representatives, executors, administrators, successors, heirs, distributee, devisees and legatees. If Executive should die while any amount is at such time payable to Executive hereunder, all such amounts, unless otherwise provided herein, shall be paid in accordance with the terms of this Agreement to Executive's devisee, legates or other designee or, if there be no such designee, to his estate.

7.3. **Notice.** For the purposes of this Agreement, notices and all other communications provided for in this Agreement shall be in writing and shall be deemed to have been duly given when delivered or mailed by certified or registered mail, return receipt requested, postage prepaid, addressed to the respective addresses set forth below or to such other address as either party may have furnished to the other in writing in accordance herewith, except that notice of change of address shall be effective only upon receipt.

To the Company:

Retrophin, Inc.

3721 Valley Centre Drive, Suite 200

San Diego, CA 92130

To Executive:

7.4. **Modification; Waiver; Entire Agreement.** This Agreement constitutes the complete, final and exclusive embodiment of the entire agreement between Executive and the Company with regard to this subject matter. It is entered into without reliance on any promise or representation, written or oral, other than those expressly contained herein, and it supersedes any other such promises, warranties or representations. No provisions of this Agreement may be modified, waived or discharged unless such waiver, modification or discharge is agreed to in writing signed by Executive and such officer as may be specifically designated by the Board of Directors of the Company. No waiver by either party hereto at any time of any breach by the other party of, or compliance with, any condition or provision of this Agreement to be performed by such other party shall be deemed a waiver of similar or dissimilar provisions or conditions at the same or any prior or subsequent time.

7.5. **Validity.** The invalidity or unenforceability of any provision of this Agreement shall not affect the validity or enforceability of any other provision of this Agreement, which shall remain in full force and effect.

7.6. **Controlling Document.** Except to the extent described in Section 6.10, in case of conflict between any of the terms and conditions of this Agreement and any document herein referred to, the terms and conditions of this Agreement shall control.

7.7. **Executive Acknowledgment.** Executive acknowledges (a) that he has consulted with or has had the opportunity to consult with independent counsel of his own choice concerning this Agreement, and has been advised to do so by the Company, and (b) that he has read and understands the Agreement, is fully aware of its legal effect, and has entered into it freely based on his own judgment.

7.8. **Dispute Resolution.** To ensure the rapid and economical resolution of disputes that may arise in connection with Executive's employment, Executive and the Company agree that any and all disputes, claims, or causes of action,

in law or equity, arising from or relating to the enforcement, breach, performance, execution, or interpretation of this Agreement, Executive's employment, or the termination of that employment, shall be resolved, to the fullest extent permitted by law pursuant to the Federal Arbitration Act, 9 U.S.C. §1-16, by final, binding and confidential arbitration in San Diego, California conducted before a single arbitrator by Judicial Arbitration and Mediation Services, Inc. ("JAMS") or its successor, under the then applicable JAMS rules; *provided, however*, that in no event shall the Arbitrator be empowered to hear or determine any class or collective claim of any type. The JAMS rules can be found online at www.jamsadr.com. By agreeing to this arbitration procedure, both Executive and the Company waive the right to resolve any such dispute through a trial by jury or judge or by administrative proceeding. The arbitrator shall: (a) have the authority to compel adequate discovery for the resolution of the dispute and to award such relief as would otherwise be permitted by law; and (b) issue a written arbitration decision including the arbitrator's essential findings and conclusions and a statement of the award. The Company shall pay all of JAMS' arbitration fees. Nothing in this letter agreement shall prevent either Executive or the Company from obtaining injunctive relief in court if necessary to prevent irreparable harm pending the conclusion of any arbitration. The parties agree that the arbitrator shall award reasonable attorneys' fees, costs, and all other related expenses to the prevailing party in any action brought hereunder, and the arbitrator shall have discretion to determine the prevailing party in an arbitration where multiple claims may be at issue.

7.9. Remedies.

(a) **Injunctive Relief.** The parties agree that the services to be rendered by Executive hereunder are of a unique nature and that in the event of any breach or threatened breach of any of the covenants contained herein, the damage or imminent damage to the value and the goodwill of the Company's business will be irreparable and extremely difficult to estimate, making any remedy at law or in damages inadequate. Accordingly, the parties agree that the Company shall be entitled to injunctive relief against Executive in the event of any breach or threatened breach of any such provisions by Executive, in addition to any other relief (including damage) available to the Company under this Agreement or under law.

(b) **Exclusive.** Both parties agree that the remedy specified in Section 7.9(a) above is not exclusive of any other remedy for the breach by Executive of the terms hereof.

7.10. **Counterparts.** This Agreement may be executed in one or more counterparts, all of which taken together shall constitute one and the same Agreement.

Executed by the parties as follows:

EXECUTIVE

RETROPHIN, INC.

By: /s/ Eric Dube, PhD

By: /s/ Gary Lyons

Date: January 4, 2019

Date: January 4, 2019

EXHIBIT A
GENERAL RELEASE
[To be signed on or after employment termination date]

Pursuant to the terms of the Employment Agreement between Retrophin, Inc. (the "Company") and _____ ("Executive") dated January __, 2019 (the "Agreement"), the parties hereby enter into the following General Release (the "Release"):

1. **Accrued Salary and Vacation.** Executive understands that, on the last date of Executive's employment with the Company, the Company will pay Executive any accrued salary and accrued and unused vacation to which Executive is entitled by law, regardless of whether Executive signs this Release.

2. **General Release.** Executive hereby generally and completely releases the Company and its directors, officers, employees, shareholders, partners, agents, attorneys, predecessors, successors, parent and subsidiary entities, insurers, affiliates, and assigns (collectively the "Released Parties") of and from any and all claims, liabilities and obligations, both known and unknown, arising out of or in any way related to events, acts, conduct, or omissions occurring at any time prior to or at the time that Executive signs this Release.

3. **Scope of Release.** This general release includes, but is not limited to: (1) all claims arising out of or in any way related to Executive's employment with the Company or the termination of that employment; (2) all claims related to Executive's compensation or benefits from the Company, including salary, bonuses, commissions, vacation pay, expense reimbursements, severance pay, fringe benefits, stock, stock options, or any other ownership or equity interests in the Company; (3) all claims for breach of contract, wrongful termination, and breach of the implied covenant of good faith and fair dealing (including claims based on or arising under the Agreement); (4) all tort claims, including claims for fraud, defamation, emotional distress, and discharge in violation of public policy; and (5) all federal, state, and local statutory claims, including claims for discrimination, harassment, retaliation, attorneys' fees, or other claims arising under the federal Civil Rights Act of 1964 (as amended), the federal Americans with Disabilities Act of 1990, the federal Age Discrimination in Employment Act (as amended) ("ADEA"), the federal Family and Medical Leave Act, the California Labor Code (as amended), the California Family Rights Act, and the California Fair Employment and Housing Act (as amended).

4. **ADEA Waiver.** Executive acknowledges that Executive is knowingly and voluntarily waiving and releasing any rights Executive may have under the ADEA, and that the consideration given for the waiver and release in the preceding paragraph is in addition to anything of value to which Executive is already entitled. If Executive is age 40 or older upon execution of this Release, Executive further acknowledges that Executive has been advised by this writing that, (1) Executive's waiver and release do not apply to any rights or claims that may arise after the date Executive signs this Release; (2) Executive should consult with an attorney prior to signing this Release (although Executive may choose voluntarily not to do so); (3) Executive has twenty-one (21) days to consider this Release (although Executive may choose voluntarily to sign it earlier); (4) Executive has seven (7) days following the date Executive signs this Release to revoke it by providing written notice of revocation to the Company's General Counsel; and (5) this Release will not be effective until the date upon which the revocation period has expired, which will be the eighth calendar day after the date Executive signs it provided that Executive does not revoke it. If Executive is under 40 years of age upon execution of this Release, the Release will be effective upon signing and not revocable.

5. **Waiver of Unknown Claims.** EXECUTIVE UNDERSTANDS THAT THIS AGREEMENT INCLUDES A RELEASE OF ALL KNOWN AND UNKNOWN CLAIMS. Executive acknowledges that Executive has read and understands Section 1542 of the California Civil Code which reads as follows: "A general release does not extend to claims which the creditor does not know or suspect to exist in his or her favor at the time of executing the release, which if known by him or her must have materially affected his or her settlement with the debtor." Executive hereby expressly waives and relinquishes all rights and benefits under that section and any law or legal principle of similar effect in any jurisdiction with respect to Executive's respective release of claims herein, including but not limited to Executive's release of unknown and unsuspected claims.

6. **Excluded Claims.** Executive understands that notwithstanding the foregoing, the following are not included in the Released Claims (the "Excluded Claims"): (i) any rights or claims for indemnification Executive may have pursuant to any written indemnification agreement to which he is a party, the charter, bylaws, or operating agreements of any of the Released Parties, or under applicable law; or (ii) any rights which are not waivable as a matter of law. In addition, Executive understands that nothing in this release prevents Executive from filing, cooperating with, or participating in any proceeding before the Equal Employment Opportunity Commission, the Department of Labor, or any similar government agency, except that Executive acknowledges and agrees that Executive shall not recover any monetary benefits in connection with any such claim, charge or proceeding with regard to any claim released herein. Executive hereby represents and warrants that, other than the Excluded Claims, Executive is not aware of any claims he has or might have against any of the Released Parties that are not included in the Released Claims.

7 . **Executive Representations.** Executive hereby represents that Executive has been paid all compensation owed and for all hours worked; Executive has received all the leave and leave benefits and protections for which Executive is eligible, pursuant to the Family and Medical Leave Act, the California Family Rights Act, or otherwise; and Executive has not suffered any on-the-job injury for which Executive has not already filed a workers' compensation claim.

8 . **Nondisparagement.** Executive agrees not to disparage the Company, its parent, or its or their officers, directors, employees, shareholders, affiliates and agents, in any manner likely to be harmful to its or their business, business reputation, or personal reputation (although Executive may respond accurately and fully to any question, inquiry or request for information as required by legal process).

9 . **Cooperation.** Executive agrees not to voluntarily (except in response to legal compulsion) assist any third party in bringing or pursuing any proposed or pending litigation, arbitration, administrative claim or other formal proceeding against the other party, or against the Company's parent or subsidiary entities, affiliates, officers, directors, employees or agents. Executive further agrees to reasonably cooperate with the other party, by voluntarily (without legal compulsion) providing accurate and complete information, in connection with such other party's actual or contemplated defense, prosecution, or investigation of any claims or demands by or against third parties, or other matters, arising from events, acts, or failures to act that occurred during the period of Executive's employment by the Company.

10. **No Admission of Liability.** The parties agree that this Release, and performance of the acts required by it, does not constitute an admission of liability, culpability, negligence or wrongdoing on the part of anyone, and will not be construed for any purpose as an admission of liability, culpability, negligence or wrongdoing by any party and/or by any party's current, former or future parents, subsidiaries, related entities, predecessors, successors, officers, directors, shareholders, agents, employees and assigns. The parties specifically acknowledge and agree that this Release is a compromise of disputed claims and that the Company denies any liability for any matter released herein.

EXECUTIVE

RETROPHIN, INC.

By: _____

By: _____

Date: _____

Date: _____

EMPLOYMENT AGREEMENT

THIS EMPLOYMENT AGREEMENT is effective as of the last date signed by the parties hereto (the "Effective Date") and is entered into by and between **Retrophin, Inc.**, a Delaware corporation (hereinafter the "Company"), and **Noah Rosenberg, M.D.** (hereinafter "Executive").

RECITALS

WHEREAS, The Company and Executive wish to set forth in this Agreement the terms and conditions under which Executive will be employed by the Company on and after the Effective Date hereof;

NOW, THEREFORE, the Company and Executive, in consideration of the mutual promises set forth herein, agree as follows:

Article 1

NATURE OF EMPLOYMENT

1.1 Effect of Agreement. This Agreement shall govern the terms of Executive's employment with the Company on and after the Effective Date until it is terminated by either the Company or Executive pursuant to the terms set forth in Article 6. Executive shall report directly to the Chief Executive Officer of the Company (the "CEO").

1.2 At-Will Employment. Executive shall continue to be employed on an at-will basis by the Company and therefore either Executive or the Company may terminate the employment relationship and this Agreement at any time, with or without Cause (as defined herein) and with or without advance notice, subject to the provisions of Article 6.

Article 2

EMPLOYMENT DUTIES

2.1 Title/Responsibilities. Executive agrees to serve the Company in the position of Chief Medical Officer. Executive shall have the powers and duties commensurate with such position.

2.2 Full Time Attention. Executive shall devote his best efforts and his full business time and attention to the performance of the services customarily incident to such office and to such other services as the CEO or Board of Directors may reasonably request.

2.3 Other Activities. Except upon the prior written consent of the CEO, Executive shall not during the period of employment engage, directly or indirectly, in any other business activity (whether or not pursued for pecuniary advantage) that is or may be competitive with, or that might place him in a competing position to that of the Company or any other corporation or entity that directly or indirectly controls, is controlled by, or is under common control with the Company, provided that Executive may own less than two percent (2%) of the outstanding securities of any such publicly traded competing corporation.

Article 3

COMPENSATION

3.1 Base Salary. Executive shall receive a Base Salary at an annual rate of \$425,000, payable semi-monthly in equal installments in accordance with the Company's normal payroll practices. The CEO shall provide Executive with annual performance reviews, and, thereafter, Executive shall be entitled to such increase in Base Salary as the Compensation Committee of the Board of Directors (the "Compensation Committee") may from time to time establish in its sole discretion.

3.2 Incentive Bonus. In addition to any other bonus Executive shall be awarded by the Compensation Committee, Executive shall be eligible to receive an annual incentive bonus as determined by the Compensation Committee and CEO based upon the achievement by the Company of annual corporate goals established by the Board of Directors or the Compensation Committee and Executive's individual performance during the applicable year. Executive's annual incentive bonus at target will be 50% of Executive's Base Salary (the "Target Annual Bonus"). The Compensation Committee in consultation with the independent members of the Board of Directors shall, in its sole discretion, determine whether the annual corporate goals have been attained. Any annual incentive bonus shall be considered earned only if Executive is employed by the Company on the date that the determination is made as to whether annual corporate goals have been met. This determination generally will be made within the first quarter following the end of the Company's fiscal year. Except as provided in Article 6 herein, no pro-rata bonus will be considered earned if Executive leaves the Company for any reason prior to the foregoing determination date. Any annual incentive bonus that is earned shall be paid no later than the fifteenth day of the third month following the end of the Company's fiscal year for which such bonus was earned.

3.3 Equity.

(a) Subject to approval by the Board of Directors or Compensation Committee, the Company will grant Executive the following equity awards (collectively, the "Initial Equity Awards"): (i) a stock option to purchase 65,000 shares of Company common stock, (ii) a performance-based restricted stock unit award covering 10,000 shares of Company common stock, and (iii) a time-based restricted stock unit award covering 10,000 shares of Company common stock. The stock option is a non-qualified stock option, has a 10-year term and will vest over four years, with one-fourth vesting on the one-year anniversary of the grant date and the remaining three-fourths vesting over the following three years in 36 equal monthly installments. The performance-based restricted stock unit award will vest upon the Company's achievement of regulatory and clinical development milestones specified in the applicable equity award agreement; provided, however, that no portion of the performance-based restricted stock unit award will vest prior to the one-year anniversary of the grant date, and provided further that the grant will expire on May 10, 2022 to the extent the specified clinical and regulatory milestones are not achieved by such date. The time-based restricted stock unit award will vest over four years, with one-fourth vesting on each anniversary of the grant date. The vesting of each Initial Equity Award is subject to Executive's continued employment through the applicable vesting dates, and is subject to accelerated vesting in certain circumstances pursuant to Article 6 below. Each of the Initial Equity Awards is intended to be a material inducement to Executive's acceptance of the Company's offer of employment with the Company, and will be granted outside the Company's 2018 Equity Incentive Plan (the "2018 Plan") but pursuant to the terms of the 2018 Plan as if such awards were granted under the 2018 Plan.

(b) Subject to approval by the Compensation Committee, in consultation with the independent members of the Board of Directors, Executive will be eligible to receive additional Stock Awards on terms to be determined by the Compensation Committee at the time of any such grant. The determination whether to grant any additional Stock Award to Executive is in the sole discretion of the Compensation Committee, in consultation with the independent members of the Board of Directors. For all purposes of this Agreement, "Stock Awards" shall mean any rights granted by the Company to Executive with respect to the common stock of the Company, including, without limitation, the Initial Equity Awards and other stock options, stock appreciation rights, restricted stock, stock bonuses and restricted stock units.

3.4 Inducement Advance. Executive shall receive a one-time cash inducement advance (the "Inducement Advance") in the total amount of \$100,000.00, subject to applicable withholding, which shall be deemed earned when Executive successfully completes two full years of employment from the Effective Date. The Inducement Advance shall be paid within thirty days following the Effective Date. Should Executive's employment terminate within 12 months after the Effective Date either pursuant to a Voluntary Resignation (as set forth in Section 6.7 herein) or pursuant to a Termination by the Company for Cause (as set forth in Section 6.4 herein), Executive shall be required to repay to the Company 100% of any amounts previously paid to him for the Inducement Advance, and hereby authorizes the Company to withhold any such amount from Executive's final paycheck or other earned compensation. Should Executive's employment terminate between 13 to 24 months after the Effective Date either pursuant to a Voluntary Resignation (as set forth in Section 6.7 herein) or pursuant to a Termination by the Company for Cause (as set forth in Section 6.4 herein), Executive shall be required to repay to the Company 50% of any amounts previously paid to him for the Inducement Advance, and hereby authorizes the Company to withhold any such amount from Executive's final paycheck or other earned compensation. Should Executive's employment terminate at any time pursuant to Sections 6.2, 6.3, 6.5, and 6.6 as set forth herein, Executive shall not be required to repay any portion of the Inducement Advance previously paid to him.

3.5 Relocation. Executive's primary office location shall be the Company's office located in San Diego, California, and as a condition of employment, Executive shall relocate to the San Diego area. Executive shall be eligible for reimbursement of certain out-of-pocket expenses incurred as a result of Executive's permanent relocation to the San Diego area in accordance with the Company's Relocation Policy Guidelines (such reimbursed amounts, if any, the "Relocation Payments"). Should Executive's employment terminate within 12 months after the Effective Date either pursuant to a Voluntary Resignation (as set forth in Section 6.7 herein) or pursuant to a Termination by the Company for Cause (as set forth in Section 6.4 herein), Executive shall be required to repay to the Company 100% of any amounts previously paid to him for the Relocation Payments, and hereby authorizes the Company to withhold any such amount from Executive's final paycheck or other earned compensation. Should Executive's employment terminate between 13 to 24 months after the Effective Date either pursuant to a Voluntary Resignation (as set forth in Section 6.7 herein) or pursuant to a Termination by the Company for Cause (as set forth in Section 6.4 herein), Executive shall be required to repay to the Company 50% of any amounts previously paid to him for the Relocation Payments, and hereby authorizes the Company to withhold any such amount from Executive's final paycheck or other earned compensation. Should Executive's employment terminate at any time pursuant to Sections 6.2, 6.3, 6.5, and 6.6 as set forth herein, Executive shall not be required to repay any portion of the Relocation Payments previously paid to him.

3.6 Withholdings. All compensation and benefits payable to Executive under this Agreement shall be subject to all federal, state, local taxes and other withholdings and similar taxes and payments required by applicable law.

Article 4

EXPENSE ALLOWANCES AND FRINGE BENEFITS

4.1 Vacation. Executive shall be eligible for all paid holidays recognized by the Company, and 20 days of paid vacation per annum, accrued at the rate of 1.67 days per month. Vacation days for the first calendar year of employment may be prorated. The Company provides for rollover of vacation from year to year up to an allowable maximum. Executive shall also be eligible for personal days and sick days consistent with the Company's policy.

4.2 Benefits. During Executive's employment hereunder, the Company shall also provide Executive with the health insurance benefits it generally provides to its other senior management employees. As Executive becomes eligible in accordance with applicable criteria adopted by the Company, the Company shall provide Executive with the right to participate in and to receive benefit from life, accident, disability, medical, and savings plans and similar benefits made available generally to employees of the Company as such plans and benefits may be adopted by the Company. With respect to long-term disability insurance coverage, Executive will pay all premiums for such coverage with after-tax dollars, and the Company will reimburse Executive for the premium costs so paid by Executive, which reimbursement benefit shall be taxable income, subject to withholding. The amount and extent of benefits to which Executive is entitled shall be governed by the specific benefit plan as it may be amended from time to time.

4.3 Business Expense Reimbursement. During the term of this Agreement, Executive shall be entitled to receive proper reimbursement for all reasonable out-of-pocket expenses incurred by him (in accordance with the policies and procedures established by the Company for its senior executive officers) in performing services hereunder. Executive agrees to furnish to the Company adequate records and other documentary evidence of such expense for which Executive seeks reimbursement. Such expenses shall be reimbursed and accounted for under the policies and procedures established by the Company, and such reimbursement shall be made promptly, but in no event later than December 31 of the calendar year following the year in which such expenses were incurred by Executive.

Article 5

CONFIDENTIALITY

5.1 Confidential Information. Executive represents and warrants that he has previously executed and delivered to the Company the Company's standard Confidentiality Agreement.

5.2 Return of Property. All documents, records, apparatus, equipment and other physical property which is furnished to or obtained by Executive in the course of his employment with the Company shall be and remain the sole property of the Company. Executive agrees that, upon the termination of his employment, he shall return all such

property (whether or not it pertains to Confidential Information as defined in the Confidentiality Agreement), and agrees not to make or retain copies, reproductions or summaries of any such property.

5.3 No Use of Prior Confidential Information. Executive will not intentionally disclose to the Company or use on its behalf any confidential information belonging to any of his former employers or any other third party.

Article 6

TERMINATION

6.1 General. As set forth in Section 1.2 herein, Executive shall be employed on an at-will basis by the Company. Notwithstanding the foregoing, Executive's employment and this Agreement may be terminated in one of six ways as set forth in this Article 6: (a) Executive's Death (Section 6.2); (b) Executive's Disability (Section 6.3); (c) Termination by the Company for Cause (Section 6.4); (d) Termination by the Company without Cause (Section 6.5); (e) Termination by Executive due to a Constructive Termination (Section 6.6); or (f) Voluntary Resignation (Section 6.7).

6.2 By Death. Executive's employment and this Agreement shall terminate automatically upon the death of Executive. In such event:

(a) **Bonus.** The Company shall pay to Executive's beneficiaries or his estate, as the case may be, a lump sum amount equal to Executive's Target Annual Bonus (as defined in Section 3.2) for the Company's fiscal year in which Executive's death occurs multiplied by a fraction, the numerator of which is the number of full months of employment by Executive in such fiscal year and the denominator of which is 12. Such amount shall be paid as soon as administratively practicable, but in no event later than March 15 following the year in which Executive's death occurred.

(b) **Accrued Compensation.** The Company shall pay to Executive's beneficiaries or his estate, as the case may be, any accrued Base Salary, any vested deferred compensation (other than pension plan or profit-sharing plan benefits that will be paid in accordance with the applicable plan), any benefits under any plans of the Company (other than pension and profit-sharing plans) in which Executive is a participant to the full extent of Executive's rights under such plans, any accrued vacation pay and any appropriate business expenses incurred by Executive in connection with his duties hereunder, all to the date of termination (collectively "Accrued Compensation").

(c) **No Severance Compensation.** The compensation and benefits set forth in Sections 6.2(a) and (b) herein shall be the only compensation and benefits provided by the Company in the event of Executive's death and no other severance compensation or benefits shall be provided.

6.3 By Disability. If Executive is prevented from performing his duties hereunder by reason of any physical or mental incapacity that results in Executive's satisfaction of all requirements necessary to receive benefits under the Company's long-term disability plan due to a total disability, then, to the extent permitted by law, the Company may terminate the employment of Executive and this Agreement at or after such time. In such event, and if Executive signs the General Release set forth as **Exhibit A** or such other form of release as the Company may require (the "Release") on or within the time period set forth therein, but in no event later than forty-five (45) days after the termination date and allows such Release to become effective (the "Release Effective Date"), then:

(a) **Accrued Compensation.** The Company shall pay to Executive all Accrued Compensation (as defined in Section 6.2(b) herein).

(b) **Base Salary Continuation.** The Company shall continue to pay Executive's Base Salary, less required withholdings, for a period of 12 months (the "Disability Base Salary Payments") following Executive's separation from service; provided that the Disability Base Salary Payments shall be reduced by any insurance or other payments to Executive under policies and plans sponsored by the Company, even if premiums are paid by Executive. Subject to the provisions of Section 6.11, the Disability Base Salary Payments shall be paid in accordance with the Company's standard payroll practices; provided, however, that any amounts that would otherwise be scheduled to be paid prior to the Release Effective Date shall instead accrue and be paid during the first payroll period following the Release Effective Date, and all other payments shall be made as originally scheduled.

(c) **Bonus.** The Company shall pay to Executive a lump sum amount equal to Executive's Target

Annual Bonus (as defined in Section 3.2) for the Company's then-current fiscal year multiplied by a fraction, the numerator of which is the number of full months of employment by Executive in the current fiscal year and the denominator of which is 12. Such payment shall be made within ten (10) days following the Release Effective Date.

(d) **Stock Awards.** The vesting of all outstanding Stock Awards held by Executive shall be accelerated such that the amount of shares vested under such Stock Awards shall equal that number of shares that would have been vested if Executive had continued to render services to the Company for 12 continuous months after the date of Executive's termination of employment.

(e) **Health Insurance Benefits.** To the extent provided by the federal COBRA law or, if applicable, state insurance laws, and by the Company's current group health insurance policies, Executive will be eligible to continue Executive's group health insurance benefits at Executive's own expense. If Executive timely elects continued coverage under COBRA, the Company shall pay Executive's COBRA premiums, and any applicable Company COBRA premiums, necessary to continue Executive's then-current coverage for a period of 12 months after the date of Executive's termination of employment; *provided, however,* that any such payments will cease if Executive voluntarily enrolls in a health insurance plan offered by another employer or entity during the period in which the Company is paying such premiums. Executive agrees to immediately notify the Company in writing of any such enrollment.

Notwithstanding the foregoing, if the Company determines, in its sole discretion, that it cannot provide the foregoing benefit without potentially incurring financial costs or penalties under applicable law (including, without limitation, Section 2716 of the Public Health Service Act), the Company shall in lieu thereof provide to Executive a taxable monthly amount to continue his group health insurance coverage in effect on the date of separation from service (which amount shall be based on the premium for the first month of COBRA coverage), which payments shall be made regardless of whether Executive elects COBRA continuation coverage and shall commence in the month following the month in which Executive incurs a separation from service and shall end on the earlier of (x) the date on which Executive voluntarily enrolls in a health insurance plan offered by another employer or entity during the period in which the Company is paying such amounts and (y) 12 months after the date of Executive's separation from service.

(f) **Disability Plans.** Nothing in this Section 6.3 shall affect Executive's rights under any disability plan in which Executive is a participant.

6.4 **Termination by the Company for Cause.**

(a) **No Liability.** The Company may terminate Executive's employment and this Agreement for Cause (as defined below) without liability at any time. In such event, the Company shall pay Executive all Accrued Compensation (as defined in Section 6.2(b) herein), but no other compensation or reimbursement of any kind, including without limitation, any severance compensation or benefits shall be paid, and thereafter the Company's obligations hereunder shall terminate.

(b) **Definition of "Cause."** For purposes of this Agreement, "Cause" shall mean one or more of the following:

(i) Executive's intentional commission of an act, or intentional failure to act, that materially injures the business of the Company; *provided, however,* that in no event shall any business judgment made in good faith by Executive and within Executive's defined scope of authority constitute a basis for termination for Cause under this Agreement;

(ii) Executive's intentional refusal or intentional failure to act in accordance with any lawful and proper direction or order of the Board of Directors or the CEO;

(iii) Executive's material breach of Executive's fiduciary, statutory, contractual, or common law duties to the Company (including any material breach of this Agreement, the Confidentiality Agreement, or the Company's written policies);

(iv) Executive's indictment for or conviction of any felony or any crime involving dishonesty; or

(v) Executive's participation in any fraud or other act of willful misconduct against the Company;

provided, however, that in the event that any of the foregoing events is reasonably capable of being cured, the Company shall provide written notice to Executive describing the nature of such event and Executive shall thereafter have ten (10) business days to cure such event.

6.5 Termination by the Company without Cause.

(a) **The Company's Right.** The Company may terminate Executive's employment and this Agreement without Cause (as defined in Section 6.4(b) herein) at any time by giving thirty (30) days advance written notice to Executive.

(b) **Severance Benefits.** If the Company terminates Executive's employment without Cause, and if Executive signs the Release on or within the time period set forth therein (but in no event later than forty-five (45) days after the termination date) and allows such Release to become effective, then:

(i) **Accrued Compensation.** The Company shall pay to Executive all Accrued Compensation (as defined in Section 6.2(b) herein).

(ii) **Cash Compensation Amount Payments.** The Company shall pay Executive an amount equal to (A) Executive's annual Base Salary plus Executive's Target Annual Bonus (as defined in Section 3.2 herein) multiplied by (B) 1.0 (the "Cash Compensation Amount"). Subject to the provisions of Section 6.11, the Cash Compensation Amount will be paid in equal installments on the Company's standard payroll dates over a period of 12 months following Executive's separation from service; provided, however, that any amounts that would otherwise be scheduled to be paid prior to the Release Effective Date shall instead accrue and be paid during the first payroll period following the Release Effective Date, and all other payments shall be made as originally scheduled.

(iii) **Stock Awards.** The vesting of all outstanding Stock Awards held by Executive shall be accelerated such that the amount of shares vested under such Stock Awards shall equal that number of shares that would have been vested if Executive had continued to render services to the Company for 12 continuous months after the date of Executive's termination of employment.

(iv) **Health Insurance Benefits.** To the extent provided by the federal COBRA law or, if applicable, state insurance laws, and by the Company's current group health insurance policies, Executive will be eligible to continue Executive's group health insurance benefits at Executive's own expense. If Executive timely elects continued coverage under COBRA, the Company shall pay Executive's COBRA premiums, and any applicable Company COBRA premiums, necessary to continue Executive's then-current coverage for a period of 12 months after the date of Executive's termination of employment; provided, however, that any such payments will cease if Executive voluntarily enrolls in a health insurance plan offered by another employer or entity during the period in which the Company is paying such premiums. Executive agrees to immediately notify the Company in writing of any such enrollment.

Notwithstanding the foregoing, if the Company determines, in its sole discretion, that it cannot provide the foregoing benefit without potentially incurring financial costs or penalties under applicable law (including, without limitation, Section 2716 of the Public Health Service Act), the Company shall in lieu thereof provide to Executive a taxable monthly amount to continue his group health insurance coverage in effect on the date of separation from service (which amount shall be based on the premium for the first month of COBRA coverage), which payments shall be made regardless of whether Executive elects COBRA continuation coverage and shall commence in the month following the month in which Executive incurs a separation from service and shall end on the earlier of (x) the date on which Executive voluntarily enrolls in a health insurance plan offered by another employer or entity during the period in which the Company is paying such amounts and (y) 12 months after the date of Executive's separation from service.

6.6 Termination by Executive due to a Constructive Termination.

(a) **Executive's Right.** Executive may resign his employment and terminate this Agreement at any time as a result of a Constructive Termination (as defined in Section 6.6(c) herein).

(b) **Severance Benefits.** If Executive resigns his employment and terminates this Agreement as a result of a Constructive Termination, and if Executive signs the Release on or within the time period set forth therein (but in no event later than forty-five (45) days after the termination date) and allows such Release to become effective, then Executive shall receive all of the severance benefits set forth in Section 6.5(b) herein.

(c) **Definition of “Constructive Termination.”** For purposes of this Agreement, “Constructive Termination” shall mean a resignation of employment and termination of this Agreement by Executive for one or more of the following reasons:

(i) Assignment to, or withdrawal from, Executive of any duties or responsibilities that results in a material diminution in such Executive’s authority, duties or responsibilities as in effect immediately prior to such change;

(ii) A material diminution in the authority, duties or responsibilities of the supervisor to whom Executive is required to report;

(iii) A material reduction by the Company of Executive’s annual Base Salary;

(iv) A relocation of Executive or the Company’s principal executive offices if Executive’s principal office is at such offices, to a location more than forty (40) miles from the location at which Executive is then performing his duties, except for an opportunity to relocate which is accepted by Executive in writing; or

(v) A material breach by the Company of any provision of this Agreement or any other enforceable written agreement between Executive and the Company;

provided however, that Executive must first provide the Company with written notice specifying the condition giving rise to a Constructive Termination within ninety (90) days following the initial existence of such condition; and Executive’s notice must specify that Executive intends to terminate his employment no earlier than thirty (30) days after providing such notice, and the Company must be given an opportunity to cure such condition within thirty (30) days following its receipt of such notice and avoid paying benefits.

6.7 Voluntary Resignation. Executive may resign his employment and terminate this Agreement at any time for any reason other than due to a Constructive Termination (as defined in Section 6.6(c) herein). In such event the Company shall pay Executive all Accrued Compensation (as defined in Section 6.2(b) herein), but no other compensation or reimbursement of any kind, including without limitation, any severance compensation or benefits shall be paid, and thereafter the Company’s obligations hereunder shall terminate.

6.8 Change in Control.

(a) **Severance Benefits.** If (i) within three (3) months prior to, or on or within twelve (12) months after, the consummation of a Change in Control (as defined in Section 6.8(b) herein), (1) the Company terminates Executive’s employment and this Agreement without Cause pursuant to Section 6.5 herein or (2) Executive resigns his employment and terminates this Agreement as a result of a Constructive Termination pursuant to Section 6.6 herein, and (ii) in either event (1) or (2), Executive signs the Release on or within the time period set forth therein, but in no event later than forty-five (45) days after the termination date and allows such Release to become effective, then Executive shall receive the following severance benefits in lieu of any severance benefits set forth in Section 6.5(b) or Section 6.6(b) herein:

(i) **Accrued Compensation.** The Company shall pay to Executive all Accrued Compensation (as defined in Section 6.2(b) herein).

(ii) **CIC Cash Compensation Amount Payment.** The Company shall pay Executive an amount equal to (A) Executive’s annual Base Salary plus Executive’s Target Annual Bonus (as defined in Section 3.2 herein) multiplied by (B) 1.5 (collectively, the “CIC Cash Compensation Amount”). The CIC Cash Compensation Amount will be paid in one lump sum within ten (10) days following the Release Effective Date.

(iii) **Stock Awards.** The vesting of all outstanding Stock Awards held by Executive shall be accelerated in full, effective as of the Release Effective Date.

(iv) **Health Insurance Benefits.** To the extent provided by the federal COBRA law or, if applicable, state insurance laws, and by the Company’s current group health insurance policies, Executive will be eligible to continue Executive’s group health insurance benefits at Executive’s own expense. If Executive timely elects continued coverage under COBRA, the Company shall pay Executive’s COBRA premiums, and any applicable Company COBRA premiums, necessary to continue Executive’s then-current coverage for a period of 18 months after

the date of Executive's termination of employment; *provided, however*, that any such payments will cease if Executive voluntarily enrolls in a health insurance plan offered by another employer or entity during the period in which the Company is paying such premiums. Executive agrees to immediately notify the Company in writing of any such enrollment.

Notwithstanding the foregoing, if the Company determines, in its sole discretion, that it cannot provide the foregoing benefit without potentially incurring financial costs or penalties under applicable law (including, without limitation, Section 2716 of the Public Health Service Act), the Company shall in lieu thereof provide to Executive a taxable monthly amount to continue his group health insurance coverage in effect on the date of separation from service (which amount shall be based on the premium for the first month of COBRA coverage), which payments shall be made regardless of whether Executive elects COBRA continuation coverage and shall commence in the month following the month in which Executive incurs a separation from service and shall end on the earlier of (x) the date on which Executive voluntarily enrolls in a health insurance plan offered by another employer or entity during the period in which the Company is paying such amounts and (y) 18 months after the date of Executive's separation from service.

(b) For purposes of this Agreement, a "Change in Control" shall have occurred if at any time following the Effective Date, any of the following events shall occur:

(i) The Company is merged, or consolidated, or reorganized into or with another corporation or other legal person, and as a result of such merger, consolidation or reorganization less than 50% of the combined voting power of the then-outstanding securities of such corporation or person immediately after such transaction are held in the aggregate by the holders of voting securities of the Company immediately prior to such transaction;

(ii) The Company sells all or substantially all of its assets or any other corporation or other legal person and thereafter, less than 50% of the combined voting power of the then-outstanding voting securities of the acquiring or consolidated entity are held in the aggregate by the holders of voting securities of the Company immediately prior to such sale;

(iii) There is a report filed after the date of this Agreement on Schedule 13D or Schedule 14D-1 (or any successor schedule, form or report), each as promulgated pursuant to the Securities Exchange Act of 1934 (the "Exchange Act") disclosing that any person (as the term "person" is used in Section 13(d)(3) or Section 14(d)(2) of the Exchange Act) has become the beneficial owner (as the term beneficial owner is defined under Rule 13d-3 or any successor rule or regulation promulgated under the Exchange Act) representing 50% or more of the combined voting power of the then-outstanding voting securities of the Company; or

(iv) During any period of two (2) consecutive years following the Effective Date, individuals who at the beginning of any such period constitute the directors of the Company cease for any reason to constitute at least a majority thereof unless the election to the nomination for election by the Company's shareholders of each director of the Company first elected during such period was approved by a vote of at least two-thirds of the directors of the Company then still in office who were directors of the Company at the beginning of such period.

6.9 Mitigation. Except as otherwise specifically provided herein, Executive shall not be required to mitigate the amount of any payment provided under this Agreement by seeking other employment or self-employment, nor shall the amount of any payment provided for under this Agreement be reduced by any compensation earned by Executive as a result of employment by another employer or through self-employment or by retirement benefits after the date of Executive's termination of employment from the Company, except as provided herein.

6.10 Coordination. If upon termination of employment, Executive becomes entitled to rights under other plans, contracts or arrangements entered into by the Company, this Agreement shall be coordinated with such other arrangements so that Executive's rights under this Agreement are not reduced, and that any payments under this Agreement offset the same types of payments otherwise provided under such other arrangements, but do not otherwise reduce any payments or benefits under such other arrangements to which Executive becomes entitled.

6.11 Application of Section 409A. Notwithstanding anything to the contrary herein, the following provisions apply to the extent severance benefits provided herein are subject to Section 409A of the Code and the regulations and other guidance thereunder and any state law of similar effect (collectively "Section 409A"). Severance benefits shall not commence until Executive has a "separation from service" for purposes of Section 409A. If Executive is a "specified employee" within the meaning of 409A(a)(2)(B)(i) of the Code, any installment payments of Disability

Base Salary Payments pursuant to Section 6.3(b) or Cash Compensation Amounts pursuant to Section 6.5(b) or 6.6(b) that are triggered by a separation from service shall be accelerated to the minimum extent necessary so that (a) the lesser of (y) the total cash severance payment amount, or (z) six (6) months of such installment payments are paid no later than March 15 of the calendar year following such termination, and (b) all amounts paid pursuant to the foregoing clause (a) will constitute separate payments for purposes of Section 1.409A-2(b)(2) of the Treasury Regulations and thus will be payable pursuant to the "short-term deferral" rule set forth in Section 1.409A-1(b)(4) of the Treasury Regulations. It is intended that if Executive is a "specified employee" within the meaning of Section 409A(a)(2)(B)(i) of the Code at the time of such separation from service the foregoing provision shall result in compliance with the requirements of Section 409A(a)(2)(B)(i) of the Code because payments to Executive will either be payable pursuant to the "short-term deferral" rule set forth in Section 1.409A-1(b)(4) of the Treasury Regulations or will not be paid until at least 6 months after separation from service. The severance benefits are intended to qualify for an exemption from application of Section 409A or comply with its requirements to the extent necessary to avoid adverse personal tax consequences under Section 409A, and any ambiguities herein shall be interpreted accordingly.

6.12 Parachute Payments.

(a) If any payment or benefit (including payments or benefits pursuant to this Agreement) that Executive would receive in connection with a Change in Control or otherwise ("Payment") would (1) constitute a "parachute payment" within the meaning of Section 280G of the Code, and (2) but for this sentence, be subject to the excise tax imposed by Section 4999 of the Code (the "Excise Tax"), then such Payment shall be equal to the Reduced Amount. The "Reduced Amount" shall be either (x) the largest portion of the Payment that would result in no portion of the Payment being subject to the Excise Tax or (y) the largest portion, up to and including the total, of the Payment, whichever amount, after taking into account all applicable federal, state and local employment taxes, income taxes, and the Excise Tax (all computed at the highest applicable marginal rate), results in Executive's receipt, on an after-tax basis, of the greater economic benefit notwithstanding that all or some portion of the Payment may be subject to the Excise Tax. If a reduction in payments or benefits constituting "parachute payments" is necessary so that the Payment equals the Reduced Amount, Executive shall have no rights to any additional payments and/or benefits, and reduction shall occur in the manner that results in the greatest economic benefit for Executive. If more than one method of reduction will result in the same economic benefit, the items so reduced will be reduced pro rata.

(b) In the event it is subsequently determined by the Internal Revenue Service that some portion of the Reduced Amount as determined pursuant to clause (x) in the preceding paragraph is subject to the Excise Tax, Executive agrees to promptly return to the Company a sufficient amount of the Payment so that no portion of the Reduced Amount is subject to the Excise Tax. For the avoidance of doubt, if the Reduced Amount is determined pursuant to clause (y) in the preceding paragraph, Executive will have no obligation to return any portion of the Payment pursuant to the preceding sentence.

(c) The independent registered public accounting firm engaged by the Company for general audit purposes as of the day prior to the effective date of the event described in Section 280G(b)(2)(A)(i) of the Code will perform the foregoing calculations. If the independent registered public accounting firm so engaged by the Company is serving as accountant or auditor for the individual, entity or group effecting such Change in Control or similar transaction, the Company will appoint a nationally recognized independent registered public accounting firm to make the determinations required hereunder. The Company will bear all expenses with respect to the determinations by such independent registered public accounting firm required to be made hereunder. Any good faith determinations of the independent registered public accounting firm made hereunder will be final, binding and conclusive upon the Company and you.

Article 7

GENERAL PROVISIONS

7.1 Governing Law. The validity, interpretation, construction and performance of this Agreement and the rights of the parties thereunder shall be interpreted and enforced under California law without reference to principles of conflicts of laws.

7.2 Assignment; Successors; Binding Agreement.

(a) **No Assignment.** Executive may not assign, pledge or encumber his interest in this Agreement or any part thereof.

(b) **Assumption by Successor.** The Company will require any successor (whether direct or indirect, by purchase, merger, consolidation or otherwise) to all or substantially all of the business and/or assets of the Company, by operation of law or by agreement in form and substance reasonably satisfactory to Executive, to assume and agree to perform this Agreement in the same manner and to the same extent that the Company would be required to perform it if no such succession had taken place.

(c) **Binding Agreement.** This Agreement shall inure to the benefit of and be enforceable by Executive's personal or legal representatives, executors, administrators, successors, heirs, distributee, devisees and legatees. If Executive should die while any amount is at such time payable to Executive hereunder, all such amounts, unless otherwise provided herein, shall be paid in accordance with the terms of this Agreement to Executive's devisee, legates or other designee or, if there be no such designee, to his estate.

7.3 Notice. For the purposes of this Agreement, notices and all other communications provided for in this Agreement shall be in writing and shall be deemed to have been duly given when delivered or mailed by certified or registered mail, return receipt requested, postage prepaid, addressed to the respective addresses set forth below or to such other address as either party may have furnished to the other in writing in accordance herewith, except that notice of change of address shall be effective only upon receipt.

To the Company:

Retrophin, Inc.
3721 Valley Centre Drive, Suite 250
San Diego, CA 92130

To Executive:

7.4 Modification; Waiver; Entire Agreement. This Agreement constitutes the complete, final and exclusive embodiment of the entire agreement between Executive and the Company with regard to this subject matter. It is entered into without reliance on any promise or representation, written or oral, other than those expressly contained herein, and it supersedes any other such promises, warranties or representations. No provisions of this Agreement may be modified, waived or discharged unless such waiver, modification or discharge is agreed to in writing signed by Executive and such officer as may be specifically designated by the Board of Directors of the Company. No waiver by either party hereto at any time of any breach by the other party of, or compliance with, any condition or provision of this Agreement to be performed by such other party shall be deemed a waiver of similar or dissimilar provisions or conditions at the same or any prior or subsequent time.

7.5 Validity. The invalidity or unenforceability of any provision of this Agreement shall not affect the validity or enforceability of any other provision of this Agreement, which shall remain in full force and effect.

7.6 Controlling Document. Except to the extent described in Section 6.10, in case of conflict between any of the terms and conditions of this Agreement and any document herein referred to, the terms and conditions of this Agreement shall control.

7.7 Executive Acknowledgment. Executive acknowledges (a) that he has consulted with or has had the opportunity to consult with independent counsel of his own choice concerning this Agreement, and has been advised to do so by the Company, and (b) that he has read and understands the Agreement, is fully aware of its legal effect, and has entered into it freely based on his own judgment.

7.8 Dispute Resolution. To ensure the rapid and economical resolution of disputes that may arise in connection with Executive's employment, Executive and the Company agree that any and all disputes, claims, or causes of action, in law or equity, arising from or relating to the enforcement, breach, performance, execution, or interpretation of this Agreement, Executive's employment, or the termination of that employment, shall be resolved, to the fullest extent permitted by law pursuant to the Federal Arbitration Act, 9 U.S.C. §1-16, by final, binding and confidential arbitration in San Diego, California conducted before a single arbitrator by Judicial Arbitration and Mediation Services, Inc. ("JAMS") or its successor, under the then applicable JAMS rules; *provided, however*, that in no event shall the Arbitrator be empowered to hear or determine any class or collective claim of any type. The JAMS rules can be found online at www.jamsadr.com. By agreeing to this arbitration procedure, both Executive and the Company

waive the right to resolve any such dispute through a trial by jury or judge or by administrative proceeding. The arbitrator shall: (a) have the authority to compel adequate discovery for the resolution of the dispute and to award such relief as would otherwise be permitted by law; and (b) issue a written arbitration decision including the arbitrator's essential findings and conclusions and a statement of the award. The Company shall pay all of JAMS' arbitration fees. Nothing in this letter agreement shall prevent either Executive or the Company from obtaining injunctive relief in court if necessary to prevent irreparable harm pending the conclusion of any arbitration. The parties agree that the arbitrator shall award reasonable attorneys' fees, costs, and all other related expenses to the prevailing party in any action brought hereunder, and the arbitrator shall have discretion to determine the prevailing party in an arbitration where multiple claims may be at issue.

7.9 Remedies.

(a) **Injunctive Relief.** The parties agree that the services to be rendered by Executive hereunder are of a unique nature and that in the event of any breach or threatened breach of any of the covenants contained herein, the damage or imminent damage to the value and the goodwill of the Company's business will be irreparable and extremely difficult to estimate, making any remedy at law or in damages inadequate. Accordingly, the parties agree that the Company shall be entitled to injunctive relief against Executive in the event of any breach or threatened breach of any such provisions by Executive, in addition to any other relief (including damage) available to the Company under this Agreement or under law.

(b) **Exclusive.** Both parties agree that the remedy specified in Section 7.9(a) above is not exclusive of any other remedy for the breach by Executive of the terms hereof.

7.10 Counterparts. This Agreement may be executed in one or more counterparts, all of which taken together shall constitute one and the same Agreement.

Executed by the parties as follows:

EXECUTIVE

RETROPHIN, INC.

By: Noah Rosenberg, M.D.

By: Stephen Aselage

Date: December 21, 2018

Date: July 26, 2018

EXHIBIT A
GENERAL RELEASE
[To be signed on or after employment termination date]

Pursuant to the terms of the Employment Agreement between Retrophin, Inc. (the "Company") and Noah Rosenberg, M.D. ("Executive") dated _____, 2018 (the "Agreement"), the parties hereby enter into the following General Release (the "Release"):

1. Accrued Salary and Vacation. Executive understands that, on the last date of Executive's employment with the Company, the Company will pay Executive any accrued salary and accrued and unused vacation to which Executive is entitled by law, regardless of whether Executive signs this Release.

2. General Release. Executive hereby generally and completely releases the Company and its directors, officers, employees, shareholders, partners, agents, attorneys, predecessors, successors, parent and subsidiary entities, insurers, affiliates, and assigns (collectively the "Released Parties") of and from any and all claims, liabilities and obligations, both known and unknown, arising out of or in any way related to events, acts, conduct, or omissions occurring at any time prior to or at the time that Executive signs this Release.

3. Scope of Release. This general release includes, but is not limited to: (1) all claims arising out of or in any way related to Executive's employment with the Company or the termination of that employment; (2) all claims related to Executive's compensation or benefits from the Company, including salary, bonuses, commissions, vacation pay, expense reimbursements, severance pay, fringe benefits, stock, stock options, or any other ownership or equity interests in the Company; (3) all claims for breach of contract, wrongful termination, and breach of the implied covenant of good faith and fair dealing (including claims based on or arising under the Agreement); (4) all tort claims, including claims for fraud, defamation, emotional distress, and discharge in violation of public policy; and (5) all federal, state, and local statutory claims, including claims for discrimination, harassment, retaliation, attorneys' fees, or other claims arising under the federal Civil Rights Act of 1964 (as amended), the federal Americans with Disabilities Act of 1990, the federal Age Discrimination in Employment Act (as amended) ("ADEA"), the federal Family and Medical Leave Act, the California Labor Code (as amended), the California Family Rights Act, and the California Fair Employment and Housing Act (as amended).

4. ADEA Waiver. Executive acknowledges that Executive is knowingly and voluntarily waiving and releasing any rights Executive may have under the ADEA, and that the consideration given for the waiver and release in the preceding paragraph is in addition to anything of value to which Executive is already entitled. If Executive is age 40 or older upon execution of this Release, Executive further acknowledges that Executive has been advised by this writing that, (1) Executive's waiver and release do not apply to any rights or claims that may arise after the date Executive signs this Release; (2) Executive should consult with an attorney prior to signing this Release (although Executive may choose voluntarily not to do so); (3) Executive has twenty-one (21) days to consider this Release (although Executive may choose voluntarily to sign it earlier); (4) Executive has seven (7) days following the date Executive signs this Release to revoke it by providing written notice of revocation to the Company's Chief Executive Officer; and (5) this Release will not be effective until the date upon which the revocation period has expired, which will be the eighth calendar day after the date Executive signs it provided that Executive does not revoke it. If Executive is under 40 years of age upon execution of this Release, the Release will be effective upon signing and not revocable.

5. Waiver of Unknown Claims. EXECUTIVE UNDERSTANDS THAT THIS AGREEMENT INCLUDES A RELEASE OF ALL KNOWN AND UNKNOWN CLAIMS. Executive acknowledges that Executive has read and understands Section 1542 of the California Civil Code which reads as follows: "A general release does not extend to claims which the creditor does not know or suspect to exist in his or her favor at the time of executing the release, which if known by him or her must have materially affected his or her settlement with the debtor." Executive hereby expressly waives and relinquishes all rights and benefits under that section and any law or legal principle of similar effect in any jurisdiction with respect to Executive's respective release of claims herein, including but not limited to Executive's release of unknown and unsuspected claims.

6. Excluded Claims. Executive understands that notwithstanding the foregoing, the following are not included in the Released Claims (the "Excluded Claims"): (i) any rights or claims for indemnification Executive may have pursuant to any written indemnification agreement to which he is a party, the charter, bylaws, or operating agreements of any of the Released Parties, or under applicable law; or (ii) any rights which are not waivable as a matter of law. In addition, Executive understands that nothing in this release prevents Executive from filing, cooperating

with, or participating in any proceeding before the Equal Employment Opportunity Commission, the Department of Labor, or any similar government agency, except that Executive acknowledges and agrees that Executive shall not recover any monetary benefits in connection with any such claim, charge or proceeding with regard to any claim released herein. Executive hereby represents and warrants that, other than the Excluded Claims, Executive is not aware of any claims he has or might have against any of the Released Parties that are not included in the Released Claims.

7. **Executive Representations.** Executive hereby represents that Executive has been paid all compensation owed and for all hours worked; Executive has received all the leave and leave benefits and protections for which Executive is eligible, pursuant to the Family and Medical Leave Act, the California Family Rights Act, or otherwise; and Executive has not suffered any on-the-job injury for which Executive has not already filed a workers' compensation claim.

8. **Nondisparagement.** Executive agrees not to disparage the Company, its parent, or its or their officers, directors, employees, shareholders, affiliates and agents, in any manner likely to be harmful to its or their business, business reputation, or personal reputation (although Executive may respond accurately and fully to any question, inquiry or request for information as required by legal process).

9. **Cooperation.** Executive agrees not to voluntarily (except in response to legal compulsion) assist any third party in bringing or pursuing any proposed or pending litigation, arbitration, administrative claim or other formal proceeding against the other party, or against the Company's parent or subsidiary entities, affiliates, officers, directors, employees or agents. Executive further agrees to reasonably cooperate with the other party, by voluntarily (without legal compulsion) providing accurate and complete information, in connection with such other party's actual or contemplated defense, prosecution, or investigation of any claims or demands by or against third parties, or other matters, arising from events, acts, or failures to act that occurred during the period of Executive's employment by the Company.

10. **No Admission of Liability.** The parties agree that this Release, and performance of the acts required by it, does not constitute an admission of liability, culpability, negligence or wrongdoing on the part of anyone, and will not be construed for any purpose as an admission of liability, culpability, negligence or wrongdoing by any party and/or by any party's current, former or future parents, subsidiaries, related entities, predecessors, successors, officers, directors, shareholders, agents, employees and assigns. The parties specifically acknowledge and agree that this Release is a compromise of disputed claims and that the Company and Executive denies any liability for any matter released herein.

EXECUTIVE

RETROPHIN, INC.

By: _____

By: _____

Date: _____

Date: _____

**RETROPHIN, INC.
LIST OF SUBSIDIARIES**

No.	Name
1	Retrophin Pharmaceutical, Inc.
2	Retrophin Therapeutics I, Inc.
3	Retrophin Therapeutics II, Inc.
4	Retrophin Europe Ltd
5	Retrophin International Holdings Ltd
6	RTRX International CV
7	Retrophin Therapeutics International LLC
8	US LLC 2
9	Retrophin Research Ltd
10	Retrophin US Holdings LLC
11	Manchester Pharmaceuticals LLC
12	Centurion Merger Sub, Inc.
13	[10-em856580]

Consent of Independent Registered Public Accounting Firm

Retrophin, Inc.
San Diego, California

We hereby consent to the incorporation by reference in the Registration Statements on Form S-3 (Nos. 333-227182, 333-202861 and 333-198648) and Form S-8 (Nos. 333-224848, 333-218582, 333-213599, 333-206510 and 333-200224) of Retrophin, Inc. of our reports dated February 26, 2019, relating to the consolidated financial statements and the effectiveness of Retrophin, Inc.'s internal control over financial reporting, which appear in this Form 10-K.

/s/ BDO USA, LLP

New York, New York
February 26, 2019

**CERTIFICATION OF CHIEF EXECUTIVE OFFICER
PURSUANT TO EXCHANGE ACT RULE 13a-14(a) OR 15d-14(a)**

I, Eric M. Dube, certify that:

1. I have reviewed this Annual Report on Form 10-K of Retrophin, 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: February 26, 2019

/s/ Eric M. Dube

Eric M. Dube

Chief Executive Officer

(Principal Executive Officer)

**CERTIFICATION OF
CHIEF FINANCIAL OFFICER
PURSUANT TO EXCHANGE ACT RULE 13a-14(a) OR 15d-14(a)**

I, Laura Clague, certify that:

1. I have reviewed this Annual Report on Form 10-K of Retrophin, 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: February 26, 2019

/s/ Laura Clague

Laura Clague

Chief Financial Officer

(Principal Financial Officer)

**CERTIFICATION OF
CHIEF EXECUTIVE OFFICER
PURSUANT TO 18 U.S.C. SECTION 1350
AS ADOPTED PURSUANT TO
SECTION 906 OF THE SARBANES-OXLEY ACT OF 2002**

In connection with the accompanying Annual Report on Form 10-K of Retrophin, Inc. (the "Company"), for the period ended December 31, 2018 (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 such officer's 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.

Date: February 26, 2019

/s/ Eric M. Dube

Eric M. Dube
Chief Executive Officer
(Principal Executive Officer)

**CERTIFICATION OF
CHIEF FINANCIAL OFFICER
PURSUANT TO 18 U.S.C. SECTION 1350
AS ADOPTED PURSUANT TO
SECTION 906 OF THE SARBANES-OXLEY ACT OF 2002**

In connection with the accompanying Annual Report on Form 10-K of Retrophin, Inc. (the "Company"), for the period ended December 31, 2018 (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 such officer's 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.

Date: February 26, 2019

/s/ Laura Clague

Laura Clague

Chief Financial Officer

(Principal Financial Officer)