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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, 2017

OR

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

For the transition period from to .

Commission File Number: 001-35619

STEMLINE THERAPEUTICS, INC.

(Exact name of registrant as specified in its charter)

Delaware	**45-0522567**
(State or other jurisdiction of incorporation or organization)	(I.R.S. Employer Identification Number)

750 Lexington Avenue
Eleventh Floor
New York, New York 10022
(Address of principal executive offices, including zip code)

(646)-502-2311
(Registrant's telephone number, including area code)

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

Common Stock, par value $0.0001 per share	**NASDAQ Capital Market**
(Title of Class)	(Name of Each Exchange on Which Registered)

Securities registered pursuant to Section 12(g) of the Act: **None**

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

Indicate by check mark if disclosure of delinquent filers pursuant to Item 405 of Regulation S-K (§229.405 of this chapter) is not contained herein, and will not be contained, to the best of registrant's knowledge, in definitive proxy or information statements incorporated by reference in Part III of this Form 10-K or any amendment to this Form 10-K. ☐ Yes ☒ No

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

Large accelerated filer	☐	Accelerated filer	☒
Non-accelerated filer	☐ (Do not check if a smaller reporting company)	Smaller reporting company	☐
		Emerging growth company	☒

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

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

The aggregate market value of voting common stock held by non-affiliates of the registrant (assuming, for purposes of this calculation, without conceding, that all executive officers and directors are "affiliates") was $205,840,220 as of June 30, 2017, based on the closing sale price of such stock as reported on the NASDAQ Capital Market.

There were 30,210,274 shares of the registrant's common stock outstanding as of March 16, 2018.

DOCUMENTS INCORPORATED BY REFERENCE

Portions of the registrant's Proxy Statement for the 2018 Annual Meeting of Stockholders are incorporated by reference in Part III of this Annual Report on Form 10-K.

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This Annual Report on Form 10-K contains trademarks and trade names of Stemline Therapeutics, Inc., including our name and logo. All other trademarks, service marks, or trade names referenced in this Annual Report on Form 10-K are the property of their respective owners.

SPECIAL CAUTIONARY NOTICE REGARDING FORWARD-LOOKING STATEMENTS

This annual report on Form 10-K ("Form 10-K") includes statements that are, or may be deemed, "forward-looking statements." In some cases, these forward-looking statements can be identified by the use of forward-looking terminology, including the terms "believes," "estimates," "anticipates," "expects," "plans," "intends," "may," "could," "might," "will," "should," "approximately" or, in each case, their negative or other variations thereon or comparable terminology, although not all forward-looking statements contain these words. They appear in a number of places throughout this Form 10-K and include statements regarding our intentions, beliefs, projections, outlook, analyses or current expectations concerning, among other things, our history of net operating losses and uncertainty regarding our ability to obtain capital and achieve profitability, our ability to develop and commercialize our product candidates, our ability to advance our development programs, enroll our trials, and achieve clinical endpoints, our ability to use or expand our technology to build a pipeline of product candidates, our ability to obtain and maintain regulatory approval of our product candidates and comply with ongoing regulatory requirements, our ability to successfully operate in a competitive industry and gain market acceptance by physician, provider, patient, and payor communities, our reliance on third parties, unstable economic or market conditions, and our ability to obtain and adequately protect intellectual property rights for our product candidates.

By their nature, forward-looking statements involve risks and uncertainties because they relate to events, competitive dynamics, and healthcare, regulatory and scientific developments and depend on the economic circumstances that may or may not occur in the future or may occur on longer or shorter timelines than anticipated. Although we believe that we have a reasonable basis for each forward-looking statement contained in this Form 10-K, we caution you that forward-looking statements are not guarantees of future performance and that our actual results of operations, financial condition and liquidity, and the development of the industry in which we operate may differ materially from the forward-looking statements contained in this Form 10-K. In addition, even if our results of operations, financial condition and liquidity, and the development of the industry in which we operate are consistent with the forward-looking statements contained in this Form 10-K, they may not be predictive of results or developments in future periods.

Some of the factors that we believe could cause actual results to differ from those anticipated or predicted include:

- the success and timing of our clinical trials, including safety and efficacy of our product candidates, patient accrual, unexpected or expected safety events, and the usability of data generated from our trials;
- our ability to successfully file and obtain timely marketing approval from the U.S. Food and Drug Administration, or FDA, or comparable foreign regulatory agency for one or more Biologics License Applications, or BLAs, or New Drug Applications, or NDAs;
- our ability to obtain and maintain marketing approval from regulatory agencies for our products in the U.S. and foreign countries;
- our ability to adhere to ongoing compliance requirements of all health authorities, in the U.S. and foreign countries;
- our ability to obtain and maintain adequate reimbursement for our products;
- our ability to obtain the desired labeling of our products under any regulatory approval we might receive;
- our plans to develop and commercialize our products;
- the successful development and implementation of sales and marketing campaigns;
- the loss of key scientific or management personnel;
- the size and growth of the potential markets for our product candidates and our ability to serve those markets;
- our ability to successfully compete in the potential markets for our product candidates, if commercialized;
- regulatory developments in the United States and foreign countries;
- the rate and degree of market acceptance of any of our product candidates;
- new products, product candidates or new uses for existing products or technologies introduced or announced by our competitors and the timing of these introductions or announcements;
- market conditions in the pharmaceutical and biotechnology sectors;
- our available cash and investments;
- the accuracy of our estimates regarding expenses, future revenues, capital requirements and needs for additional financing;
- our ability to obtain additional funding;
- our ability to obtain and maintain intellectual property protection for our product candidates;
- our ability to maintain the license agreements for SL-401, SL-801, SL-701 and our other in-licensed product candidates;
- the success and timing of our preclinical studies, including those intended to support an Investigational New Drug, or IND, application;
- the ability of our product candidates to successfully perform and advance in clinical trials;
- our ability to obtain and maintain authorization from regulatory authorities for use of our product candidates for initiation and conduct of clinical trials;

- our ability to manufacture and supply our products, gain access to products we plan to use in combination studies and the performance of and reliance on third-party manufacturers and suppliers;
- the performance of our clinical research organizations, clinical trial sponsors, and clinical trial investigators; and
- our ability to successfully implement our strategy.

Any forward-looking statements that we make in this Form 10-K speak only as of the date of such statement, and we undertake no obligation to update such statements to reflect events or circumstances after the date of this Form 10-K. You should also read carefully the factors described in the "Risk Factors" section of this Form 10-K to better understand the risks and uncertainties inherent in our business and underlying any forward-looking statements. As a result of these risks and uncertainties, our actual results may differ materially from those reflected in the forward-looking statements in this Form 10-K.

This Form 10-K includes statistical and other industry and market data that we obtained from industry publications and research, surveys and studies conducted by third-parties. Industry publications and third-party research, surveys and studies generally indicate that their information has been obtained from sources believed to be reliable, although they do not guarantee the accuracy or completeness of such information. While we believe these industry publications and third-party research, surveys and studies are reliable, we have not independently verified such data.

We qualify all of our forward-looking statements by these cautionary statements. In addition, with respect to all of our forward-looking statements, we claim the protection of the safe harbor for forward-looking statements contained in the Private Securities Litigation Reform Act of 1995.

Part I

Unless the context requires otherwise, references in this report to "Stemline," "Company," "we," "us" and "our" refer to Stemline Therapeutics, Inc.

Item 1. Business

Overview

We are a clinical stage biopharmaceutical company focused on discovering, acquiring, developing and potentially commercializing innovative oncology therapeutics that target difficult to treat cancers. Our clinical pipeline includes: SL-401, SL-801, and SL-701.

SL-401 pivotal data; plans for registration and potential commercialization: SL-401 has completed a pivotal trial in patients with blastic plasmacytoid dendritic cell neoplasm, or BPDCN, and has successfully met the primary endpoint of the trial. SL-401 is a targeted therapy directed to the interleukin-3 receptor-α, or CD123. SL-401 was granted breakthrough therapy designation, or BTD, by the U.S. Food and Drug Administration, or FDA, for the treatment of patients with BPDCN. Based on the trial results and other data, we expect to complete submission of a rolling Biologics License Application, or BLA, with the FDA in the first half of 2018. If successful, SL-401 would be the first drug ever approved for this indication, and pre-launch activities are underway in preparation for this potential outcome.

SL-401 additional clinical activities: SL-401 is also being assessed in additional indications, including in Phase 1/2 clinical trials of chronic myelomonocytic leukemia, or CMML, myelofibrosis, or MF, acute myeloid leukemia, or AML, and multiple myeloma. Preliminary data from some of these programs were presented at the 2017 American Society of Hematology, or ASH, Annual Meeting and Exposition, and further updates are expected this year.

Additional clinical-stage candidates: SL-801 is a novel, oral, small molecule, reversible inhibitor of Exportin-1, or XPO1, a nuclear transport protein involved in a variety of cancers. SL-801 is being assessed in a Phase 1 dose escalation trial of patients with advanced solid tumors. Preliminary data were presented at the European Society of Medical Oncology, or ESMO, Annual Congress 2017, and further updates are expected this year as dose escalation continues. SL-701, an immunotherapeutic, has completed a Phase 2 trial of patients with relapsed/refractory glioblastoma, or GBM. Data were presented at the 2017 Society for Neuro-Oncology, or SNO, meeting, and further updates are expected this year.

Our Company

We were incorporated under the laws of the State of Delaware in August 2003. Our principal executive office is located at 750 Lexington Avenue, Eleventh Floor, New York, New York 10022 and our telephone number is (646) 502-2311.

Our website address is www.stemline.com. The information set forth on our website is not a part of this report. We will make available free of charge through our website our annual reports on Form 10-K, quarterly reports on Form 10-Q and current reports on Form 8-K, and any amendments to these reports, as soon as reasonably practicable after we electronically file such material with, or furnish such material to, the Securities and Exchange Commission, or SEC. We are not including the information on our website as a part of, nor incorporating it by reference into, this report. You may read and copy any materials we file at the SEC's Public Reference Room at 100 F Street, N.E., Washington, D.C. 20549. You may obtain information on the operation of the Public Reference Room by calling the SEC at 1-800-SEC-0330. Additionally, the SEC maintains a website that contains annual, quarterly, and current reports, proxy statements, and other information that issuers (including us) file electronically with the SEC. The SEC's website address is **http://www.sec.gov/.**

Management

We are led by a team with extensive experience in the biopharmaceutical industry including:

- Ivan Bergstein, M.D. — Chairman, Chief Executive Officer and President. Dr. Bergstein is Chief Executive Officer and Founder of Stemline Therapeutics. Dr. Bergstein has managed the company's evolution from early-stage research and development to current late clinical stage. Prior to founding Stemline, Dr. Bergstein was Medical Director of Access Oncology, Inc., a clinical stage oncology-focused biotechnology company where he was a key member of a small team responsible for the acquisition and development of the company's clinical stage assets and ultimately the sale of the company to Keryx Biopharmaceuticals, Inc. (Nasdaq: KERX). Previously, he was a senior biopharmaceuticals analyst at

a Wall Street-based firm that advised funds on investment opportunities in public companies with late clinical stage assets. He received a BA in mathematics from the University of Pennsylvania and was elected to the Pi Mu Epsilon National Mathematics Honor Society, and then received an MD from the Mount Sinai School of Medicine where he was elected to the Alpha Omega Alpha Honor Medical Society, received the Merck Award for Clinical Excellence, and subsequently completed an internship in general surgery. He then became the Jerome A. Urban Post-Doctoral Research Fellow at the Cornell University Medical College where he studied and published work relating to Wnt genes in human breast cancer. He then completed an internal medicine residency and hematology-oncology fellowship at the New York Presbyterian Hospital — Weill Medical College of Cornell University where he studied and published work on gene therapy manipulations of the sonic hedgehog pathway.

- Kenneth Hoberman — Chief Operating Officer. Mr. Hoberman has extensive financial, accounting, investor relations, corporate governance and business development experience including M&A, strategic alliances and partnerships both domestic and international. His operational expertise includes regulatory oversight, human resources, manufacturing and clinical development. He was previously Vice President of Corporate and Business Development of Keryx Biopharmaceuticals, Inc. (Nasdaq: KERX), where he was instrumental in the success of the company. He also helped secure multiple sources of capital including over $200 million in equity investments through public and private offerings. He also initiated and executed a $100 million strategic alliance and originated, negotiated and closed dozens of licensing and operational contracts, helping to grow the company's market capitalization to over $1 billion. He also led the team that originated, in-licensed, and developed Auryxia™ which was approved by the FDA in September 2014. He is on the Board of Directors of TG Therapeutics, Inc. (Nasdaq: TGTX). He received a B.S.B.A. in Finance from Boston University and completed post-baccalaureate studies at Columbia University.

- David Gionco — Vice President of Finance and Chief Accounting Officer. Mr. Gionco was previously Vice President, Chief Financial Officer and Chief Accounting Officer of Savient Pharmaceuticals, Inc. where he oversaw the finance function for the organization and was instrumental in helping to grow the company, raising over $350 million. Prior to this, Mr. Gionco held audit, corporate accounting, financial planning, finance and controller roles at companies including Merck & Co., Inc. ("Merck") and, previously, Medco Health Solutions, Inc., which was acquired by Merck during his tenure. At Merck, Mr. Gionco held various financial and accounting positions of increasing responsibility. Mr. Gionco also held senior financial positions at Progenics Pharmaceuticals, Inc. (Nasdaq: PGNX) and Odyssey Pharmaceuticals, Inc. (a subsidiary of Pliva, Inc., now Teva Pharmaceutical Industries Ltd. (NYSE: TEVA)). Mr. Gionco previously had 7 years of financial auditing experience with a major public accounting firm. Mr. Gionco holds a B.S. in Accounting from Fairleigh Dickinson University and an MBA in Finance from Rutgers University. Mr. Gionco is a Certified Public Accountant in the State of New York.

Strategy

Our goal is to build a leading biopharmaceutical company focused on improving the lives of patients by developing and commercializing innovative therapeutics for difficult to treat cancers. The fundamental components of our business strategy to achieve this goal include the following:

- *Develop and potentially commercialize SL-401.* SL-401 has been granted breakthrough therapy designation, or BTD, by the FDA for the treatment of blastic plasmacytoid dendritic cell neoplasm, or BPDCN. We have completed a pivotal Phase 2 trial of SL-401 in patients with BPDCN and the trial has met its primary endpoint. We plan to complete submission of a biologics license application, or BLA, in the first half of 2018. In anticipation of success, which we cannot guarantee, we are currently building out a commercial infrastructure to market and sell SL-401, if it attains marketing approval.

- *Develop SL-401 in additional indications.* We are also assessing SL-401 in additional indications including in Phase 1/2 trial of patients with chronic myelomonocytic leukemia, or CMML, and myelofibrosis, or MF. SL-401 is also being assessed in Phase 1/2 trials of other indications including acute myeloid leukemia, or AML, and multiple myeloma, as a single agent and in combination with other therapies.

- *Develop SL-801 in multiple cancer types.* We are advancing SL-801 through a Phase 1 trial in adult patients with advanced solid tumors. Patients are currently enrolling in this dose escalation study and receiving SL-801 as a single agent.

- *Develop SL-701 in brain cancer.* We have completed a Phase 2 trial of SL-701 in adult patients with second-line glioblastoma multiforme, or GBM. Patients received SL-701 alone or in combination with bevacizumab, with immunostimulants. Data are being analyzed and we expect to provide updates for the program later this year.

SL-401

Overview

SL-401 is a novel targeted therapy directed to the interleukin-3 receptor-α, or CD123, a target present on a wide range of malignancies. SL-401 was granted breakthrough therapy designation, or BTD, by the U.S. Food and Drug Administration, or FDA, for the treatment of patients with blastic plasmacytoid dendritic cell neoplasm, or BPDCN. SL-401 has completed a pivotal Phase 2 trial in patients with BPDCN and has met the primary endpoint of the trial. Based on these and other data, we expect to complete submission of a rolling Biologics License Application, or BLA, with the FDA in the first half of 2018. If successful, SL-401 would be the first drug ever approved for this indication. Pre-launch activities are underway in preparation for this potential outcome.

In parallel, we are also conducting additional development activities. SL-401 is being assessed in Phase 1/2 clinical trials of other indications including chronic myelomonocytic leukemia, or CMML, and myelofibrosis, or MF, acute myeloid leukemia, or AML, and multiple myeloma. Preliminary data from some of these programs were presented at the 2017 American Society of Hematology, or ASH, Annual Meeting and Exposition, and further updates are expected this year. Factors that may impact next steps for SL-401 in these additional indications include enrollment trends, adverse events, safety and efficacy results, regulatory paths, commercial landscape, and other medical, business, and practical considerations.

SL-401 design and mechanism of action

SL-401 is a novel targeted therapy directed to the IL-3R (CD123). SL-401 is comprised of human IL-3 recombinantly fused to a truncated diphtheria toxin, or DT, payload engineered such that IL-3 replaces the native DT receptor-binding domain. In this way, the IL-3 domain of SL-401 directs the cytotoxic DT payload to cells expressing CD123. Upon internalization, SL-401 irreversibly inhibits protein synthesis and induces apoptosis of the target cell. Given this novel mechanism of action, there is a potential to develop SL-401 as a single agent and in combination with other therapies.

CD123 is normally expressed on certain maturing hematopoietic cells, including maturing myeloid cells, B cells, plasmacytoid dendritic cells, or pDCs, mast cells, basophils and eosinophils, and appears to be involved in cell maturation, differentiation, and survival. CD123 does not appear to be expressed to a significant degree on normal hematopoietic stem cells.

CD123 is expressed on multiple malignancies including BPDCN, AML, certain myeloproliferative neoplasms, or MPNs, myelodysplastic syndrome, or MDS, chronic myeloid leukemia, or CML, B-cell acute lymphoid leukemia, or B-ALL, hairy cell leukemia, Hodgkin's and certain non-Hodgkin's lymphomas. In addition to expression on tumor bulk, CD123 expression has been reported on the cancer stem cells, or CSCs, of certain hematologic cancers including AML, CML, MDS, and potentially T-cell ALL. In addition, elevated CD123 expression has been correlated with poor prognosis in certain hematologic cancers (Vergez in Haematologica, 2011, Testa in Biomarker Research, 2014).

Notably, CD123+ pDCs, which are the cell of origin for BPDCN, have also been reported in the microenvironment of certain tumors including multiple myeloma, CMML and other MPNs, and some solid tumors where they could play a tumor-promoting role (and preclinically, have been shown to be tumor-promoting in myeloma). CD123+ pDCs have also been implicated in the pathogenesis of certain autoimmune diseases, including scleroderma and cutaneous lupus, and is a potential therapeutic target for these conditions.

SL-401 preclinical activity

SL-401 has demonstrated preclinical in vitro and in vivo activity against a wide range of hematologic cancer types. In AML, SL-401 is highly active against both leukemia blasts (i.e., tumor bulk) and CSCs of a variety of human leukemia cell lines and primary leukemia cells from patients. In particular, SL-401 demonstrated potent cytotoxicity against leukemic cells in vitro in a dose-dependent fashion with concentrations that inhibit the growth of fifty percent (50%) of cells, or an IC50, in the low picomolar range. Notably, normal bone marrow stem cells were relatively insensitive to SL-401. SL-401 also exhibited anti-CSC activity. In particular, SL-401 inhibited AML colony formation, an assay for stem cell activity, compared with normal human bone marrow. As further validation of an anti-CSC effect, SL-401 reduced the incorporation and growth (i.e., tumorigenicity) of AML cells, relative to normal human bone marrow, when treated ex vivo and reimplanted into immunodeficient mice indicating activity at the level of the CSC. In addition, SL-401 prolonged the survival of mice implanted with human leukemia xenografts compared with untreated mice.

In addition, SL-401 demonstrated high potency against BPDCN cells from patients, with an IC50 in the femtomolar (10^{-15} molar) range. SL-401 has also demonstrated preclinical activity against additional CD123+ malignancies including chronic eosinophilic

leukemia, where it produced IC50 values in the low single-digit picomolar (10^{-12} molar) range. SL-401 has also shown potent in vitro anti-leukemia activity against CML tumor bulk and CML CSCs, and increased survival in mouse models of human CML taken from patients who were resistant to tyrosine kinase inhibitors, or TKIs. SL-401 has also been shown to possess a synergistic anti-CML effect when used in combination with certain TKIs. SL-401 has also demonstrated in vitro anti-tumor activity against several lymphoid cancer types, including lymphoid leukemia (e.g., T cell acute lymphoid leukemia, or T-ALL), Hodgkin's and non-Hodgkin's lymphoma, and multiple myeloma, or MM. SL-401 appears to have both a direct as well as an indirect anti-MM effect, the latter seemingly caused by SL-401's ability to target IL-3R+ hyperproliferative plasmacytoid dendritic cells, or pDC, (the cell of origin of BPDCN) that may provide a novel immune-associated growth stimulus to their neighboring MM cells. This is notable for several reasons including the drug's novel mechanism of anti-MM action as well as linking MM and BPDCN via a common cell type (pDC), and the CD123 target. SL-401 has also been shown to have a synergistic effect against MM in preclinical systems when combined with existing therapies including pomalidomide (Pomalyst®), lenalidomide (Revlimid®) and bortezomib (Velcade®). Neighboring pDCs have been reported in the microenvironment of additional tumor types including chronic myelomonocytic leukemia, or CMML.

Phase 1/2 clinical trial of SL-401 in BPDCN

SL-401 was initially evaluated in an investigator sponsored Phase 1/2 clinical trial in patients with advanced hematologic cancers; a trial which has since completed. In this trial, SL-401 administered intravenously over a single, five-day cycle demonstrated anti-tumor activity, including complete responses, or CRs, largely in BPDCN, but also in relapsed/refractory, or r/r, AML with common adverse reactions being transaminitis, thrombocytopenia, fever and chills, and capillary leak syndrome (*Frankel et al. Blood 124, 2014; ASH 2013 Poster #2682; ASCO 2013 Poster #7029; ASH 2015 Poster #3795*). We then conducted a corporate-sponsored pivotal trial of SL-401 in BPDCN.

Pivotal Phase 2 clinical trial of SL-401 in BPDCN

On October 31, 2017, we announced that the pivotal Phase 2 trial of SL-401 in BPDCN met its primary endpoint.

The pivotal Phase 2 trial of SL-401 in BPDCN was a multicenter, open label, non-randomized, single arm clinical trial. We believe this trial is the largest multicenter prospective study ever conducted in BPDCN. The trial enrolled 45 BPDCN (32 first-line, 13 relapsed/refractory) patients at 7 sites in the U.S. Patients received SL-401 dosed intravenously on days 1-5 of a 21-day cycle for multiple consecutive cycles. The trial consisted of 3 Stages: Stage 1 (lead-in, dose escalation), Stage 2 (expansion), and Stage 3 (pivotal, confirmatory). To ensure ongoing patient access to SL-401, we are currently enrolling both first-line and relapsed/refractory BPDCN patients in an additional cohort, Stage 4.

In December 2017, we presented detailed data from the pivotal trial at the 2017 American Society of Hematology, or ASH, Annual Meeting and Exposition, in Atlanta, GA. Based on results from Stage 1, which included patients with BPDCN or acute myeloid leukemia, SL-401 dosed at 12 mcg/kg/day was selected for use in subsequent Stages for BPDCN. The most common treatment-related adverse events, or TRAEs, with SL-401 at 12 mcg/kg/day in BPDCN (Stages 1, 2, and 3) (n=42) were alanine aminotransferase increase (52%), aspartate aminotransferase increase (50%), hypoalbuminemia (50%), and thrombocytopenia (38%). TRAEs included capillary leak syndrome, or CLS, (19%), which was grade 5 in 2.4% (1/42) of BPDCN patients at 12 mcg/kg/day, 2.0% (3/153) of all patients across all trial indications at all doses, and 0.8% (1/119) of patients across all trial indications at 12 mcg/kg/day. After the ASH presentation, and as reflected here, we obtained additional information that led to one fewer grade 5 CLS event than previously reported at ASH. In first-line BPDCN patients who received SL-401 at 12 mcg/kg/day, the overall response rate, or ORR, was 90% (26/29) with a 72% (21/29) rate of CR + CRc + CRi (CR = complete response; CRc = clinical complete response: absence of gross disease with minimal residual skin abnormality; CRi = CR with incomplete hematologic recovery) by investigator assessment. 45% (13/29) of these patients were bridged to stem cell transplant, or SCT, following remission on SL-401. In relapsed/refractory BPDCN patients (all of whom received SL-401 at 12 mcg/kg/day in Stages 1 and 2; n=13), there was a 69% (9/13) ORR and a 38% (5/13) CR + CRc + CRi rate.

Stage 3 of the Phase 2 trial was designed to provide the pivotal, confirmatory evidence of efficacy of SL-401 in BPDCN. In Stage 3, 13 first-line BPDCN patients were enrolled and received SL-401 at 12 mcg/kg/day. Stage 3 met its primary endpoint, with a CR + CRc rate of 54% (7/13) (95% Confidence Interval: 25.1, 80.8) by investigator assessment. The lower bound of the 95% confidence interval of the primary endpoint exceeded the pre-specified 10% rate. ORR was 77% (10/13). 46% (6/13) of patients were bridged to SCT following remission on SL-401. 86% (6/7) of complete responders were relapse-free at 5+ to 8+ months, ongoing as of the presentation.

Regulatory, biologics license application (BLA), and potential commercialization of SL-401 in BPDCN

SL-401 was granted breakthrough therapy designation, or BTD, by the FDA in August 2016. SL-401 was also awarded Orphan Drug status by the FDA for the treatment of AML in February 2011 and for BPDCN in June 2013. The European Medicines Agency, or EMA, awarded Orphan Drug status to SL-401 for the treatment of AML in September 2015 and for BPDCN in November 2015.

We believe that the results of this pivotal trial of SL-401 in BPDCN could support marketing approval, and we plan to include such results as part of a Biologics License Application, or BLA, that seeks U.S. marketing approval. We are pursuing a rolling BLA submission and believe that such a submission could be completed in the first half of 2018. If successful, we project marketing approval could be attained in the second half of 2018, or soon thereafter. Later this year, we anticipate feedback from the European Medicines Agency, or EMA, regarding a potential regulatory filing.

Stemline's commercial group continues to build out its infrastructure and optimize launch readiness within the BPDCN market. The organization is focused on preparing the market, the product, and the organization for the successful launch of SL-401 should it be approved. In December 2017, we launched our BPDCN disease awareness campaign at the American Society of Hematology, or ASH, Annual Meeting. One of the campaign's primary goals is to try to ensure that multidisciplinary healthcare professionals, including hematologist-oncologists, dermatologists, pathologists, and allied healthcare professionals are appropriately testing for CD123 to bring the diagnosis of BPDCN to the forefront and to limit misdiagnoses and underdiagnoses. The campaign highlights the importance of CD123 as a key diagnostic marker for correct patient diagnosis. Access to SL-401, should it be approved, remains a top priority within our managed care group with key success criteria identified as removing hurdles to product access and reimbursement. We are setting up a formal patient assistance program and 501(c)(3) foundation support for those that require assistance. Marketing, sales and medical affairs efforts are focused on scaling up to a "right size" staffing model with continued refinement to launch strategies and tactics for launch effectiveness.

SL-401 Clinical Trials in Additional Indications

Chronic myelomonocytic leukemia, or CMML, and Myelofibrosis, or MF

SL-401 is being assessed in a Phase 1/2 clinical trial in advanced myeloproliferative neoplasms, or MPN, focused on chronic myelomonocytic leukemia, or CMML, and myelofibrosis, or MF. This trial consists of a lead-in, dose escalation stage, 3x3 design (Stage 1) in which patients receive SL-401 as a daily intravenous infusion at 7, 9, or 12 mcg/kg/day for days 1-3 of a 21-day cycle. Stage 1 was followed by an expansion stage (Stage 2) enrolling MPN patients at the dose (12 mcg/kg/day) determined by Stage 1.

As reported at the 2017 ASH annual meeting in December 2017, 24 patients with advanced MPN (11 relapsed/refractory CMML; 12 relapsed/refractory MF) received SL-401 in Stages 1 and 2. In Stage 1, 12 mcg/kg/day was the highest tested dose for MPN, and a maximum tolerated dose, or MTD, was not reached. Stage 1 (n=9 patients) completed enrollment, and Stage 2 (n=15 patients) is ongoing. Median age was 69 years (range: 43-81); 54% were male. 71% (17/24) of patients had splenomegaly by physical examination. In Stage 1, no dose limiting toxicities, or DLT, were identified and a MTD was not reached. In Stages 1 and 2, the most common treatment-related adverse events, or TRAEs, included hypoalbuminemia (33%), thrombocytopenia (33%), and fatigue (29%). Most common TRAEs (grade 3 or higher), include thrombocytopenia (24%) and anemia (19%). Capillary leak was reported in 24% (5/21) evaluable patients: 4 cases were grades 1-2 and 1 case was grade 3.

In relapsed/refractory CMML, 71% (5/7) of patients with baseline splenomegaly had a $\geq$50% reduction in spleen size by physical examination. One relapsed/refractory CMML patient had a CR (14+ months on treatment) comprised of a bone marrow CR, or BMCR, and a 100% spleen reduction (5 to 0 cm, or not palpable).

In relapsed/refractory MF, 50% (5/10) of patients with baseline splenomegaly had spleen reductions of $\geq$25% (range: 29-100%) by physical exam, including 3 patients (30%) with spleen reductions >35%. Notably, 2 out of 3 patients had baseline thrombocytopenia: 1 patient with platelets <100K/microliter and 1 patient with platelets <50K/microliter.

We continue patient enrollment and follow-up in the trial. Based on the results to date, we believe SL-401's favorable tolerability and preliminary signs of activity support continued development and evaluation of possible registration-directed trial designs. Updates relating to this trial are expected later this year, including decisions regarding next steps for SL-401 in one or more of these indications. Factors that may impact next steps include enrollment trends, overall safety and efficacy results, regulatory paths, commercial landscape, and other medical, business, and practical considerations.

AML in complete remission with minimal residual disease, or MRD

SL-401 is being assessed in a Phase 1/2 clinical trial in AML in complete remission with minimal residual disease, or MRD. This trial consists of a lead-in, dose escalation stage, 3x3 design (Stage 1) in which patients received SL-401 as a daily intravenous infusion at

7, 9, or 12 mcg/kg/day for days 1-5 of a 21 or 28-day cycle. Stage 1 was followed by an expansion stage (Stage 2) enrolling AML patients in complete remission with MRD at the dose (12 mcg/kg/day) determined in Stage 1.

As reported at the 2017 ASH annual meeting in December 2017, 16 AML patients in complete remission with high risk of relapse including MRD received SL-401 in Stages 1 and 2, including 14 patients in first CR and two patients in second CR. In Stage 1, 12 mcg/kg/day was the highest tested dose for this trial; a maximum tolerated dose, or MTD, was not reached. Stage 1 (n=9 patients) has completed enrollment and enrollment in Stage 2 with SL-401 at 12 mcg/kg/day is ongoing. No DLTs or MTD were identified in Stage 1. The most common TRAEs in Stages 1 and 2 include hypoalbuminemia (44%), ALT increase (38%), AST increase (38%), and thrombocytopenia (38%). The most common TRAEs, grade 3 or higher, included ALT increase (31%), AST increase (25%), and thrombocytopenia (19%). Five patients were relapse-free for over 5 months (range 5+ to 14+), including 1 patient who went to stem cell transplant, or SCT, after 3+ months on SL-401, 2 patients (8+ and 14+ months on SL-401) following allogeneic SCT.

We continue patient enrollment and follow-up in the trial, including for MRD alterations and response duration. Given preclinical data indicating potential synergies between SL-401 and azacitidine in AML and high-risk MDS, and the ongoing clinical trial assessing the combination of SL-401 and azacitidine in AML, we are also considering a transition to combination therapy in this indication. Further updates are expected later this year. Factors that may impact next steps include enrollment trends, overall safety and efficacy results, regulatory paths, commercial landscape, and other medical, business, and practical considerations.

Multiple myeloma

SL-401, in combination with pomalidomide and dexamethasone, is being assessed in a Phase 1/2 clinical trial in relapsed/refractory multiple myeloma. In previous studies, plasmacytoid dendritic cells, or pDCs, the cells which when malignant become BPDCN, were found to be present in the bone marrow microenvironment of patients with multiple myeloma (Chauhan in Cancer Cell, 2009). These CD123+ pDCs were also found to possess growth-promoting interactions with their neighboring myeloma cells, as well as additional interactions with immune effector T cells and natural killer, or NK, cells in the myeloma bone marrow milieu (Ray in Leukemia, 2015). In preclinical studies, SL-401 has been shown to possess an anti-myeloma effect both directly against myeloma cells as well as indirectly via inhibition of surrounding pDCs, a potentially novel immune-associated mechanism (Chauhan in Journal of Clinical Oncology, 2013; Chauhan in Journal of Clinical Oncology, 2014; Chauhan et al. Blood 2015). SL-401 has also demonstrated synergy with several standard anti-myeloma agents, including pomalidomide, in preclinical studies of myeloma (Ray in Blood, 2014). This trial has a lead-in dose escalation stage (Stage 1) and an expansion stage (Stage 2) designed to enroll patients at the dose and regimen determined by Stage 1. The main objectives of the clinical study are to determine safety, potential signals of anti-pDC activity, and potential signals of clinical activity with SL-401 in combination with other agents. The trial is currently open to enroll patients in Stage 1, and we plan to consider the information from this trial, moving forward, to inform key elements of a combination regimen to align administration schedules with available agents. We believe that this potential anti-pDC mechanism may have ramifications not only relating to myeloma but also other indications including certain MPNs as well as some solid tumors and autoimmune diseases.

Updates relating to this trial are expected later this year, including decisions regarding next steps for SL-401 in this indication. Factors that may impact next steps include enrollment trends, overall safety and efficacy results, regulatory paths, commercial landscape, and other medical, business, and practical considerations.

Potential SL-401 Indications

Blastic plasmacytoid dendritic cell neoplasm (BPDCN)

BPDCN is an aggressive hematologic cancer that carries a poor prognosis. BPDCN had been previously classified as blastic NK cell lymphoma, agranular CD4+/CD56+ hematodermic neoplasm, and plasmacytoid dendritic cell cancer. In 2008, this disease was renamed BPDCN by the World Health Organization, or WHO, due to its derivation from plasmacytoid dendritic cells, which are specialized immune cells. BPDCN is a rare malignancy most commonly affects middle-aged and older patients and is approximately three times more common in men than women. This malignancy typically presents with skin lesions, as well as bone marrow involvement. BPDCN growth in the bone marrow results in decreased blood cell counts, which can lead to serious infections, fatigue, bleeding, and death. Although BPDCN responses have been reported with various combination chemotherapy regimens, overall prognosis remains poor. There are currently no approved therapies for BPDCN, and an optimal therapeutic regimen for BPDCN has not yet been established.

Acute myeloid leukemia (AML)

AML is a hematologic cancer characterized by dysregulated maturation of myeloid cells and failure of the bone marrow to properly function. AML is the most common type of acute leukemia in adults. Approximately 21,350 new AML cases occur annually in the United States, and approximately 27,500 new cases occur annually in Europe. The average age of an AML patient is 67 years. The

one-year survival rate for AML after first relapse is approximately 20%. Current first-line treatments for AML include chemotherapy drugs such as cytarabine in combination with an anthracycline such as daunorubicin. In certain circumstances, allogeneic stem cell transplantation is also used. In second-line AML, typical therapies include additional chemotherapy, often cytarabine again at various dosages and regimens. Despite a moderate to high proportion of patients obtaining a CR with first- and second-line chemotherapy, many of these responding patients still unfortunately have a high relapse rate and poor OS and thus are in need for additional measures for longer term benefit.

Myeloproliferative Neoplasms (MPN)

A number of other rare hematologic diseases, each qualifying as an unmet medical need, express IL-3R including certain myeloproliferative syndromes, or MPNs, such as mastocytosis, clonal eosinophilic disorders, myelofibrosis, and chronic myelomonocytic leukemia. For a majority of patients with these conditions, there is no effective, disease modifying therapy.

Chronic myelomonocytic leukemia. CMML is characterized by increased numbers of monocytes and immature blood cells (blasts) in the peripheral blood and bone marrow, as well as abnormal appearing cells (dysplasia) in at least one type of blood cell. CMML features characteristics of both MDS as well as a myeloproliferative disorder, or MPD. In the United States, the incidence of CMML is approximately 1,500 individuals per year. The median age at diagnosis of 65 to 70 years. One of the most common symptoms of CMML is splenomegaly, found in approximately half of cases. Other less frequent symptoms consist of anemia, fever, weight loss, night sweats, infection, bleeding, synovitis, lymphadenopathy, skin rashes, pleural effusion, pericardial effusion and peritoneal effusion. CMML can transform into AML in about 15%-20% of cases. Most cases are dealt with as supportive rather than curative because most therapies do not effectively increase survival. Supportive measures include blood transfusions and growth factors such as erythropoietic and granulocyte-stimulating factor. Reasons for more definitive treatment include the presence of fevers, chills, weight loss, symptomatic organ involvement, increasing blood counts, leukostasis, blood clotting, and/or progressive decreasing blood cell counts. The demethylating agents azacitidine (Vidaza®) and decitabine (Dacogen®) are FDA approved to treat CMML. High dose chemotherapy followed by bone marrow transplantation is also employed to treat CMML, and may provide long term benefit.

Myelofibrosis. Primary myelofibrosis, or PMF, is characterized by the proliferation of an abnormal clone of hematopoietic progenitor cells in the bone marrow and other sites, which results in fibrosis, or the replacement of the bone marrow with collagenous connective tissue fibers that, in turn, causes decreased blood cell counts. In the United States, the incidence of PMF is approximately 3,500 individuals per year. Median age at diagnosis is 66 years. About 30% of affected patients are less than 60 years of age. Manifestations include decreased blood cell counts, splenomegaly that is commonly painful, and increased immature white blood cells and basophils in the peripheral blood. The one known treatment of potential long-term benefit is high-dose chemotherapy followed by allogeneic stem cell transplantation. Other treatment options are largely supportive, and do not alter the course of the disorder. These options may include administration of folic acid, allopurinol, and/or blood cell transfusions. Corticosteroids, alpha-interferon and/or hydroxyurea are also used. Splenectomy is sometimes considered as a treatment option for patients with PMF in whom massive splenomegaly is contributing to anemia because of hypersplenism, particularly if there is a heavy requirement for blood transfusions. Ruxolitinib (Jakafi®) has received regulatory approval in the United States and elsewhere for the treatment for PMFs, and has been associated with symptomatic improvement and increased overall survival, but its overall benefits can be short lived. Lenalidomide (Revlimid®) and thalidomide (Thalomid®) may also be used in its treatment, although peripheral neuropathy can develop with long term use.

Multiple myeloma (MM)

MM is a hematologic malignancy that is characterized by the dysfunction of plasma cells, which are white blood cells that produce antibodies. During MM, malignant plasma cells overproduce abnormal monoclonal antibodies and can interfere with normal blood cell function in the bone marrow leading to immunodeficiency. Other common clinical manifestations of advanced MM include osteolytic bone lesions and renal disease. The bone marrow, or BM, microenvironment confers growth, survival, and drug resistance of MM cells, and it has recently been shown that plasmacytoid dendritic cells, or pDCs, which express high levels of IL-3R, are significantly increased in the BM of patients with MM and promote MM proliferation. Approximately 30,000 new cases of MM are reported annually in the United States (National Institute of Health, National Cancer Institute, 2016) and approximately 33,000 new MM cases are reported annually in Europe. The median age at diagnosis is 69 years. Patients who are transplant eligible have five-year survival rates of over 70%, while for elderly transplant-ineligible patients the rate is approximately 50%. Despite FDA approved therapies for MM, including thalidomide (Thalomid®), lenalidomide (Revlimid®), bortezomib (Velcade®), dexamethasone (Decadron®), carfilzomib (Krypolis®), pomalidomide (Pomalyst®), daratumaumab (Darzalex®), ixazomib (Ninlaro®), elotuzumab (Empliciti®), and panobinostat (Farydak®), many patients relapse from the disease.

Hairy cell leukemia (HCL)

HCL is an uncommon hematological malignancy characterized by a clonal accumulation of abnormal B lymphocytes. Approximately 1,500 new cases of HCL occur annually in the United States. The median age at diagnosis is approximately 52 years with male

predominance. Although there are FDA approved therapies for HCL, including cladribine, pentostatin, and interferon-alpha, there is no permanent cure for the disease and the relapsed/refractory setting represents an area of unmet medical need.

Myelodysplastic syndrome (MDS)

MDS is a group of hematologic malignancies characterized by dysfunction of the blood and bone marrow, resulting in decreased peripheral blood counts and, at times, evolution into AML. Approximately 16,000 new cases of MDS are reported annually in the United States and approximately 30,000 new MDS cases are reported annually in Europe. MDS occurs most commonly in males 70 years or older. Five-year survival rates for MDS patients vary significantly depending on disease severity and prognosis and range from approximately 60% for low-risk patients, to 25% for intermediate-risk patients. Virtually all high-risk MDS patients die within five years. Treatment paradigms for MDS patients vary depending on disease classification and risk category. Current first-line treatments include azacitidine (Vidaza®), decitabine (Dacogen®), lenalidomide (Thalomid®), growth factors such as erythropoietic and granulocyte-stimulating factor, chemotherapy, and stem cell transplantation in certain cases. We believe that a large number of patients either do not respond or relapse following first-line treatment, and there are no approved therapies and limited effective treatment options in this high-risk setting.

Chronic Myeloid Leukemia (CML)

Chronic myeloid leukemia, or CML, is a hematopoietic stem cell disease resulting in impaired bone marrow function. Approximately 8,950 new cases are reported annually in the United States and approximately 10,000 new cases are reported annually in Europe. The five-year OS rate for CML patients is 62%. When CML advances to an accelerated or blastic phase, the median OS is approximately one year. In patients who have failed or are intolerant to tyrosine kinase inhibitors, or TKIs, a relapsed or refractory setting, the median OS is four to 11 months. Current first-line treatments for CML include TKIs such as: imatinib (Gleevec®), nilotinib (Tasigna®), dasatinib (Sprycel®) and ponatinib (Iclusig®). In cases of relapse, second and third-line treatments include a TKI not previously used in that patient. In certain circumstances, interferon or bone marrow transplantation is also used.

Hodgkin's lymphoma (HL)

Hodgkin's lymphoma, or HL, is a cancer of the lymphatic system that commonly affects lymph nodes in the neck or the area between the lungs and behind the breastbone. Approximately 8,250 new HL cases occur annually in the United States (National Institute of Health, National Cancer Institute, 2016) and approximately 19,000 cases occur annually in Europe (World Health Organization, International Agency for Research on Cancer). The disease has four subtypes, including nodular sclerosis, lymphocyte-rich, mixed cellularity, and lymphocyte-depleted HL, all of which produce increased numbers of a unique cell type called "Reed-Sternberg" cells. These cells are considered to be the clonal tumor cells of HL and are known to express the IL-3R. Although combination chemotherapy and/or radiation therapy are effective at combating this disease, 20-30% of patients relapse after initial treatment or have primary refractory disease. Of these patients, those who do not obtain CR prior to transplantation, or who relapse after second line therapy, have few effective therapeutic options. In 2015, brentuximab vedotin (Adcentris®) received regulatory approval in the United States and elsewhere for the treatment of relapsed or refractory HL. In addition, immunotherapies are approved for third-line patients.

SL-801

SL-801 is a structurally novel, oral, small molecule, reversible inhibitor of Exportin-1, or XPO1, a nuclear transport protein implicated in a variety of malignancies. SL-801 has demonstrated preclinical in vitro and in vivo antitumor activity against a wide array of solid and hematologic cancers. SL-801's potential ability to reversibly bind XPO1 may offer the possibility to mitigate side effects and help optimize the therapeutic index. We are currently enrolling patients with advanced solid tumors in a Phase 1 dose escalation trial of single agent SL-801.

XPO1 has been shown to regulate nuclear export of many of the major tumor suppressor proteins and oncogenic cell growth regulators. Overexpression of XPO1 has been reported in many cancer types and is associated with aggressive tumor behavior and poor patient prognosis. Inhibition of XPO1 has been shown to restore tumor suppressor function and proper cell cycle regulation, leading to apoptosis of cancer cells. XPO1 has also been shown to be a clinically validated target in both solid and hematological cancers. SL-801 has demonstrated broad and potent preclinical activity in a wide array of solid and hematologic tumors in both in vitro and in vivo xenograft experiments. In a screen against 240 cancer cell lines, SL-801 possessed strong anti-tumor activity, with 50% growth inhibitory values less than 10 nM in 21.3% of cell lines and less than 100 nM in 95.8% of cell lines. As a single agent, SL-801 also significantly prolonged overall survival and inhibited tumor growth in several mouse xenograft models of human multiple myeloma, as well as in xenograft models of acute lymphoblastic leukemia, non-small cell lung cancer and prostate carcinoma, in well-tolerated single-dose or multi-dose regimens. In contrast to an earlier XPO1 inhibitor not developed by us, leptomycin B, which may bind irreversibly to XPO1 and which caused significant toxicities in Phase 1 trials, preclinical data indicate that SL-801 binding to

XPO1 is more reversible than leptomycin B. SL-801's ability to reversibly bind XPO1 offers the potential to improve the therapeutic index in humans.

In September 2017, we presented an update on the SL-801 Phase 1 trial in patients with advanced solid tumors at the European Society of Medical Oncology, or ESMO, Annual Congress 2017 in Madrid, Spain. No dose limiting toxicity, or DLT, or maximum tolerated dose, or MTD, was identified. The most common TRAEs, through six dosing cohorts, include nausea (42%), fatigue (29%), diarrhea (21%), vomiting (17%), and decreased appetite (17%). The most common TRAEs, grade 3, were nausea (4%) and diarrhea (4%). There were no TRAEs above grade 3. Through these six dosing cohorts, stable disease, or SD, was reported in 38% (9/24) of patients, with tumor shrinkages (range: 3% to 21%) noted in some heavily pre-treated patients. Dose escalation is ongoing, with the ninth dosing cohort currently enrolling.

SL-701

SL-701 is an immunotherapy designed to direct the immune system to attack targets present on certain malignancies including brain cancer. SL-701 is comprised of 3 short synthetic peptides that correspond to epitopes of targets including IL-13Rα2, EphA2, and survivin; two of these synthetic peptides (IL-13Rα2 and survivin) are mutant and believed to enhance immune activity. We completed a Phase 2 trial of SL-701 in adult patients with second-line glioblastoma, or GBM.

Several previous investigator-sponsored trials utilizing an earlier version of SL-701 demonstrated clinical activity and manageable safety in adults and children with advanced brain cancers. We subsequently conducted and completed a corporate-sponsored Phase 2 trial, which consisted of 2 stages (Stage 1 and Stage 2). In Stage 1 of this trial (n=46 patients), SL-701 was administered as a single agent, with the immunostimulants GM-CSF and Imiquimod. In Stage 2 of the trial (n=28 patients), SL-701 was administered in combination with bevacizumab, with the immunostimulant poly-ICLC. Both Stages of the trial have completed enrollment and dosing, and patients are being followed for outcomes including survival. Preliminary data were presented at the Society for Neuro-Oncology, or SNO, meeting in November 2017 demonstrating major responses and durable stable diseases with SL-701 alone and in combination with bevacizumab in second-line GBM with a manageable safety profile. In Stage 1, one patient had a partial response, or PR, of 18+ month duration (ongoing), and there were 15 stable diseases, or SD, 6 of which were at least 5 months duration (range: 5 to 28+ months, ongoing). In Stage 2, 2 patients had complete responses, or CR, and 4 patients had PRs, for an ORR of 21% (6/28). SL-701 was generally well-tolerated. The most common treatment-related adverse events, or TRAEs, were fatigue (22%) and injection site reaction (18%). In Stage 2, the median overall survival, or OS, was 11.7 months with a 48% 12-month OS rate. In addition, analyses indicate that SL-701 generated target-specific CD8+ T-cell responses in some patients experiencing clinical benefit, consistent with its mechanism of action. Data are being analyzed and will be considered when deciding next steps for the program. These steps may include conducting larger studies, including randomized studies, single arm studies, further combination studies with novel agents, (e.g., checkpoint inhibitors), that could be conducted alone or via partnerships, or cessation of the program. If additional studies are conducted, this may entail significant manufacturing campaigns and commitments around SL-701 and certain immunostimulants depending upon the choice and availability of immunostimulants.

SL-701 was awarded Orphan Drug designation from the FDA for the treatment of glioma in January 2015.

Preclinical pipeline

SL-501 and SL-101

We believe that CD123 is a rapidly emerging target in oncology with potential for broad application in hematologic cancer with promise beyond hematologic cancer in certain solid tumors and autoimmune disease. With this in mind, we are developing a platform of compounds that target CD123, led by our lead clinical stage asset, SL-401. SL-501 and SL-101 are both novel CD123-targeted therapies in preclinical development. SL-501 is a high-affinity variant of SL-401 that has shown potency, in vitro and in vivo, against several hematologic tumor types, including acute myeloid leukemia, or AML, chronic myeloid leukemia, or CML, Hodgkin's lymphoma, or HL, and Non-Hodgkin's lymphoma, or NHL. SL-101 is a single chain monoclonal antibody fragment (mAb)-conjugate that binds to CD123 and has shown in vitro and in vivo activity against a variety of hematologic cancers. In addition to oncology opportunities, we may also choose to evaluate the utility of these CD123- targeted agents in various autoimmune diseases, such as scleroderma and cutaneous lupus, in which the CD123-expressing plasmacytoid dendritic cell, or pDC, the precursor cell of BPDCN, may play a role.

SL-901

SL-901 is a small molecule kinase inhibitor. In December 2017, we in-licensed this drug candidate from UCB Biopharma Sprl, or UCB. Prior to in-licensing, the agent had shown preclinical activity in several tumors, and was evaluated in a small Phase 1 clinical trial in Europe. Neither a dose limiting toxicity, or DLT, nor a maximum tolerated dose, or MTD, was reached in the trial, and a partial response, or PR, in one patient with advanced lung cancer was reported. We are currently evaluating plans to produce drug supply under good manufacturing practice, or GMP, and conduct necessary non-clinical studies to enable a new regulatory filing to continue clinical dose escalation.

Patents and Proprietary Rights

Our intellectual property portfolio consists of 40 issued patents and 37 pending applications in the U.S. and worldwide of both in-licensed and Stemline-originated inventions.

We continually assess our intellectual property strategy in order to fortify our position in our market space. To that end, we are prepared to file additional patent applications in any of the above families should our intellectual property strategy require such filings and/or where we seek to adapt to competition or seize business opportunities. Further, we are prepared to file patent applications relating to the other products in our pipeline soon after the experimental data necessary for a strong application become available and our cost-benefit analyses justify filing such applications.

In addition to filing and prosecuting patent applications in the United States, we typically file counterpart patent applications in Europe, Canada, Japan, Australia, and additional countries where we think such foreign filing is likely to be beneficial.

We do not know if patents will be issued for all of the patent applications in our portfolio, and there is no assurance that they will be. Furthermore, for patent claims now issued and for claims to be issued in the future, we do not know if such claims will provide significant proprietary protection to our drug candidates and proprietary technologies or if they will be challenged, circumvented, or invalidated. Our success will in part depend on our ability to obtain and maintain patents protecting our drug candidates, technologies and inventions, to operate without infringing the proprietary rights of third-parties, and to enforce and defend our patents and ensure others do not infringe on our proprietary rights.

The term of individual patents depends upon the legal term of the patents in the countries in which they are obtained. In most countries in which we file, the patent term is 20 years from the earliest date of filing a non-provisional patent application. In the United States, a patent's term may be shortened if a patent is terminally disclaimed over another patent or as a result of delays in patent prosecution by the patentee, and a patent's term may be lengthened by patent term adjustment, which compensates a patentee for administrative delays by the U.S. Patent and Trademark Office in granting a patent.

The term of a patent that covers an FDA-approved drug or biologic may also be eligible for patent term extension, which permits patent term restoration as compensation for the patent term lost during the FDA regulatory review process. The Drug Price Competition and Patent Term Restoration Act of 1984, or the Hatch-Waxman Act, permits a patent term extension of up to five years beyond the expiration of the patent. The length of the patent term extension is related to the length of time the drug or biologic is under regulatory review. Patent extension cannot extend the remaining term of a patent beyond a total of 14 years from the date of product approval and only one patent applicable to an approved drug or biologic may be extended. Similar provisions are available in Europe and other foreign jurisdictions to extend the term of a patent that covers an approved drug or biologic. In the future, if and when our pharmaceutical products receive FDA approval, we expect to apply for patent term extensions on patents covering those products. We anticipate that some of our issued patents may be eligible for patent term extensions. For more information regarding U.S. patent laws, see "Business — Government Regulation."

In addition to the patent term extension rights described above, any of our product candidates that receive FDA approval may also be eligible for market exclusivity protection under the Federal Food, Drug and Cosmetic Act or the Biologics Price Competition and Innovation Act of 2009. For more information regarding market exclusivity laws, see "Business — Government Regulation."

Many pharmaceutical companies, biotechnology companies and academic institutions are competing with us in the field of oncology and filing patent applications potentially relevant to our business. In order to contend with the inevitable possibility of third-party intellectual property conflicts, from time to time, we review and assess the third-party intellectual property landscape for competitive and other developments that may inform or impact our intellectual property development and commercialization strategies.

From time to time, we may find it necessary or prudent to obtain licenses from third-party intellectual property holders. Where licenses are readily available at reasonable cost, such licenses are considered a normal cost of doing business. In other instances, however, where a third-party holds relevant intellectual property and is a direct competitor, a license might not be available on

commercially reasonable terms or available at all. Accordingly, we attempt to manage the risk that such third-party intellectual property may pose by conducting, among other measures, freedom-to-operate studies to guide our early-stage research away from areas where we are likely to encounter obstacles in the form of third-party intellectual property. As our programs advance, we continue to monitor the intellectual property landscape in an effort to assess the advisability of licensing third-party intellectual property or taking other appropriate steps to address such freedom-to-operate or development issues in the manner we deem in the best interests of the Company.

With respect to third-party intellectual property, it is impossible to establish with certainty that our product candidates or discovery platform will be free of claims by third-party intellectual property holders or whether we will require licenses from such third-parties. Even with modern databases and on-line search engines, literature searches are imperfect and may fail to identify relevant patents and published applications. Even when a third-party patent is identified, we may conclude, upon a thorough analysis, that we do not infringe the patent or that the patent is invalid. If the third-party patent owner disagrees with our conclusion and we continue with the business activity in question, we might face patent litigation by the third-party. Alternatively, we might decide to initiate litigation in an attempt to have a court declare the third-party patent invalid or not infringed by our activity. In either scenario, patent litigation typically is costly and time-consuming, and the outcome is uncertain. The outcome of patent litigation is subject to uncertainties that cannot be quantified in advance, for example, the credibility of expert witnesses who may disagree on technical interpretation of scientific data. Ultimately, in the case of an adverse outcome in litigation, we could be prevented from commercializing a product or using certain aspects of our discovery platform as a result of patent infringement claims asserted against us and/or face a significant monetary damages award. This could have a material adverse effect on our business.

To protect our competitive position, it may be necessary to enforce our patent rights through litigation against infringing third-parties. Litigation to enforce our own patent rights is subject to the same uncertainties discussed above. In addition, however, litigation involving our patents carries the risk that one or more of our patents will be held invalid (in whole or in part, on a claim-by-claim basis) or held unenforceable. Such an adverse court ruling could allow third-parties to commercialize our products or our platform technology, and then compete directly with us, without payment to us.

Patents and Proprietary Rights Covering Stemline's Drug Candidates

We have an exclusive worldwide license to SL-401. These patent rights include issued U.S. Patents, 7,763,242, 8,470,307, 9,181,317, and 9,631,006 covering methods of treating AML, BPDCN and MDS that expire in 2027 and 2028, as applicable, as well as six issued foreign patents. There are additional pending U.S. applications directed to methods of using SL-401 to treat other diseases that, if issued, would also expire in 2027. In addition, we have filed foreign patent applications for the method of using SL- 401 to treat various diseases, although there can be no assurances that such patents will be issued. In addition to patent protection, we also have the exclusivity afforded by the FDA's orphan designation of SL-401 for the treatment of both AML and BPDCN and by the provisions of the Biologics Price Competition and Innovation Act of 2009. See "Government Regulation — Orphan Drug Designation" and "— U.S. Patent Term Restoration and Marketing Exclusivity—Biologics Price Competition and Innovation Act of 2009."

We have an exclusive worldwide license (with the exception of Japan, Korea, Taiwan, and China) to patents covering SL-801. These patent rights include issued U.S. Patents 8,084,454 and U.S. Patent 8,415,357 covering composition of matter and uses of SL-801 that expire in 2030 and 2028, respectively, as well as nine issued foreign patents that expire in 2028. We also have additional pending patent applications directed to SL-801 which if issued, for which there can be no guarantee, would provide additional protection in certain non-U.S. territories and would expire in 2028.

We have an exclusive worldwide license to patents covering the SL-701 component, IL-13Rα2 mutant, a non-exclusive worldwide license to patents covering the SL-701 component, EphA2, and have filed U.S. and foreign patent applications covering the SL-701 component, survivin mutant. This intellectual property consists of an issued U.S. composition of matter patent (U.S. Patent 7,612,162) directed to an immunogenic mutant IL-13Rα2 peptide expiring in 2026, an issued U.S. composition of matter patent (U.S. Patent 8,574,584) directed to an immunogenic EphA2 peptide expiring in 2024, issued U.S. method of use patents (U.S. Patents 8,114,407 and 9,359,402) directed to the use of EphA2 peptide expiring in 2024 and 2025, issued U.S. method of use patent (U.S. Patent 8,859,488) directed to the combined use of IL-13Rα2 mutant and EphA2 peptides expiring in 2026, and pending U.S. and foreign patent applications directed to the use of an immunogenic mutant survivin peptide which, to the extent it issues, would be expected to expire in 2033. We also have additional pending patent applications directed to methods of using SL-701 components to treat certain diseases which if issued, for which there can be no guarantee, would provide additional protection in the United States and certain non-U.S. territories and would expire in 2025, 2031, or 2033. In addition to patent protection, we also have the exclusivity afforded by the FDA's orphan designation of SL-701 for the treatment of glioma and by the provisions of the Biologics Price Competition and Innovation Act of 2009. See "Government Regulation — Orphan Drug Designation" and "— U.S. Patent Term Restoration and Marketing Exclusivity—Biologics Price Competition and Innovation Act of 2009".

We also in-licensed or own certain patent rights, which includes issued patents and pending patent applications in the U.S. and abroad, to our preclinical assets.

Patents and Proprietary Rights Covering Cancer Stem Cell, or CSC, Focused Intellectual Property

We have exclusive worldwide rights to early and broad patents and patent applications in the CSC field covering CSC therapeutics, diagnostics, including companion diagnostics, and drug discovery:

- One unexpired patent (U.S. Patent 8,038,998) that covers a method to treat cancer through use of monoclonal antibodies and other antibody-based compounds that target CSCs. Patent protection extends to 2020;
- Two issued patents (U.S. Patents 8,715,945 and 8,846,325) that cover methods to detectcancer through use of monoclonal antibodies and other antibody-based compounds directed to CD44, Frizzled, and, ESA. Patent protection extends to 2018or 2019, as applicable;
- Two pending U.S. patent applications filed in 2007 directed to CSC-directed therapies and regimens, including CSC-directed therapies and regimens for use in combination with companion diagnostics. Patent protection, to the extent it issues, would be expected to extend to 2027;
- A pending patent application that covers oligonucleotide-based oncology therapies, including CSC-targeted therapeutics, which target microRNA. Patent protection, to the extent it issues, would be expected to extend to 2022;
- A family of intellectual property covering methods to treat cancer through use of antibody-based compounds directed to IL-3Rα as well as composition of matter covering IL-3Rα-targeted antibody conjugates, including U.S. Patent 6,733,743; U.S. Patent 7,651,678; U.S. Patent 8,163,279; U.S. Patent 8,852,551; U.S. Patent 8,992,910; U.S. Patent 9,518,119; U.S. Patent 9,873,743; and other pending applications. Patent protection, to the extent it has or may issue, would be expected to extend to 2021 or 2028, as applicable; and
- A pending U.S. patent application covering CSC-focused drug discovery, including a novel high throughput screen to discover compounds that target CSCs. Patent protection, to the extent it issues, would be expected to extend to 2025.

License and Research Agreements

Scott and White Memorial Hospital

Research and License Agreement (SL-401)

In June 2006, we entered into a research and license agreement with Scott and White Memorial Hospital (Temple, Texas) for SL-401, our biologic targeted therapy directed to the IL-3R. Under the agreement, Scott and White has granted us an exclusive, royalty-bearing, worldwide license under certain patent rights, know-how and materials to research, develop, make, have made, formulate, use, sell, offer to sell and import SL-401, and any products containing or comprising such compound in finished dosage pharmaceutical form, for the diagnosis, prophylaxis and/or treatment of any disease or condition in humans or animals. The patent rights exclusively licensed to us under the agreement are described in more detail above under "Business — Patents and Proprietary Rights."

We must pay Scott and White royalties based on adjusted gross sales, by us or our sublicensees, of products containing the licensed compound for a period of ten years following the first commercial sale of each product in each country. The royalty rates for each product range from the low- to mid-single digits and are tiered based on our annual sales. We have sublicensing rights under the agreement, subject to our paying to Scott and White a percentage of the up-front payments we receive from a sublicensee.

We must exercise commercially reasonable efforts to develop and commercialize a licensed product and to achieve certain regulatory milestones within certain periods, subject to extensions based on unforeseen technical, scientific, intellectual property or regulatory issues. If we fail to comply with our diligence obligations with respect to at least one licensed product, then Scott and White may convert our exclusive license to a non-exclusive license.

The agreement survives until the later of the expiration of the last to expire licensed patent or the date on which we owe no further payments to Scott and White, after which our license becomes fully paid, irrevocable, perpetual, non-exclusive and royalty-free. We may terminate the license in whole or on a country-by-country and product-by-product basis upon prior written notice to Scott and

White. If either we or Scott and White breach a material obligation under the agreement, and such obligation is not cured within a specified period of time following written notice from the other party, then the non-breaching party may terminate the agreement upon an additional written notice.

In addition, the agreement provides for Scott and White to conduct a research program with SL-401. In March 2010, the agreement was amended to further the regulatory advancement of SL-401. We have made certain payments to Scott and White for such research services pursuant to the agreement, which to date total approximately $1.0 million in the aggregate. Additionally, we have been granted the exclusive right of reference to its IND for our own regulatory filings. We may assign the agreement to an affiliate of ours, a purchaser of all or substantially all of our assets or in connection with a merger, change in control or similar transaction by us.

CanBas, Ltd

License for SL-801

On December 26, 2014, we entered into a license agreement with CanBas, Ltd. for SL-801. SL-801 is a small molecule, reversible inhibitor of XPO1. Under the terms of the agreement, CanBas has granted us an exclusive, royalty-bearing, worldwide (excluding Japan, Korea, China and Taiwan) license, under certain patent rights, know-how and materials to research, develop, make, have made, formulate, use, sell, offer to sell and import SL-801, and any products containing or comprising such compound in finished dosage pharmaceutical form, for the treatment of any disease or condition in humans. The patent rights exclusively licensed to us under the agreement are described in more detail above under "Patents and Proprietary Rights Covering Stemline's Drug Candidates."

We are responsible to pay an annual technical advisory fee in December 2018 totaling 110 million Japanese Yen (JPY), if the clinical development continues over this time period. Additionally, we must pay CanBas tiered royalties based on aggregate net sales, by us or our sublicensees, of products containing the licensed compound until the latest date of a period of ten years following the first commercial sale of each product in each country; the date upon which there are no more valid claims or the expiration or termination of the last regulatory exclusivity period. The royalty rates start in the low single digits and are tiered up based on annual net sales. In the future, we may also be responsible, based on the achievement of specific clinical-development, regulatory and sales-based commercial milestones, for certain payments to CanBas of up to $86 million. We have sublicensing rights under the agreement, subject to our paying to CanBas a standard royalty percentage of the payments we receive from a sublicensee.

We must exercise commercially reasonable efforts to develop and commercialize a licensed product and to achieve certain regulatory milestones within certain periods, subject to extensions based on unforeseen technical, scientific, intellectual property or regulatory issues.

The agreement survives until the later of ten years following the first commercial sale of each product in each country; the date upon which there are no more valid claims; or the expiration or termination of the last regulatory exclusivity period, after which our license becomes fully paid, irrevocable, perpetual, non-exclusive and royalty-free. We may terminate the license for any or no reason upon 60 days advance written notice to CanBas. If either we or CanBas breach a material obligation under the agreement, and such obligation is not cured within a specified period of time following written notice from the other party, then the non-breaching party may terminate the agreement upon an additional written notice.

University of Pittsburgh

Exclusive License Agreement to IL-13Rα2 peptide (SL-701 component)

In September 2009, we entered into an exclusive license agreement with the University of Pittsburgh, or the University, for the composition of matter, and use with other components, of a proprietary immunogenic mutant analog peptide of IL-13Rα2, an active ingredient of SL-701, our brain cancer immunotherapy candidate. Under the agreement, the University grants us an exclusive worldwide license under certain patent rights to make, have made, use, sell and import brain cancer peptide antigen immunotherapies (including SL-701, which has been developed by the University under a separate immunotherapy name designated by the University). The patent rights exclusively licensed to us under the agreement are described in more detail above under "Business — Patents and Proprietary Rights." The University retains the right to practice the licensed patent rights for non-commercial education and research purposes. The license is also subject to certain retained rights of the United States government. Our right to grant sublicenses to third parties is subject to the prior written approval of the University, which the University may not unreasonably withhold or delay.

We paid the University an initial license fee and will pay the University annual license maintenance fees until the first commercial sale of a licensed product. To date, we have paid an aggregate of approximately $0.7 million in fees to the University under the agreement. We must also pay the University a low-single digit royalty as a percentage of net sales of licensed products by us or our sublicensees, with standard provisions for royalty offsets to the extent we need to obtain any rights from third-parties to commercialize

the licensed products. We must also pay a minimum annual royalty following the first commercial sale of a licensed product, but only to the extent the minimum annual royalty amount is greater than the annual royalty otherwise due. We also must pay the University a percentage of non-royalty revenue we receive from our sublicensees, which decreases if we enter into the applicable sublicense agreement after a certain clinical milestone has been met. We also must make certain payments to the University of up to approximately $4.2 million upon the achievement of specific regulatory and commercial milestone events.

We must use our commercially reasonable best efforts to develop or commercialize a licensed product as soon as practicable, and to continue active, diligent marketing efforts throughout the term of the agreement. We also must adhere to certain specific regulatory milestones with respect to initiating clinical trials and submitting an application for regulatory approval of a licensed product. If we fail to meet any such milestone through no fault of our own, we may negotiate with the University a one-time extension of the applicable dates, subject to paying the University a fee. If we do not meet the extended milestone dates, then the University may terminate the agreement.

The agreement survives until the expiration of the last to expire licensed patent. The University may terminate the agreement if we default in the performance of any of our obligations and do not cure the default within a specified period of time after receiving notice from the University, or if we challenge the validity, enforceability or ownership of the license patent rights anywhere in the world. The University may also terminate the agreement if we cease to carry out our business or become bankrupt or insolvent. We may terminate the agreement for any reason upon prior written notice to the University and payment of all amounts due to the University through the date of termination. Any sublicense agreement entered into prior to termination will survive, subject to certain customary conditions. We may assign the agreement to an affiliate of ours, a purchaser of all or substantially all of our assets or in connection with a merger, change in control or similar transaction by us.

Non-Exclusive License Agreement to EphA2 peptide (SL-701 component)

In March 2012, we entered into a non-exclusive license agreement with the University for the use of EphA2 epitopes, another active ingredient of SL-701. Under the agreement, the University grants us a non-exclusive worldwide license under certain patent rights to use the EphA2 peptide in or packaged with the IL-13Rα2 peptide, as well as other immunotherapies we may develop and own or exclusively control, for the diagnosis, treatment or prevention of diseases and tumors of the brain in human patients. The patent rights licensed to us under the agreement are described in more detail above under "Business — Patents and Proprietary Rights." The University retains the right to practice the licensed patent rights for non-commercial education and research purposes. The license grant is also subject to certain retained rights of the United States government. We may only grant sublicenses to third parties who are permitted sublicensees under the exclusive IL-13Rα2 peptide license agreement with the University.

We must pay the University an initial license fee, and will pay the University annual license maintenance fees until the net sales of a licensed product exceed a specified amount. To date, we have paid an aggregate of approximately $0.1 million in fees to the University under the agreement. We must also pay the University a customary low-single digit royalty for the license as a percentage of net sales of licensed products by us or our sublicensees, with standard provisions for royalty offsets to the extent we need to obtain any rights from third-parties to commercialize the licensed products. We must also pay a minimum annual royalty following the first commercial sale of a licensed product, but only to the extent the minimum annual royalty amount is greater than the annual royalty otherwise due.

We must use our commercially reasonable best efforts to develop or commercialize a licensed product as soon as practicable, and to continue active, diligent marketing efforts throughout the term of the agreement. We also must adhere to certain specific regulatory milestones with respect to initiating clinical trials and submitting an application for regulatory approval of a licensed product. If we fail to meet any such milestone by certain specified dates, then the University may terminate the agreement.

The agreement survives until the expiration of the last to expire licensed patent. The University may terminate the agreement if we default in the performance of any of our obligations and do not cure the default within a specified time period of receiving notice from the University. The University may also terminate the agreement if we cease to carry out our business or become bankrupt or insolvent. We may terminate the agreement for any reason upon prior written notice to the University and payment of all amounts due to the University through the date of termination. Any sublicense agreement entered into prior to termination will survive, subject to certain customary conditions. We may assign the agreement to an affiliate of ours, a purchaser of all or substantially all of our assets or in connection with a merger, change in control or similar transaction by us.

Non-Exclusive License Agreement to use and reference certain data, information and regulatory filings (SL-701)

In March 2012, we entered into a non-exclusive license agreement with the University. Pursuant to the agreement, we acquired a non-exclusive, worldwide license to use and reference certain know-how, information and data that is contained in the INDs covering the clinical trials of SL-701 that were conducted by the University for the development, manufacture, regulatory approval and

commercialization of pharmaceutical products. We may grant sublicenses in conjunction with a sublicense to a permitted sublicensee under the exclusive IL-13Rα2 peptide license agreement with the University.

We paid the University an initial license fee, as well as payments following a regulatory milestone. To date, we have paid an aggregate of approximately $27,500 in fees to the University under the agreement. We also must pay the University a percentage of non-royalty revenue we receive from our sublicensees. We must use our commercially reasonable best efforts to develop or commercialize a product derived from the use of the licensed data or information as soon as practicable. We also must adhere to a specific regulatory milestone with respect to submitting an application for regulatory approval that incorporates the licensed data or information, and if we fail to meet the milestone, the University may terminate the agreement unless we have pre-paid the milestone payment listed above.

The term of the license agreement is 20 years, and the University may terminate the agreement earlier (i) if we default in the performance of any of our obligations and do not cure the default within a specified time period, (ii) upon the termination of the exclusive IL-13Rα2 peptide license agreement with the University, or (iii) if we cease to carry out our business or become bankrupt or insolvent. We may terminate the agreement at any time prior to incorporating or referencing the data or University INDs, after a specified number of days following written notice. We may assign the agreement to an affiliate of ours, a purchaser of all or substantially all of our assets or in connection with a merger, change in control or similar transaction by us.

Cambridge University Technical Services Limited

Exclusive Patent and Non-Exclusive Know-How License Agreement (Platform Technology)

In September 2004, we entered into a license agreement with Cambridge University Technical Services Limited, or CUTS, relating to our StemScreen® platform technology. Under the agreement, we acquired an exclusive, royalty-bearing, worldwide license under patent rights owned by CUTS to develop, manufacture, have manufactured, use, sell, offer to sell, market, have marketed, import, have imported, export and have exported products covered by the patent rights, including a platform technology to discover and screen for compounds that target CSCs. The patent rights exclusively licensed to us under the agreement are described in more detail above under "Business — Patents and Proprietary Rights." The license is subject to certain rights retained by CUTS for academic research and teaching. We also acquired a non-exclusive, worldwide license to know-how related to the licensed patent rights. The agreement provides us with full sublicensing rights. Under the agreement, we paid an upfront license fee and are obligated to make milestone payments of up to an aggregate of $1.7 million upon specified regulatory events, as well as pay royalties of less than 1% on sales of licensed products. CUTS may terminate the agreement, including our rights to the platform technology, for specified cause or upon certain events involving our bankruptcy or insolvency.

Competition

The biotechnology and pharmaceutical industries are characterized by rapidly advancing technologies, intense competition and a strong emphasis on proprietary products. Additionally, there has been an increase in development of therapeutics targeting ultra orphan and rare oncologic indications, our main area of focus. While we believe that our scientific knowledge, technology, and development experience provide us with competitive advantages, we face potential competition from many different sources, including major pharmaceutical, specialty pharmaceutical and biotechnology companies, academic institutions, governmental agencies and public and private research institutions. Any product candidates that we successfully develop and commercialize will compete with existing therapies and new therapies that may become available in the future.

There are several biopharmaceutical companies whose primary focus appears to be developing therapies against CSCs, including Verastem, Inc., OncoMed Pharmaceuticals, Inc., Sumitomo Dainippon Pharma Co. Ltd., Bionomics Limited and Stemcentrx, Inc. (an AbbVie, Inc., company). There are also several biopharmaceutical companies that do not appear to be primarily focused on CSCs, but may be developing at least one CSC-directed compound. These companies include Astellas Pharma US, Inc., Boehringer Ingelheim GmbH, Geron Corp., GlaxoSmithKline plc, Ignyta, Inc., Macrogenics Inc., Micromet, Inc. (an Amgen, Inc. company), Pfizer Inc., Roche Holding AG, Sanofi U.S. LLC, and others. Additionally, there are a number of companies working to develop new treatments for hematologic cancers, which may compete with SL-401 and SL-801, including AbbVie, Inc., Ambit Biosciences Corporation (a Daiichi Sankyo company), Amgen, Inc., Astex Pharmaceuticals (now an Otsuka Pharmaceutical company), Celator Pharmaceuticals, Inc., Celgene Corporation, Cellectis, Cyclacel Pharmaceuticals, Inc., Eisai Co. Ltd., Genzyme Corporation (a Sanofi company), Immunogen, Inc., Janssen Pharmaceutical Companies of Johnson and Johnson, Karyopharm Therapeutics, Inc., Novartis AG, Seattle Genetics, Inc., and Sunesis Pharmaceuticals, Inc., among others. There are also a number of drugs used for the treatment of brain cancer that may compete with SL-701, including, Avastin® (Roche Holding AG), Gliadel® (Eisai Co. Ltd.), and Temodar® (Merck & Co., Inc.). There are a number of companies working to develop brain cancer therapeutics with programs in clinical testing, including Agenus Inc., Bristol-Myers Squibb, Inc., Cortice Biosciences, Inc., Celldex Therapeutics, Inc., CytRx Corporation,

GenSpera, Inc., GlaxoSmithKline plc., ImmunoCellular Therapeutics, Ltd., Northwest Biotherapeutics, Inc., Novartis AG, Roche Holding AG and others.

Many of our competitors have significantly greater financial resources and expertise in research and development, manufacturing, preclinical testing, conducting clinical trials, obtaining regulatory approvals and marketing approved products than we do. Mergers and acquisitions in the pharmaceutical, biotechnology and diagnostic industries may result in even more resources being concentrated among a smaller number of our competitors. These competitors also compete with us in recruiting and retaining qualified scientific and management personnel and establishing clinical trial sites and patient registration for clinical trials, as well as in acquiring technologies complementary to, or necessary for, our programs. Small or early-stage companies may also prove to be significant competitors, particularly through collaborative arrangements with large and established companies.

The key competitive factors affecting the success of all of our product candidates, if approved, are likely to be their efficacy, safety, convenience, price, the level of generic competition and the availability of reimbursement from government and other third-party payors.

Our commercial opportunity could be reduced or eliminated if our competitors develop and commercialize products that are safer, more effective, have fewer or less severe side effects, are more convenient or are less expensive than any products that we may develop. Our competitors also may obtain FDA or other regulatory approval for their products more rapidly than we may obtain approval for ours, which could result in our competitors establishing a strong market position before we are able to enter the market. Also, if our competitors receive marketing approval for a product for which it has an orphan designation, we may not be able to receive marketing approval for one of our products for the same indication unless it demonstrates clinical superiority to such product. Our ability to compete may be affected in many cases by insurers or other third-party payors seeking to encourage the use of generic products. If our therapeutic product candidates are approved, we expect that they will be priced at a significant premium over any competitive generic products.

The most common methods of treating patients with cancer are surgery, radiation and drug therapy, including chemotherapy, hormone therapy and targeted drug therapy. These therapies are numerous and varied in their design, therapeutic application and mechanism of action. As a result, they may provide significant competition for any of our product candidates for which we obtain market approval. In addition to currently marketed oncology therapies, there are also a number of products in late stage clinical development to treat cancer. These products in development may provide efficacy, safety, convenience and other benefits that are not provided by currently marketed therapies. As a result, they may provide significant competition for any of our product candidates for which we obtain market approval.

Competition for SL-401

There are a number of companies working to develop new treatments for AML and other hematologic cancers, including Agios, Inc., Ambit Biosciences Corporation (now a Daiichi Sankyo company), Astex Pharmaceuticals (an Otsuka Pharmaceutical company), Boehringer Ingelheim, Celator Pharmaceuticals, Inc. (a Jazz Pharmaceuticals company), Celgene Corporation, Cellectis, Cyclacel Pharmaceuticals, Inc., Eisai Co. Ltd., Epizyme, Inc., Genzyme Corporation (a Sanofi company), Immunogen, Inc., Janssen Pharmaceutical Companies of Johnson and Johnson, Seattle Genetics, Inc., and Sunesis Pharmaceuticals, Inc., among others.

Competition for SL-801

Karyopharm Therapeutics is the only company, to our knowledge, that currently has XPO1 inhibitors in clinical development. Karyopharm's selinexor is being evaluated in a number of clinical trials in both solid and hematologic cancers, with the most advanced clinical programs in AML, multiple myeloma and diffuse large B-Cell lymphoma, or DLBCL. Karyopharm has also advanced a second generation compound, KPT-8602, into clinical trials in relapsed/refractory multiple myeloma.

Competition for SL-701

There are a limited number of drugs used for the treatment of brain cancer, including Temodar® (Merck & Co., Inc.), nitrosoureas including Gliadel® (Eisai Co., Inc.), and Avastin® (Roche Holding AG). There are a number of companies working to develop brain cancer therapeutics with programs in clinical testing including Agenus Inc., Bristol-Myers Squibb, Inc., Cortice Biosciences, Inc., Celldex Therapeutics, Inc., CytRx Corporation, GenSpera, Inc., GlaxoSmithKline plc., ImmunoCellular Therapeutics, Ltd., Northwest Biotherapeutics, Inc., Novartis AG, Roche Holding AG and others.

Government Regulation

Government authorities in the United States, at the federal, state and local level, and in other countries extensively regulate, among other things, the research, development, approval, manufacture, testing, quality control, packaging, labeling, storage, record-keeping, promotion, advertising, distribution, post-approval monitoring and reporting, marketing and export and import of products such as those we are developing. Any pharmaceutical candidate that we develop must be approved by the FDA before it may be legally marketed in the United States or by the appropriate foreign regulatory agency before it may be legally marketed in foreign countries.

United States Drug Development Process

In the United States, the FDA regulates drugs under the Federal Food, Drug and Cosmetic Act, or FDCA, and implementing regulations. Drugs are also subject to other federal, state and local statutes and regulations. Biologics are subject to regulation by the FDA under the FDCA, the Public Health Service Act, or the PHSA, and related regulations, and other federal, state and local statutes and regulations. Biological products include, among other things, viruses, therapeutic serums, vaccines and most protein products. The process of obtaining regulatory approvals and the subsequent compliance with appropriate federal, state, local and foreign statutes and regulations require the expenditure of substantial time and financial resources. Failure to comply with the applicable United States requirements at any time during the product development process, approval process or after approval, may subject an applicant to administrative or judicial sanctions. FDA sanctions could include a clinical hold, refusal to approve pending applications, warning or untitled letters, product recalls, product seizures, total or partial suspension of production or distribution, injunctions, fines, refusals of government contracts, restitution, disgorgement of profits, civil or criminal penalties, or withdrawal of an approval. Any administrative action or judicial enforcement action could have a material adverse effect on us.

The process required by the FDA before a drug or biological product may be marketed in the United States generally involves the following:

- Completion of preclinical laboratory tests, animal studies and formulation studies according to Good Laboratory Practices or other applicable regulations;
- Submission to the FDA of an IND which FDA must clear before human clinical trials may begin;
- Performance of adequate and well-controlled human clinical trials according to the FDA's current Good Clinical Practices, or cGCPs, and in accordance with human subject protection regulations, to establish the safety and efficacy of the proposed drug or biologic for its intended use;
- Submission to the FDA of a NDA for a new drug product, or a BLA for a new biological product;
- Satisfactory completion of an FDA pre-approval inspection of the manufacturing facility or facilities where the drug or biologic is to be produced to assess compliance with the FDA's current good manufacturing practice regulations, or cGMPs, to assure that the facilities, methods and controls are adequate to preserve the drug's or biologic's identity, strength, quality and purity;
- Potential FDA inspection of the nonclinical and clinical trial sites that generated the data in support of the NDA or BLA; and
- FDA review and approval of the NDA or BLA and successful resolution of any questions that arise in the review process.

The lengthy process of seeking required approvals and the continuing need for compliance with applicable statutes and regulations post-approval require the expenditure of substantial resources. There can be no certainty that approvals will be granted.

Before testing any compounds with potential therapeutic value in humans, the drug or biological candidate enters the preclinical testing stage. Preclinical tests include laboratory evaluations of product chemistry, toxicity and formulation, as well as animal studies to assess the potential safety and activity of the drug or biological candidate. The conduct of the preclinical tests must comply with federal regulations and requirements including good laboratory practices. The sponsor must submit the results of the preclinical tests, together with manufacturing information, analytical data, any available clinical data or literature and a proposed clinical protocol, to the FDA as part of the IND. The IND automatically becomes effective 30 days after receipt by the FDA, unless the FDA places the clinical trial on a clinical hold within that 30-day time period. In such a case, the IND sponsor and the FDA must resolve any outstanding concerns before the clinical trial can begin. The FDA may also impose clinical holds on a drug or biological candidate at

any time before or during clinical trials due to safety concerns or non-compliance. Accordingly, we cannot assure that submission of an IND will result in the FDA allowing clinical trials to begin, or that, once begun, issues will not arise that suspend or terminate such trial.

Clinical trials involve the administration of the drug or biological candidate to healthy volunteers or patients having the disease being studied under the supervision of qualified investigators; often these are physicians not employed by or under the trial sponsor's control. Clinical trials are conducted under protocols detailing, among other things, the objectives of the clinical trial, dosing procedures, subject selection and exclusion criteria, and the parameters to be used to monitor subject safety. Each protocol must be submitted to the FDA as part of the IND. Clinical trials must be conducted in accordance with the FDA's good clinical practices requirements, regulations for the protection of human subjects and with applicable cGMP requirements. Further, each clinical trial must be reviewed and approved by an institutional review board, or IRB, at or servicing each institution at which the clinical trial will be conducted. An IRB is charged with protecting the welfare and rights of trial participants and considers such items as whether the risks to individuals participating in the clinical trials are minimized and are reasonable in relation to anticipated benefits. The IRB also approves trial recruitment materials and the informed consent form that must be used as part of the informed consent process with each clinical trial subject or his or her legal representative and must monitor the clinical trial until it is completed.

Human clinical trials prior to approval are typically conducted in three sequential Phases that may overlap or be combined:

- *Phase 1.* The drug or biologic is initially introduced into healthy human subjects and tested for safety, dosage tolerance, absorption, metabolism, distribution and excretion. In the case of some products for severe or life-threatening diseases, especially when the product may be too inherently toxic to ethically administer to healthy volunteers, the initial human testing is often conducted in patients having the specific disease.

- *Phase 2.* The drug or biologic is evaluated in a limited patient population to identify possible adverse effects and safety risks, to preliminarily evaluate the efficacy of the product for specific targeted diseases and to determine dosage tolerance, optimal dosage and dosing schedule for patients having the specific disease.

- *Phase 3.* Clinical trials are undertaken to further evaluate dosage, clinical efficacy and safety in an expanded patient population at geographically dispersed clinical trial sites. These clinical trials, which usually involve more subjects than earlier trials, are intended to establish the overall risk/benefit ratio of the product and provide an adequate basis for product labeling. Generally, at least two adequate and well-controlled Phase 3 clinical trials are required by the FDA for approval of an NDA or BLA.

Post-approval studies, or Phase 4 clinical trials, may be conducted after initial marketing approval. These studies are used to gain additional experience from the treatment of patients in the intended therapeutic indication and may be required by the FDA as part of the approval process.

Progress reports detailing the results of the clinical trials must be submitted at least annually to the FDA and written IND safety reports must be submitted to the FDA by the investigators for serious and unexpected adverse events or any finding from tests in laboratory animals that suggests a significant risk for human subjects. Phase 1, Phase 2 and Phase 3 clinical trials may not be completed successfully within any specified period, if at all. The FDA or the sponsor or its data safety monitoring board may suspend a clinical trial at any time on various grounds, including a finding that the research subjects or patients are being exposed to an unacceptable health risk. Similarly, an IRB can suspend or terminate approval of a clinical trial at its institution if the clinical trial is not being conducted in accordance with the IRB's requirements or if the drug or biologic has been associated with unexpected serious harm to patients.

Concurrent with clinical trials, companies usually complete additional animal studies and develop additional information about the chemistry and physical characteristics of the drug or biologic as well as finalize a process for manufacturing the product in commercial quantities in accordance with cGMP requirements. The manufacturing process must be capable of consistently producing quality batches of the drug or biological candidate. In addition, companies must develop and validate analytical methods for testing the identity, strength, quality and purity of raw materials, in-process material and the final drug or biologic. Additionally, appropriate packaging must be selected and tested and stability studies must be conducted to demonstrate that the drug or biological candidate does not undergo unacceptable deterioration over its shelf life.

U.S. Review and Approval Processes

The results of product development, preclinical studies and clinical trials, along with descriptions of the manufacturing process, analytical tests conducted on the drug or biologic, proposed packaging and labeling and other relevant information are submitted to the FDA as part of an NDA or BLA requesting approval to market the product. The submission of an NDA or BLA is subject to the

payment of substantial user fees; a waiver of such fees may be obtained under certain limited circumstances. We believe that we will be required to submit BLAs for SL-401 and SL-701, and to submit an NDA for SL-801.

In addition, under the Pediatric Research Equity Act, or PREA, an NDA or a BLA, or supplement to an NDA or a BLA, that covers a new active ingredient, new indication, new dosage form, new dosing regimen, or new route of administration must contain data to assess the safety and effectiveness of the drug or biologic for the claimed indications in all relevant pediatric subpopulations and to support dosing and administration for each pediatric subpopulation for which the product is safe and effective. The FDA may grant deferrals for submission of pediatric data or full or partial waivers. PREA does not apply to any drug or biologic for an indication for which orphan designation has been granted unless FDA were to issue a regulation to require pediatric assessments.

The FDA reviews all NDAs and BLAs submitted before it accepts them for filing and may request additional information rather than accepting an NDA or BLA for filing. Once the submission is accepted for filing, the FDA begins an in-depth review of the NDA or BLA. Under the goals and policies agreed to by the FDA under the Prescription Drug User Fee Act, or PDUFA, the FDA has established a performance goal of ten months in which to complete its initial review of a standard NDA or BLA and respond to the applicant, and a performance goal of six months for a priority NDA or BLA. The FDA does not always meet its PDUFA goal dates for standard and priority NDAs and BLAs.

After the NDA or BLA submission is accepted for filing, the FDA reviews the NDA to determine, among other things, whether the proposed product is safe and effective for its intended use, and whether the product is being manufactured in accordance with cGMP to assure and preserve the product's identity, strength, quality and purity. The FDA reviews a BLA to determine, among other things, whether the product is safe, pure and potent and the facility in which it is manufactured, processed, packaged or held meets standards designed to assure the product's continued safety, purity and potency. In addition to its own review, the FDA may refer applications for novel drug or biological products or drug or biological products which present difficult questions of safety or efficacy to an advisory committee, typically a panel that includes clinicians and other experts in the disease area, for review, evaluation and a recommendation as to whether the application should be approved and under what conditions. The FDA is not bound by the recommendations of an advisory committee, but it considers such recommendations carefully when making decisions. On September 27, 2007, the Food and Drug Administration Amendments Act of 2007 was enacted, giving the FDA enhanced post-market authority, including the authority to require post-marketing studies and post-marketing clinical trials related to serious risks, labeling changes based on new safety information, and compliance with risk evaluation and mitigation strategies, or REMS, approved by the FDA. The FDA's exercise of this authority can result in delays or increased costs during the period of product development, clinical trials and regulatory review and approval, which may also increase costs related to complying with new post-approval regulatory requirements, and increase potential FDA restrictions on the sale or distribution of approved products. During the approval process, the FDA will determine whether a REMS is necessary to assure the safe use of the drug or biologic post-approval. If the FDA concludes that a REMS is needed, the sponsor of the NDA or BLA must submit a proposed REMS; the FDA will not approve the NDA or BLA without a REMS, if required.

Before approving an NDA or BLA, the FDA will inspect the facilities at which the product is to be manufactured. The FDA will not approve the product unless it determines that the manufacturing processes and facilities are in compliance with cGMP requirements and adequate to assure consistent production of the product within required specifications. Also, in this inspection, the FDA seeks to determine whether the manufacturing conforms with application commitments, the authenticity and accuracy of data, and the adequacy of the company's analytical methodology. Additionally, before approving an NDA or BLA, the FDA will typically inspect one or more clinical sites to assure compliance with current good clinical practices, or cGCPs. If the FDA determines the application, data, manufacturing process or manufacturing facilities are not acceptable, it will outline the deficiencies in the submission and often will request additional testing or information.

The NDA or BLA review and approval process is lengthy and difficult and the FDA may refuse to approve an NDA or BLA if the applicable regulatory criteria are not satisfied or the agency requires additional clinical data or other data and information. Even if such data and information is submitted, the FDA may ultimately decide that the NDA or BLA does not satisfy the criteria for approval. Data obtained from clinical trials are not always conclusive and may be susceptible to varying interpretations, which could delay, limit or prevent regulatory approval. In addition, protocol deviations or data discrepancies could delay, limit or prevent regulatory approval. The FDA will issue a "complete response" letter if the agency decides not to approve the NDA or BLA. The complete response letter usually describes all of the specific deficiencies in the NDA or BLA identified by the FDA. The deficiencies identified may be minor, for example, requiring labeling changes, or major, for example, requiring additional clinical trials.

Additionally, the complete response letter may include recommended actions that the applicant might take to place the application in a condition for approval. If a complete response letter is issued, the applicant may either resubmit the NDA or BLA, addressing all of the deficiencies identified in the letter, or withdraw the application.

If a product receives regulatory approval, the approval may be limited to specific diseases and dosages or the indications for use may otherwise be limited, which could restrict the commercial value of the product. Further, the FDA may require that certain contraindications, warnings or precautions be included in the product labeling. In addition, the FDA may require Phase 4 testing which involves clinical trials designed to further assess a product's safety and effectiveness and may require testing and surveillance programs to monitor the safety of approved products that have been commercialized.

Orphan Drug Designation

Under the Orphan Drug Act, the FDA may grant orphan designation to a drug or biological product intended to treat a rare disease or condition, which is generally a disease or condition that affects fewer than 200,000 individuals in the United States, or more than 200,000 individuals in the United States and for which there is no reasonable expectation that the cost of developing and making a drug or biological product available in the United States for this type of disease or condition will be recovered from sales of the product. Orphan product designation must be requested before submitting an NDA or BLA. After the FDA grants orphan product designation, the identity of the therapeutic agent and its potential orphan use are disclosed publicly by the FDA. Orphan product designation does not convey any advantage in or shorten the duration of the regulatory review and approval process.

If a product that has orphan designation subsequently receives the first FDA approval for the disease or condition for which it has such designation, the product is entitled to orphan product exclusivity, which means that the FDA may not approve any other applications to market the same drug or biological product for the same indication for seven years, except in limited circumstances, such as a showing of clinical superiority to the product with orphan exclusivity. Competitors, however, may receive approval of different products for the indication for which the orphan product has exclusivity or obtain approval for the same product but for a different indication for which the orphan product has exclusivity. Orphan product exclusivity also could block the approval of one of our products for seven years if a competitor obtains approval of the same drug or biological product for the same indication as defined by the FDA or if our drug or biological candidate is determined to be contained within the competitor's product for the same indication or disease. If a drug or biological product designated as an orphan product receives marketing approval for an indication broader than what is designated, it may not be entitled to orphan product exclusivity. Orphan drug status in the European Union has similar but not identical benefits in the European Union.

In June 2013, SL-401 was awarded Orphan Drug Designation from the FDA for the treatment of BPDCN. Previously, in February 2011, we received Orphan Drug Designation for SL-401 for the treatment of AML. In September 2015, SL-401 received Orphan Drug designation from the EMA for the treatment of AML. In November 2015, SL-401 was granted Orphan Drug designation by the EMA for the treatment of BPDCN. In addition, we received Orphan Drug Designation for SL-701 for the treatment of glioma in January 2015.

Expedited Development and Review Programs

The FDA has established a Breakthrough Therapy designation program wherein a drug may receive this designation if it is intended to treat a serious condition where preliminary clinical evidence indicates that the drug may demonstrate substantial improvement on a clinically significant endpoint(s) over available therapies. Drugs receiving FDA designation as breakthrough therapies typically receive intensive guidance from FDA's review teams for efficient drug development, organizational commitment from FDA, and rolling review of applications submitted for approval, among other things.

The FDA also has a Fast Track program that is intended to expedite or facilitate the process for reviewing new drug and biological products that meet certain criteria. Specifically, new drug and biological products are eligible for Fast Track designation if they are intended to treat a serious or life-threatening condition and demonstrate the potential to address unmet medical needs for the condition. Fast Track designation applies to the drug product alone or in combination with one or more other drugs for the specific indication for which it is being studied. Unique to a Fast Track product, the FDA may consider reviewing sections of the NDA or BLA on a rolling basis before the complete application is submitted. In addition, the sponsor and FDA would agree on a schedule for the submission of the sections of the NDA or BLA. If the FDA agrees to a rolling review of a NDA or BLA, and determines that the schedule is acceptable, the sponsor pays any required user fees upon submission of the first section of the NDA or BLA.

Any product submitted to the FDA for marketing approval, including those submitted to a Fast Track program, may also be eligible for other types of FDA programs intended to expedite development and review, such as priority review and accelerated approval. Any product is eligible for priority review if it has the potential to provide safe and effective therapy where no satisfactory alternative therapy exists or a significant improvement in the treatment, diagnosis or prevention of a disease compared with marketed products. The FDA will attempt to direct additional resources to the evaluation of an application for a new drug or biological product designated for priority review in an effort to facilitate the review with the goal of taking Agency action on a marketing application within 6 months.

Additionally, a product may be eligible for accelerated approval. Drug or biological products studied for their safety and effectiveness in treating serious or life-threatening illnesses and that provide meaningful therapeutic benefit over existing treatments may receive accelerated approval, which means that they may be approved on the basis of adequate and well-controlled clinical studies establishing that the product has an effect on a surrogate endpoint that is reasonably likely to predict a clinical benefit, or on the basis of an effect on a clinical endpoint other than survival or irreversible morbidity. As a condition of approval, the FDA generally requires that a sponsor of a drug or biological product receiving accelerated approval perform adequate and well-controlled post-marketing clinical studies to establish safety and efficacy for the approved indication. Failure to conduct such studies, or conducting such studies that do not establish the required safety and efficacy may result in revocation of the original approval. In addition, the FDA currently requires as a condition for accelerated approval pre-approval of promotional materials, which could adversely impact the timing of the commercial launch or subsequent marketing of the product. Fast Track designation, priority review and accelerated approval may expedite the development or approval process.

Lastly, a product may be eligible for breakthrough designation and expedited development and review by FDA. A Breakthrough Therapy is defined as a drug that is intended, alone or in combination with one or more other drugs, to treat a serious or life-threatening disease or condition, and preliminary clinical evidence indicates that the drug may demonstrate a substantial improvement over existing therapies on one or more clinically significant endpoints, such as substantial treatment effects observed early in clinical development. The benefits of Breakthrough Therapy designation include more intensive FDA guidance on an efficient drug development program, an organizational commitment involving senior FDA managers, and eligibility for rolling review and priority review of marketing applications. The receipt of a Breakthrough Therapy designation for a product candidate may not result in a faster development process, review or approval compared to drugs considered for approval under conventional FDA procedures and a Breakthrough Therapy designation may be rescinded by FDA where subsequent data no longer support the Breakthrough Therapy designation of the candidate. In August 2016, we announced that the FDA granted Breakthrough Therapy designation to SL-401 for the treatment of BPDCN. There can be no assurance that SL-401 will be approved by the FDA on a faster timeline with this indication or at all. In addition, the FDA may later decide that SL-401 no longer meets the conditions for designation.

Post-Approval Requirements

Any drug or biological products for which we receive FDA approvals are subject to continuing regulation by the FDA, including, among other things, cGMP compliance, record-keeping requirements, reporting of nonconforming distributed products which would require field alert reports (FARs) for NDAs and biological product deviation reports (BPDRs) for BLAs, reporting of adverse events, providing the FDA with updated safety and efficacy information on an annual basis or as required more frequently for specific events, product sampling and distribution requirements, complying with certain electronic records and signature requirements and complying with FDA promotion and advertising requirements, which include, among others, standards for direct-to-consumer advertising, prohibitions against promoting drugs and biologics for uses or in patient populations that are not described in the drug's or biologic's approved labeling (known as "off-label promotion"), rules for conducting industry-sponsored scientific and educational activities, limitations on comparative or superiority claims and promotional activities involving data presentations. Failure to comply with FDA requirements can have negative consequences, including for cause inspections; warning or untitled letters from the FDA, including demands for correction or removal of noncomplying product; adverse publicity; mandated corrective advertising or communications with doctors; and civil or criminal penalties. Although physicians may prescribe legally available drugs and biologics for off-label uses, manufacturers may not market or promote such off-label uses.

We rely, and expect to continue to rely, on third parties for the production of clinical and commercial quantities of our product candidates. Manufacturers of our product candidates are required to comply with applicable FDA manufacturing requirements contained in the FDA's cGMP regulations. cGMP regulations require among other things, quality control and quality assurance as well as the corresponding maintenance of comprehensive records and documentation. We are required by law to establish adequate oversight and control over raw materials, components and finished products furnished by our third-party manufacturers, which we establish by contract, supplier qualification and periodic audits, but unforeseen circumstances could affect our third-party manufacturers' compliance with applicable regulations and standards. Drug and biologic manufacturers and other entities involved in the manufacture and distribution of approved drugs and biologics are also required to register their establishments and list any products made there with the FDA and comply with related requirements in certain states, and are subject to periodic unannounced inspections by the FDA and certain state agencies for compliance with cGMP and other laws. Accordingly, manufacturers must continue to expend time, money and effort in the area of production and quality control to maintain cGMP compliance. Discovery of problems with a product after approval may result in serious and extensive restrictions on a product, manufacturer, or holder of an approved NDA or BLA, including suspension of a product until the FDA is assured that quality standards can be met, continuing oversight of manufacturing by the FDA under a "consent decree," which frequently includes the imposition of penalties for failure to comply with the terms of the consent decree, audits conducted by outside experts, extensive reporting requirements, and possible withdrawal of the product from the market. Historically, the minimum term of an FDA consent decree has been five years, and violation of consent decree terms can result in the extension of the consent decree term.

Major changes to the manufacturing process and other types of major changes, such as adding new indications, require prior FDA approval before being implemented. Moderate and minor changes require FDA notification but not prior approval.

The FDA also may require post-marketing testing, known as Phase 4 testing, risk minimization action plans and surveillance to monitor the effects of an approved product or place conditions on an approval that could otherwise restrict the distribution or use of the product.

On July 9, 2012, the Food and Drug Administration Safety and Innovation Act, among other things, renewed the drug user fee program, expanded the FDA's inspection records access and required manufacturers to establish appropriate oversight and controls over their suppliers and the supply chain, including raw material suppliers and contract manufacturers, as a part of cGMP compliance. On November 27, 2013, the Drug Quality and Security Act, which included the Drug Supply Chain Security Act, was enacted to, among other things, build an electronic, interoperable system to identify and trace certain prescription drugs as they are distributed in the United States. Requirements for the tracing of products through the pharmaceutical distribution supply chain took effect on January 1, 2015 for manufacturers and building internal systems to ensure compliance with this law will require dedication of resources. In addition, this law requires engaging in transactions only with authorized trading partners and can limit the pool of available trading partners. On August 18, 2017, the FDA Reauthorization Act of 2017, among other things, reauthorized and amended the user fee program eliminating the establishment fee and replacing the Prescription Drug Product Fee with a Prescription Drug Program Fee.

U.S. Patent Term Restoration and Marketing Exclusivity

Drug Price Competition and Patent Term Restoration Act of 1984

Depending upon the timing, duration and specifics of the FDA approval of the use of our drug and biologics candidates, some of our United States patents may be eligible for limited patent term extension under the Drug Price Competition and Patent Term Restoration Act of 1984, commonly referred to as the Hatch-Waxman Amendments. Although drafted to cover small-molecule drugs, as opposed to biologics, the Hatch-Waxman Amendments permit a patent restoration term of up to five years as compensation for patent term lost during federal regulatory review preceding the FDA regulatory review process. The FDA has been granting patent term extensions to biologics. However, patent term restoration cannot extend the remaining term of a patent beyond a total of 14 years from the product's approval date. The patent term restoration period is generally one-half the time between the effective date of an IND and the submission date of an NDA or a BLA plus the time between the submission date of an NDA or a BLA and the approval of that application. Only one patent applicable to an approved drug or biologic is eligible for the extension and the application for the extension must be submitted within 60 days of approval and prior to the expiration of the patent. Further, to be eligible for patent term extension a drug must be the first permitted commercial marketing for which there was a regulatory review period. The United States Patent and Trademark Office, in consultation with the FDA, reviews and approves the application for any patent term extension or restoration. In the future, we may apply for restoration of patent term for one of our currently owned or licensed patents to add patent life beyond its current expiration date, depending on the expected length of the clinical trials and other factors involved in the filing of the relevant NDA.

Federal Food, Drug and Cosmetic Act

Market exclusivity provisions under the FDCA, which are independent of patent status and any patent-related extensions and can run concurrently with a patent or not, can also delay the submission or effective approval of certain applications of companies seeking to reference another company's NDA. The length of time that the FDA grants depends on the type of exclusivity.

If the new drug that is the subject of an approved NDA contains a new chemical entity, the FDCA provides a five-year period of non-patent marketing exclusivity within the United States that runs from the date of NDA approval. FDA regulations define "new chemical entity" as "a drug that contains no active moiety that has been approved by FDA in any other NDA submitted under section 505(b) of the Federal Food, Drug, and Cosmetic Act." 21 C.F.R. § 314.108. During the five-year exclusivity period, no company may submit an ANDA or a 505(b)(2) NDA for a drug product that contains the same active moiety as in the new chemical entity. However, such application may be submitted after four years if it contains a certification of patent invalidity or noninfringement to one of the patents listed in FDA's Orange Book by the innovator NDA holder.

The FDCA also provides three years of marketing exclusivity for an NDA, or supplement to an existing NDA, that contained new clinical investigations (other than bioavailability studies) that were conducted or sponsored by the applicant and are deemed by FDA to be essential to the approval of the NDA or supplement. Such new clinical investigations may support approval of, for example, new indications, dosages or strengths of an existing drug. This three-year exclusivity period runs from the date of approval of the NDA or supplement containing the new clinical investigations and covers only the conditions associated with the new clinical investigations. That is, it bars the FDA from approving an ANDA or 505(b)(2) NDA for those conditions of approval. Unlike NCE exclusivity, exclusivity for new clinical investigations does not prohibit the submission of an ANDA or 505(b)(2) NDA by another company during that three-year period.

Pediatric exclusivity is another type of regulatory market exclusivity in the United States. Pediatric exclusivity, if granted by the FDA, adds six months to existing exclusivity periods and patent terms. This six-month exclusivity, which runs from the end of other exclusivity protection or patent term, may be granted based on the completion of a pediatric trial in accordance with an FDA-issued "Written Request" for such a trial. Biologic products that are subject to the PHSA are not eligible for pediatric exclusivity under the FDCA.

Biologics Price Competition and Innovation Act of 2009

The Biologics Price Competition and Innovation Act of 2009, or BPCIA, amended the PHSA to create a new licensure framework for biosimilar products, which could ultimately subject our biological product candidates to competition. Under the BPCIA, a manufacturer may submit an application for licensure of a biological product that is "biosimilar to" or "interchangeable with" a referenced, branded biologic product. Previously, there had been no licensure pathway for such biosimilar or interchangeable products. For purposes of the BPCIA, a reference product is defined as the single biological product licensed under a full BLA against which a biological product is evaluated in an application submitted under a follow-on BLA.

The BPCIA also created a 12-year period of reference product exclusivity, which can be extended to 12.5 years with pediatric exclusivity. The 12-year exclusivity period begins on the date of first licensure of the reference product under the PHSA and during which the licensure of a follow-on application for a biosimilar or interchangeable product cannot be made effective. During the first four years (or four and one-half years with pediatric exclusivity) of the 12-year period, an application for a biosimilar or interchangeable version of the reference product cannot be submitted to the FDA. Recently. members have begun introducing bills that would decrease the reference product exclusivity reference product exclusivity from 12 to seven years. Congress has not yet enacted such a change in the BPCIA, but could move to enact such a decrease in the reference product exclusivity period.

The BPCIA includes limits on obtaining 12-year reference product exclusivity for certain changes or modifications to the reference product. A separate 12-year reference product exclusivity period does not apply to:

- a BLA supplement for the product that is the reference product;
- a subsequent BLA filed by the same reference product sponsor or manufacturer (or a licensor, predecessor in interest, or other related entity) for a change (not including a modification to the structure of the biological product) that results in a new indication, route of administration, dosing schedule, dosage form, delivery system, delivery device or strength; or
- a modification to the structure of the biological product that does not result in a change in safety, purity or potency.

In addition to creating a 12-year period of reference product exclusivity, the BPCIA clarifies the interaction of that exclusivity with orphan drug exclusivity, such that, if a reference product has been designated for a rare disease or condition the licensure of a biosimilar or interchangeable version of a reference product for such disease or condition may only occur after the later of the expiration of any applicable seven-year orphan drug exclusivity or the 12-year reference product exclusivity (or seven and one-half years and 12.5 years with pediatric exclusivity).

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

Our biological product candidates, if approved, could be considered reference products entitled to 12-year exclusivity. Even if our products are considered to be reference products eligible for exclusivity, another company could market a competing version of any of our biological products if the FDA approves a full BLA for such product containing the sponsor's own preclinical data and data from adequate and well-controlled clinical trials to demonstrate the safety, purity and potency of their product.

The BPCIA also sets forth a complex mechanism for resolving patent disputes that involves a step-wise exchange of information prior to the initiation of a patent infringement lawsuit against a biosimilar or interchangeable product sponsor. Unlike the Hatch-Waxman Act, the BPCIA provides no automatic stay on approval of a biosimilar product application, except an interchangeable product receives the lesser of one year of exclusivity after the date of first commercial marketing or 18 months of exclusivity after a final court decision or dismissal of a patent challenge or, if the applicant has not been sued, after approval. The BPCIA does not prevent a competitor from conducting its own clinical trials and submitting a full BLA on the same or similar product.

There is also currently substantial uncertainty as to how certain terms of the BPCIA will be interpreted by the Courts, which may affect the timing of the entry of a biosimilar or interchangeable product to market, and the required notice that the owner of the reference product exclusivity must be given by the owner of the application of a biosimilar or interchangeable product. Should the courts resolve the interpretation issues in favor of the biosimilar or interchangeable product applicants, the BPCIA may offer more limited exclusivity to the reference product than currently believed.

Other U.S. Healthcare Laws and Compliance Requirements

In the United States, our activities are potentially subject to regulation by various federal, state and local authorities in addition to the FDA, including the Centers for Medicare and Medicaid Services, or CMS (formerly the Health Care Financing Administration), other divisions of the United States Department of Health and Human Services (e.g., the Office of Inspector General and the Office of Civil Rights), the United States Department of Justice and individual United States Attorney offices within the Department of Justice, state attorney generals and state and local governments. For example, sales, marketing and scientific/educational grant programs must comply with the federal Anti-Kickback Statute, the federal False Claims Act, the privacy and security provisions of the Health Insurance Portability and Accountability Act, or HIPAA, as amended by the Health Information Technology for Economic and Clinical Health Act ("HITECH"), and similar state laws, each as amended. Pricing and rebate programs must comply with the Medicaid rebate requirements of the Omnibus Budget Reconciliation Act of 1990, the Veterans Health Care Act of 1992, and the federal Anti-Kickback Statute, each as amended. If products are made available to authorized users of the Federal Supply Schedule (FSS) of the General Services Administration, additional laws and requirements apply. Under the Veterans Health Care Act, or VHCA, drug companies are required to offer certain pharmaceutical products at a reduced price to four federal agencies including the United States Department of Veterans Affairs, the United States Department of Defense, the Coast Guard, the Public Health Service and certain private Public Health Service designated entities (including the Indian Health Service) in order for reimbursement to be available for our product under Medicare and Medicaid. FSS pricing to these four agencies must be equal to or less than the federal ceiling price ("FCP"), which is, at a minimum, 24% off the Non-Federal Average Manufacturer Price for the prior fiscal year. Participation under the VHCA requires submission of pricing data and calculation of discounts and rebates pursuant to complex statutory formulas, as well as the entry into government procurement contracts governed by the Federal Acquisition Regulations.

In order to distribute products commercially, we must comply with state laws that require the registration of manufacturers and wholesale distributors of pharmaceutical products in a state, including, in certain states, manufacturers and distributors who ship products into the state even if such manufacturers or distributors have no place of business within the state. Some states also impose requirements on manufacturers and distributors to establish the pedigree of product in the chain of distribution, including some states that require manufacturers and others to adopt new technology capable of tracking and tracing product as it moves through the distribution chain. Several states have enacted legislation requiring pharmaceutical companies to establish marketing compliance programs, file periodic reports with the state, make periodic public disclosures on sales, marketing, pricing, clinical trials and other activities, and/or register their sales representatives, as well as to prohibit pharmacies and other healthcare entities from providing certain physician prescribing data to pharmaceutical companies for use in sales and marketing, and to prohibit certain other sales and marketing practices. All of our activities are potentially subject to federal and state consumer protection and unfair competition laws. In addition, in August 2013, the federal Physician Payment Sunshine Act took effect and requires annual reporting by prescription drug manufacturers with at least 1 approved product of certain payments and transfers of value made to physicians and teaching hospitals. Post-approval of any of our product candidates, we will need to ensure compliance with annual tracking and reporting of these payments and transfers of value to CMS.

Europe and Worldwide Government Regulation

In addition to regulations in the United States, we will be subject to a variety of regulations in other jurisdictions governing, among other things, clinical trials and any commercial sales and distribution of our products.

Whether or not we obtain FDA approval for a product, we must obtain the requisite approvals from regulatory authorities in foreign countries prior to the commencement of clinical trials or marketing of the product in those countries. Certain countries outside of the United States have a similar process that requires the submission of a clinical trial application much like the IND prior to the commencement of human clinical trials. In the European Union, for example, a clinical trial application, or CTA, must be submitted to each country's national health authority and an independent ethics committee, much like the FDA and IRB, respectively. Once the CTA is approved in accordance with a country's requirements, clinical trials may proceed.

The requirements and process governing the conduct of clinical trials, product licensing, pricing and reimbursement vary from country to country. In all cases, the clinical trials are required to be in accordance with International Conference on Harmonisation (ICH) / WHO Good Clinical Practice standards and the applicable regulatory requirements and the ethical principles that have their origin in the Declaration of Helsinki.

To obtain regulatory approval of an investigational drug or biological product under European Union regulatory systems, we must submit a marketing authorization application to the EMA. The application used to file an NDA or a BLA in the United States is similar to that required in the European Union, with the exception of, among other things, country-specific document requirements. For example, the EMA has already established a number of guidelines for approval of various biosimilars.

For other countries outside of the European Union, such as countries in Eastern Europe, Latin America or Asia, the requirements governing the conduct of clinical trials, product licensing, pricing and reimbursement vary from country to country. In all cases, again, the clinical trials are conducted in accordance with cGCP and the applicable regulatory requirements and the ethical principles that have their origin in the Declaration of Helsinki.

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

Pharmaceutical Coverage, Pricing and Reimbursement

Significant uncertainty exists as to the coverage and reimbursement status of any drug or biological candidates for which we obtain regulatory approval. In the United States and markets in other countries, sales of any products for which we receive regulatory approval for commercial sale will depend in part on the availability of reimbursement from third-party payors. Third-party payors include government health administrative authorities, managed care providers, private health insurers and other organizations. The process for determining whether a payor will provide coverage for a drug or biological product may be separate from the process for setting the price or reimbursement rate that the payor will pay for the drug or biological product. Third-party payors may limit coverage to specific drug or biological products on an approved list, or formulary, which might not include all of the FDA-approved drug or biological products for a particular indication. Third-party payors are increasingly challenging the price and examining the medical necessity and cost-effectiveness of medical products and services, in addition to their safety and efficacy. We may need to conduct expensive pharmacoeconomic studies in order to demonstrate the medical necessity and cost-effectiveness of our products, in addition to the costs required to obtain the FDA approvals. Our drug or biological candidates may not be considered medically necessary or cost-effective. A payor's decision to provide coverage for a drug or biological product does not imply that an adequate reimbursement rate will be approved. Adequate third-party reimbursement may not be available to enable us to maintain price levels sufficient to realize an appropriate return on our investment in product development.

In 2003, the United States government enacted legislation providing a partial prescription drug benefit for Medicare recipients, which became effective at the beginning of 2006. Government payment for some of the costs of prescription drugs and biologics may increase demand for any products for which we receive marketing approval. However, to obtain payments under this program, we would be required to sell products to Medicare recipients through prescription drug plans operating pursuant to this legislation. These plans will likely negotiate discounted prices for our products. Federal, state and local governments in the United States continue to consider legislation to limit the growth of healthcare costs, including the cost of prescription drugs and biologics. Future legislation could limit payments for pharmaceuticals such as the drug or biological candidates that we are developing.

Different pricing and reimbursement schemes exist in other countries. In the European Community, governments influence the price of pharmaceutical products through their pricing and reimbursement rules and control of national healthcare systems that fund a large part of the cost of those products to consumers. Some jurisdictions operate positive and negative list systems under which products may only be marketed once a reimbursement price has been agreed. To obtain reimbursement or pricing approval, some of these countries may require the completion of clinical trials that compare the cost-effectiveness of a particular drug or biological candidate to currently available therapies. Other member states allow companies to fix their own prices for medicines, but monitor and control company profits. The downward pressure on healthcare costs in general, particularly prescription drugs and biologics, has become very intense. As a result, increasingly high barriers are being erected to the entry of new products. In addition, in some countries, cross-border imports from low-priced markets exert a commercial pressure on pricing within a country.

The marketability of any drug or biological candidates for which we receive regulatory approval for commercial sale may suffer if the government and third-party payors fail to provide adequate coverage and reimbursement. In addition, emphasis on managed care in the United States has increased and we expect will continue to increase the pressure on pharmaceutical pricing. Coverage policies and third-party reimbursement rates may change at any time. Even if favorable coverage and reimbursement status is attained for one or more products for which we receive regulatory approval, less favorable coverage policies and reimbursement rates may be implemented in the future.

Manufacturing

We do not currently own or operate any manufacturing facilities for the clinical or commercial production of our drug candidates. For past investigator sponsored studies, all drug substance and drug product for SL-401 and SL-701 was manufactured at academic and contract manufacturing organizations, or CMO, facilities, as directed by our academic collaborators. We have now developed manufacturing processes that are suitable for full-scale cGMP manufacturing. Additionally, we have qualified FDA-audited third-party CMOs to produce sufficient quantities of SL-401, SL-801, and SL-701 drug substance and drug product of suitable quality for our active and contemplated corporate sponsored clinical trials and potential commercialization. For SL-401, activities are underway to deliver commercial supplies in support of potential expedited approval pathways. Our manufacturing programs are being managed with oversight by our manufacturing team, which is comprised of full-time employees and consultants with experience in manufacturing pharmaceutical drug substance and drug products.

SL-401 Manufacturing and Supply

SL-401 is a recombinant protein generated from an antibiotic-resistance driven DNA-based plasmid vector and manufactured by bacterial fermentation in E. Coli. For past investigator sponsored studies, SL-401 was manufactured at Wake Forest University. We have optimized the protein expression, generated cGMP master and working cell banks, and developed the fermentation and purification steps of our manufacturing process to be suitable for scale-up in standard manufacturing equipment. This technology has been transferred to a third-party CMO with expertise in bacterial fermentation, which has further optimized and scaled-up the process in their cGMP production suite. The SL-401 drug substance has now met standard industry quality specifications and is adequate to support our ongoing and planned corporate sponsored clinical trials. The drug product formulation and manufacturing process has been transferred to a third-party CMO with expertise in sterile product manufacture for clinical and commercial supply, and they have successfully produced drug product meeting cGMP requirements for use in clinical studies. We expect the same process, scale and CMOs will be used to manufacture drug substance and drug product for commercial supply. We have a lyophilized formulation of SL-401, which is currently being developed.

SL-801 Manufacturing and Supply

SL-801 is a small molecule that is prepared via synthetic organic chemistry. We have completed process development and cGMP manufacturing at an adequate scale to supply our ongoing and planned corporate sponsored clinical studies. We have also developed a stable solid-oral tablet formulation that has been produced using cGMP manufacturing equipment at our third-party CMO. We believe that the manufacturing scale and product quality procedures and oversight ensure an adequate supply for our active and planned corporate sponsored clinical studies.

SL-701 Manufacturing and Supply

SL-701 is an immunotherapy that is comprised of several short synthetic peptides. Each of the component peptides of SL-701 is manufactured individually by solid-phase synthesis and all have been prepared to acceptable quality specifications in cGMP manufacturing equipment by our third-party CMO. The manufacturing scale and product quality procedures and oversight ensure an adequate supply for our active and planned corporate sponsored clinical studies. We have also developed a stable formulation that combines the individual peptides in a single sterile solution to generate SL-701 drug product. This manufacturing process was transferred to a third-party CMO with expertise in sterile product manufacture. This CMO has produced multiple cGMP drug product batches of sufficient quality and quantity to supply our corporate sponsored clinical trials. SL-701 has been utilized with several different adjuvants including Montanide, GM-CSF, Imiquimod, and poly-ICLC. We have been, and may continue to be, reliant on the availability of these adjuvants from their respective manufacturers.

Sales and Marketing

We believe the infrastructure required to commercialize oncology products is relatively limited, which may make it cost-effective for us to internally develop, train, and deploy a marketing sales force. If SL-401 or our other compounds are approved by the FDA and other regulatory authorities, we plan to potentially build the infrastructure to commercialize these products in North America and

Europe ourselves. However, we will remain opportunistic in seeking strategic partnerships in these and other markets when advantageous.

The commercial infrastructure of specialty oncology products typically consists of a targeted, specialty sales force that calls on a limited and focused group of physicians supported by sales management, internal sales support, an internal marketing group, and distribution support. Additional capabilities important to the oncology marketplace include the management of key accounts, such as managed care organizations, group-purchasing organizations, specialty pharmacies, oncology group networks, and government accounts. As SL-401 and our other compounds may initially be developed for orphan indications with a relatively small number of treating physicians, we anticipate that a reduced infrastructure, including a small, targeted sales force, will be sufficient to support our sales and marketing objectives. In order to implement this infrastructure, we will have to allocate management resources and make significant financial investments including some prior to product approval to prepare for the commercial launch of an approved product, including preparation of marketing and sales training materials in compliance with legal and regulatory requirements.

We may elect in the future to utilize strategic partners, distributors, or contract sales forces to assist in the commercialization of our products.

Research and Development

Company sponsored research and development expenses totaled $50.2 million in 2017, $27.9 million in 2016 and $29.5 million in 2015. "Research and development expenses" consist of costs associated with the development of our product candidates and our platform technology, which include: clinical trial costs, CMC-related costs, nonclinical costs, employee related expenses, external research and development expenses, license fees and milestone payments related to in-licensed products and technology, and facilities, depreciation and other allocated expenses. See "Item 7. Management's Discussion and Analysis of Financial Condition and Results of Operations—Overview."

Employees

As of March 16, 2018, we had 37 full-time employees, 6 of whom hold Ph.D. or M.D. degrees. None of our employees is subject to a collective bargaining agreement or represented by a trade or labor union. We believe that we have a good relationship with our employees.

Item 1A. Risk Factors

You should carefully consider the following risks and uncertainties. If any of the following occurs, our business, financial condition and/or operating results could be materially harmed. These factors could cause the trading price of our common stock to decline, and you could lose all or a substantial part of your investment.

Risks Related to Development, Clinical Testing, Regulatory Approval, and Commercialization of Our Product Candidates

We are heavily dependent on the success of our clinical product candidates, SL-401, SL-801, and SL-701, and we cannot provide any assurance that any of our product candidates will be approved, commercialized, or successfully marketed in the future.

To date, we have invested a significant portion of our efforts and financial resources in the acquisition and development of our product candidates, SL-401, SL-801, and SL-701 which we are advancing through clinical development. Our future success depends heavily on our ability to successfully manufacture, develop, obtain regulatory approval for, and commercialize these product candidates, which may never occur. We currently generate no revenues from our product candidates, and we may never be able to develop or commercialize a marketable drug.

Before we generate any revenues from product sales in the United States or elsewhere, we must complete preclinical and clinical development of one or more of our product candidates, conduct human subject research, submit clinical and manufacturing data to the U.S. Food and Drug Administration, or FDA, or foreign equivalent, qualify a third-party contract manufacturing organization, or CMO, satisfy the FDA that our CMO is capable of manufacturing the product in compliance with the FDA's current good manufacturing practices, or CGMPs, submit a Biologics License Application, or BLA (or foreign equivalent), receive regulatory approval from the FDA or foreign regulatory agency, build a commercial organization, make substantial investments and undertake significant marketing efforts ourselves or in partnership with others to ensure compliant marketing and market acceptance of any products we commercialize. We are not permitted to market or promote any of our product candidates before we receive regulatory approval from the FDA or comparable foreign regulatory authorities, and we may never receive such regulatory approval for any of our product candidates.

We have not submitted a BLA or a New Drug Application, or NDA, to the FDA, or similar market approval applications to comparable foreign authorities, for any of our product candidates. We cannot be certain that any BLA or NDA will be filed within a specified period of time, or that any BLA or NDA or similar foreign marketing application will allow us to obtain or maintain marketing approval. In addition, any marketing approval we may obtain may be for uses more limited than we expect or include contraindications or risk measures that limit its market acceptance. We also cannot be certain that any of our product candidates will be successful in clinical trials that the clinical trials or data will support filing a BLA or NDA in the U.S. or elsewhere. We also cannot be certain that any of our product candidates will receive regulatory approval for trial initiation or marketing. Further, the FDA, an independent review committee, or IRC, or an oncologic drugs advisory committee, or ODAC, may not agree with the interpretation by our investigators or us of the clinical safety and efficacy of our product candidates and our product candidates may not receive regulatory approval. If we do not receive regulatory approvals for our product candidates, we may not be able to continue our operations. Even if we successfully obtain regulatory approvals to market one or more of our product candidates, our revenues will be dependent, in part, upon the prescribing information, adoption within clinical practice guidelines, and the size of the markets in the territories for which we gain regulatory approval and have commercial rights. In addition, our revenues will be dependent, in part, upon the market acceptance of our products once approved as well as upon reimbursement and coverage, among other things. If the markets for patient subsets that we are targeting are not as significant as we estimate, we may not generate significant revenues from sales of such products, if approved.

We do not have the resources to conduct and oversee our product development programs without assistance from third parties. In the execution of our product development programs, we may have to rely on collaborations with clinical partners as well as clinical research organizations, or CROs, CMOs, vendors and other service providers. Failure of these entities to satisfactorily conduct clinical research or to provide the services requested by the Company may negatively impact our product development programs, including but not limited to program delays or preventing approval of our product candidates. We plan to seek regulatory approval to commercialize our product candidates in the United States, and potentially in the European Union and additional foreign countries. While the scope of regulatory review and approval is typically similar in other countries, to obtain separate regulatory review and approval in many other countries, we must comply with the numerous and varying regulatory requirements of such countries regarding safety and efficacy, clinical trials, manufacturing, post-marketing commitments, and commercial sales, pricing and distribution of our product candidates, and we cannot predict success in these jurisdictions.

Clinical drug development involves a lengthy and expensive process with an uncertain outcome.

Clinical testing is expensive and can take a substantial amount of time to complete. Its outcome is inherently uncertain. In addition, failure can occur at any time during clinical development, including after significant resources have been invested. We cannot predict whether we will encounter challenges with any of our clinical trials that will cause us, or regulatory authorities, to delay, suspend or terminate those trials.

Clinical trials can be delayed or halted for many reasons, including:

- delays or failure reaching agreement on acceptable terms with prospective CMOs, CROs, and clinical trial sites, the terms of which can be subject to extensive negotiation and may vary significantly depending on the circumstances;
- failure of our third-party contractors, including CROs and CMOs, or our investigators, to comply with regulatory requirements or otherwise meet contractual obligations in a timely manner;
- delays or failure in obtaining the necessary approvals from regulators, institutional review boards, or IRBs, or scientific review committees, or SRCs, in order to commence or continue a clinical trial;
- our inability to manufacture, or obtain from third-parties, adequate supply of drug substance, drug product or adjuvant therapies sufficient to complete our preclinical studies and clinical trials;
- risk of loss of drug product, adjuvants and/or other components of the product, due to third-party storage and distribution of such supplies;
- the FDA, or other regulatory authority, issuing a clinical hold or requiring alterations to any of our study designs, including extending a study or requiring new studies, overall strategy or manufacturing plans;
- delays in patient enrollment, variability in the number and types of patients available for clinical trials, poor accrual, or high drop-out rates of patients in our clinical trials;

- clinical trial sites deviating from trial protocols or dropping out of a trial and our inability to add new clinical trial sites;
- difficulty in maintaining contact with patients after treatment, resulting in incomplete data;
- poor effectiveness of our product candidates during clinical trials;
- safety issues, including serious adverse events associated with our product candidates and patients' exposure to unacceptable health risks;
- receipt by a competitor of marketing approval for a product targeting an indication that one of our product candidates target, such that we are not "first to market" with our product candidate;
- governmental or regulatory delays and changes in regulatory leadership, requirements, policy and guidelines; or
- differing interpretations of data by the FDA or similar foreign regulatory agencies.

We could also encounter delays if a clinical trial is suspended or terminated by us, by the IRB where such trial is being conducted, by the Data Safety Monitoring Board, or DSMB, if one is utilized for any such trial, or by the FDA or other regulatory authorities. Such authorities may suspend or terminate a clinical trial due to a number of factors, including, among other things, failure to conduct the clinical trial in accordance with regulatory requirements or our clinical protocols, regulatory violations identified during an inspection of the clinical trial operations or trial site, imposition of a clinical hold by the FDA or other regulatory authorities, study subject safety concerns, adverse events or severe adverse events including deaths, failure to demonstrate a benefit from using a drug, changes in governmental regulations or administrative actions or lack of adequate funding to continue the clinical trial. For example, we have observed serious adverse events, including deaths, from or relating to capillary leak syndrome, or CLS, with SL-401. The occurrence of these and other adverse events could jeopardize or preclude our ability to develop, obtain or maintain marketing approval for, or successfully commercialize, market, and sell any or all of our product candidates for one or more indications.

We intend to have ongoing interactions with the FDA over the course of 2018 and beyond regarding our product candidates, including our SL-401 Phase 2 pivotal trial in patients with blastic plasmacytoid dendritic cell neoplasm, or BPDCN. If the FDA reviews these data and determines additional data are needed to support a submission for regulatory approval in BPDCN, or that the data will not support a submission for regulatory approval in BPDCN, this could delay or halt our clinical trials or commercialization plans for SL-401 or our other product candidates, including requiring us to enroll additional cohorts or conduct additional clinical trials.

We have also advanced SL-801 into a Phase 1 clinical trial. There are unknown risks for SL-801 with respect to dosing, administration, pharmacokinetics, bioavailability, safety and efficacy that we expect we will learn about during clinical development which could halt or delay this development program and which could alter our current strategy for the development of this product candidate.

We may not have the necessary expertise or capabilities, including adequate staffing, to successfully manage the execution and completion of any of our clinical trials, prepare clinical study reports and marketing authorization applications, and ultimately obtain marketing approval for our product candidates in a timely manner, or at all.

In any clinical trial of a product candidate, the results of such trial may not be adequate to support submission of a marketing application or marketing approval. Because our product candidates are intended for use in life-threatening diseases, in many cases we ultimately intend to seek marketing approval for each product candidate based on the results of a single clinical trial which may be open-label and single-group in nature. As a result, these trials may receive enhanced scrutiny from the FDA. For any such trial, if the FDA disagrees with our choice, or definition, of primary endpoint or the results for the primary endpoint are not robust or significant or clinically beneficial enough, including relative to control, or historical data, the FDA may refuse to approve a BLA or NDA based on such intended pivotal trial, despite meeting a primary endpoint of the study. In addition, the results of any such intended pivotal trial may be subject to confounding factors, or may not be adequately supported by other study endpoints, including possibly overall survival, or OS, overall response rate, or ORR, rate of complete response, or CR, and/or response duration, in which case the FDA may refuse to approve a BLA or NDA based on such intended pivotal trial, despite meeting a primary endpoint of the study. The FDA may also require the completion of additional clinical trials before or as a condition for approving our product candidates.

If we experience delays in the completion of, or a termination of, any clinical trial of our product candidates, the commercial prospects of our product candidates will be harmed, which will have a negative impact on our ability to commence product sales and generate product revenues from any of our product candidates. In addition, any delays in completing our clinical trials will increase our costs and slow down our product candidate development and approval process and may negatively impact our ability to raise additional

capital to support these increased costs. Delays in completing our clinical trials could also allow our competitors to obtain marketing approval before we do or shorten the patent protection period during which we may have the exclusive right to commercialize our product candidates. Any of these occurrences may harm our business, financial condition and prospects significantly. In addition, many of the factors that cause, or lead to, a delay in the commencement or completion of clinical trials may also ultimately lead to the denial of regulatory approval of our product candidates.

Reports of adverse events or other safety concerns involving SL-401, SL-801, or SL-701 could delay clinical development, delay or prevent us from obtaining or maintaining regulatory approvals, or negatively impact sales or the commercial prospects for our product candidates.

Reports of adverse events or other safety concerns involving SL-401, SL-801, or SL-701 could interrupt, delay or halt clinical trials of our clinical candidates, including the ongoing Phase 2 pivotal trial in BPDCN. For example, CLS is a known, sometimes fatal, and well-documented side effect of SL-401. Reports of additional CLS cases, or other adverse events or other safety concerns involving SL-401 or our other product candidates, could result in clinical trial delays including regulatory authorities putting trials on clinical hold or denying or withdrawing approval for trials of any or all indications. Further, there are no assurances that patients receiving SL-401 or our other product candidates with co-morbid diseases and/or indications not previously well-studied, will not experience new or different serious adverse events in the future. Likewise, reports of adverse events or other safety concerns involving SL-401 or our other product candidates could interrupt, delay or halt ongoing or planned clinical trials of such product candidates, could require redesign of study protocols and conduct of additional trials, could result in our inability to file for or obtain regulatory approvals for any of our product candidates, or negatively impact commercial prospects for our product candidates.

Results of earlier clinical trials may not be predictive of the results of later-stage clinical trials.

The results of preclinical studies and early stage, including investigator-sponsored, clinical trials and late stage clinical trials of product candidates may not be predictive of the results of subsequent later stage, including corporate sponsored, clinical trials. Product candidates in later stage or larger clinical trials may fail to show the safety and efficacy results demonstrated in earlier studies despite having progressed through preclinical studies and earlier clinical trials. Many companies in the biopharmaceutical industry have suffered significant setbacks in later stage clinical trials due to adverse safety profiles or lack of efficacy, notwithstanding promising results in earlier studies. Similarly, our clinical trial results may not be successful for these or other reasons. For example, Stage 3 results from the Phase 2 pivotal trial of SL-401 in BPDCN may not corroborate the earlier Stage 1 and 2 results and/or may not be adequate for marketing approval for any of a number of reasons including clinical safety and efficacy results, including choice and definition of endpoints and regulatory hurdles for success, as well as data from chemistry, manufacturing and controls, or CMC, clinical pharmacology, bioanalytical, immunogenicity, non-clinical, and other areas.

This drug development risk is heightened by any changes in ongoing and future clinical trials compared to completed clinical trials. As product candidates are advanced through preclinical studies to early and later stage clinical trials and towards approval and commercialization, it is customary that various aspects of the development program, such as manufacturing and methods of administration and dosing, are altered along the way in an effort to optimize processes and results. While these types of changes are common and are intended to optimize the product candidates for late stage clinical trials, approval and commercialization, such changes do carry the risk that they will not achieve these intended objectives. For example, the results of our ongoing and future clinical trials may be adversely affected by the following changes:

- As we optimize and scale-up production of SL-401, SL-801, and SL-701 there have been manufacturing, formulation, fill-finish and other process and analytical changes that are part of the optimization and scale-up necessary for producing drug substance and drug product of a quality, quantity and stability sufficient for later stage clinical development and commercialization. Delays, including failures, in any of these steps may delay initiation and completion of clinical trials, regulatory submissions, or commercial launch. We may also need to demonstrate comparability between newly manufactured drug substance and/or drug product relative to previously manufactured drug substance and/or drug product. Demonstrating comparability may cause us to incur additional costs or delay initiation or completion of our clinical trials including the need or choice to initiate a dose escalation study and, if unsuccessful, could require us to complete additional preclinical or clinical studies of our product candidates. Failure to demonstrate comparability could also result in delays in regulatory submissions or commercial launch. We are also developing a new lyophilized formulation of SL-401. In the event it does not demonstrate adequacy or comparability with the current liquid/frozen formulation, regulatory approval and/or commercial utilization of SL-401 could be negatively impacted.

- We have changed the experimental regimen of SL-401 to a multi-cycle regimen, in which patients will receive more than one treatment cycle, rather than a single-cycle treatment as used in the completed investigator-initiated clinical trial. Although we anticipate that patients receiving multiple cycles of SL-401 may derive greater clinical benefit than from a single cycle, there is a risk of toxicity or a lack of efficacy arising from multiple cycles.

- We are, or may in the future be, treating patients with certain diseases or conditions that have not been previously treated with SL-401. In these instances, we may choose to treat patients at several different doses and use multi-cycle dosing regimens to determine the optimal doses and schedules for both near-term and long-term safety and disease control in each indication. Use of SL-401 in new disease populations and at new dosing regimens could produce unforeseen adverse reactions and events that could impact the development and ability to obtain marketing approval for SL-401.

- We may determine, based on safety and efficacy, that certain doses and regimens of SL-401 for particular indications are optimal for initial near-term therapy whereas the same, or other, doses and regimens are optimal for longer-term maintenance therapy.

- We are developing SL-701 as an injection administered under the skin, or subcutaneously, in our trials. Two previous investigator-sponsored trials of an earlier version of SL-701 used this method of delivery. Another previous investigator-sponsored trial of an earlier version of SL-701 used a different method of delivery, in which dendritic cells, which are a type of immune cell, were removed from the patient, exposed to immunogenic peptides, and then re-injected into or near a lymph node of the patient (intra/peri-nodally). Our plan continued the subcutaneous injection method used in two of the previous studies and represents a change from one of the previous studies.

- We manufactured and formulated SL-701 as a mixture of IL-13Ra2 mutant peptide, EphA2 peptide, a new survivin mutant peptide, and a tetanus toxoid peptide. An earlier version of this immunotherapy, which included IL-13Ra2 mutant and EphA2 peptides, was mixed with additional peptides in previous studies, including a different survivin peptide in some studies.

- In the initial stage of our SL-701 corporate-sponsored trial we used granulocyte-macrophage-colony-stimulating factor, or GM-CSF, and imiquimod as the immunostimulants. In the second stage of our SL-701 trial, we used poly-ICLC as the immunostimulant, which was the immunostimulant used, along with an earlier version of SL-701, in the previous investigator-sponsored study but is not currently commercially available. If the poly-ICLC regimen is found to be superior, it would require successful registration and commercialization of poly-ICLC in addition to SL-701 to support product launch, which would entail a more complicated regulatory and commercialization strategy than required for a single product launch.

- In some of our current or future trials, we may combine our product candidates with each other or with other therapies such as chemotherapy, radiation, targeted therapy, or anti-angiogenic therapy. We may not have yet clinically tested these combinations and the combinations could result in unforeseen toxicities. We are currently combining SL-401 with pomalidomide in myeloma and have combined SL-701 with bevacizumab and immunostimulants in brain cancer.

Any of the aforementioned, or other, changes could make the timing, including initiation, patient accrual, or results of our clinical trials or other future clinical trials less predictable and could cause our product candidates to perform differently, including causing toxicities, which could delay or suspend completion of our clinical trials, delay or prevent approval of our product candidates, and/or jeopardize our ability to obtain regulatory approval, commence product sales and generate revenues.

If we experience delays in the enrollment of patients in our clinical trials, our receipt of necessary regulatory approvals could be delayed or prevented.

We may not be able to continue clinical trials for our product candidates if we are unable to enroll a sufficient number of eligible patients to participate in these trials, including as required by the FDA or other regulatory authorities. Patient enrollment, a significant factor in the timing of clinical trials, is affected by many factors including the size and nature of the patient population, the proximity of patients to clinical sites, the eligibility criteria for the trial, the design of the clinical trial, competing clinical trials and clinicians' and patients' perceptions as to the potential advantages of the drug being studied in relation to other available therapies, including any new drugs that may be approved or may commence competing clinical trials for the indications we are investigating. SL-401 and SL-701 are being developed in hematologic and brain cancers, respectively, both of which are orphan indications (i.e., rare diseases) with small available study populations. SL-401 is being developed initially in BPDCN and other rare diseases, including certain myeloproliferative disorders, as well as AML, and SL-701 is being developed in brain cancer. Some of these represent orphan indications for which there are very limited independently reported data on annual incidences. If the prevalence of these diseases are very low, including lower than our estimates or estimates of our third-party contractors, this could significantly delay patient enrollment in any one or more of our ongoing or future clinical trials. SL-801 is being assessed in a number of advanced solid tumors, some of which may have low prevalence rates and which could significantly delay patient enrollment in any one or more of our ongoing or future clinical trials.

Further, if we fail to enroll and maintain the number of patients for which the clinical trial was designed, the statistical power of that clinical trial may be reduced, which would make it harder to demonstrate that the product candidate being tested in such clinical trial is safe and effective. Additionally, enrollment delays in our clinical trials may result in increased development costs for our product candidates, which would cause the value of our common stock to decline and limit our ability to obtain additional financing. 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 terminate or not initiate one or more clinical trials.

The regulatory review and approval processes of the FDA and comparable foreign authorities are lengthy, time-consuming and inherently unpredictable, and if we are ultimately unable to obtain regulatory approval for our product candidates, our business will be substantially harmed.

The time required to obtain approval by the FDA and comparable foreign authorities is unpredictable and depends upon numerous factors, including the substantial discretion of the regulatory authorities which could include the pre-requisite of an advisory panel, e.g. ODAC, review. In addition, approval policies, regulations, or the type and amount of preclinical, CMC, clinical pharmacology, and clinical data necessary to gain approval may change during the course of a product candidate's clinical development, and may vary among jurisdictions. We may be required to undertake and complete certain additional preclinical, CMC, clinical pharmacology, bioanalytical, immunogenicity or clinical studies to generate additional data required to support the submission of an Investigational New Drug application, or IND, or a BLA or an NDA to the FDA or comparable foreign authorities. An inadequacy in any of these areas, or a lack of personnel, financial resources or performance, including by third parties, could result in a delayed or unsuccessful regulatory filing. We have not obtained regulatory approval for any product candidate and it is possible that none of our existing product candidates or any product candidates we may seek to develop in the future will ever obtain regulatory approval. Furthermore, approval by the FDA does not ensure approval by regulatory authorities in other countries or jurisdictions, and approval by one foreign regulatory authority does not ensure approval by regulatory authorities in other foreign countries or by the FDA.

Our product candidates alone or in combination with any adjuvant, immunostimulant including GM-CSF or Imiquimod or poly-ICLC, or other agents with which we may combine our drug candidates, could fail to receive regulatory approval for many reasons, including the following:

- the FDA or comparable foreign regulatory authorities may disagree with the design, conduct or findings of our clinical trials;
- the FDA or comparable foreign regulatory authorities may identify protocol deviations or data quality or integrity concerns with our preclinical or clinical trials;
- we may be unable to demonstrate to the satisfaction of the FDA or comparable foreign regulatory authorities that a product candidate is safe and effective for its proposed indication;
- the results of clinical trials may not meet the level of statistical significance required by the FDA or comparable foreign regulatory authorities for approval;
- we may be unable to demonstrate that a product candidate's clinical and other benefits outweigh its safety risks;
- the FDA or comparable foreign regulatory authorities may disagree with our interpretation of data or the study design or execution from preclinical studies or clinical trials;
- the FDA or comparable foreign regulatory authorities may not accept our definition, or criteria, for primary endpoint and/or other endpoints for evaluation of clinical benefit, patient efficacy and potential marketing approval, despite meeting the primary endpoint of the trial;
- the data collected from clinical trials of our product candidates may not be sufficient to support the submission of a BLA or an NDA or other submission or to obtain regulatory approval in the United States or elsewhere;
- we may fail to secure an appropriate right of reference to the data from clinical trials of our product candidates that we did not sponsor;
- the FDA or comparable foreign regulatory authorities may fail to approve the manufacturing processes or facilities of third-party manufacturers with which we contract for clinical and commercial supplies;

- the FDA or comparable foreign regulatory authorities may fail to approve any companion diagnostics we develop; and
- the approval policies or regulations of the FDA or comparable foreign regulatory authorities may significantly change in a manner rendering our clinical data insufficient for approval.

This lengthy and costly review process as well as the unpredictability of future clinical trial results may result in our failing to obtain regulatory approval to market any of our other product candidates that we may advance into and through clinical trials, which would significantly harm our business.

In addition, even if we were to obtain approval, regulatory authorities may approve any of our product candidates for fewer or more limited indications than we request or may grant approval contingent on the performance of costly post-marketing commitments, including additional clinical trials, observation studies, and/or pregnancy registries which could impact market adoption and acceptance and exceed commercialization budgets. Regulatory authorities may also approve a product candidate with a label that includes labeling claims which may be undesirable for the successful commercialization of that product candidate, including product contraindications, warnings or precautions, the need for inpatient versus outpatient administration, or limitations on administration schedule such as number of infusions or cycles. In addition, we may not be able to ultimately achieve the price we intend to charge for our product candidates or obtain satisfactory reimbursement or coverage for our product candidates. Moreover, in many foreign countries, a product candidate must be approved for reimbursement before it can be approved for sale in that country and the reimbursement may be suboptimal. Any of the foregoing scenarios could materially harm the commercial prospects for our product candidates.

Our approach to the discovery and development of product candidates includes the targeting of cancer stem cells (CSC) which is unproven, and we do not know whether we will be able to develop any products of commercial value.

Research on CSCs is an emerging field and, consequently, there is ongoing debate regarding the importance of CSCs as an underlying cause of tumor initiation, propagation, recurrence, resistance, and metastasis. In addition, there is some debate in the scientific community regarding the defining characteristics of these cells.

Although there is general consensus that some cancer cells have tumor-initiating capacity, there also is some debate in the scientific community regarding the defining characteristics and the origin of these cells. Some believe that normal adult stem cells transform into CSCs. Others believe that non-CSC cancer cells can transform into CSCs and, therefore, a definitive CSC cannot be readily isolated or targeted. In addition, some believe that targeting CSCs should be sufficient for a positive clinical outcome, while others believe that, at times or always, targeting CSCs should be coupled with targeting tumor bulk for a positive clinical outcome.

Based on preclinical and clinical data, we believe that, for some cancers, SL-401 may target both tumor bulk and CSCs. However, it is conceivable that SL-401 and any other product candidates that we develop may not effectively target tumor bulk or CSCs or, even if they do, they may not have a beneficial or durable clinical outcome that outweighs the risks associated with the product. In addition, it is conceivable that our platform technology may ultimately fail to identify any commercially viable drugs to treat human patients with cancer or any other disease or condition.

If we are not successful in discovering, developing and commercializing additional product candidates, our ability to expand our business and achieve our strategic objectives may be impaired.

Although we expect to focus a substantial amount of our efforts on the continued clinical testing and regulatory interactions for our clinical stage drug candidates, SL-401, SL-801, and SL-701, another key element of our strategy is to identify and test additional compounds. A portion of the preclinical research that we are conducting involves new and unproven drug discovery methods, including our proprietary StemScreen® platform technology, as well as the preclinical testing of new compounds and potential new uses of existing compounds. The drug discovery that we are conducting using our StemScreen® platform technology may not be successful in identifying compounds that are useful in treating humans with cancer. Research programs designed to identify product candidates require substantial technical, financial and human resources, whether or not any product candidates are ultimately identified. Our research programs may initially show promise in identifying potential product candidates, yet fail to yield product candidates for clinical development or commercialization for many reasons, including the following:

- the research methodology used may not be successful in identifying potential product candidates;
- competitors may develop alternatives that render our product candidates obsolete;

- a product candidate may, on further study, be shown to have harmful effects or other characteristics that indicate it is unlikely to be safe and effective or otherwise does not meet applicable regulatory criteria;
- a product candidate may not be capable of being produced in commercial quantities at an acceptable cost, or at all; and
- a product candidate may not be accepted as safe and effective by regulatory authorities, patients, the medical community or third-party payors.

If we are unable to identify suitable compounds for preclinical and clinical development, we may not be able to obtain sufficient product revenues in future periods, which could result in significant harm to our financial position and adversely impact our stock price.

Even if we receive regulatory approval for any of our product candidates, we will be subject to ongoing FDA regulatory requirements, which require significant resources. Additionally, our product candidates, if approved, could be subject to labeling and other restrictions and market withdrawal and we may be subject to penalties if we fail to comply with regulatory requirements or experience unanticipated problems with our products.

Any regulatory approvals that we or our potential strategic partners receive for our product candidates may also be subject to limitations on the approved indicated uses for which the product may be marketed or to the conditions of approval, may contain product contraindications, warnings, or precautions that limit use of our product candidates or may contain requirements for potentially costly post-marketing testing, including Phase 4 clinical trials, and surveillance to monitor the safety and efficacy of our product candidates. In addition, if the FDA approves any of our product candidates, the manufacturing processes, testing, packaging, labeling, storage, distribution, field alert or biological product deviation reporting, adverse event reporting, advertising, promotion and recordkeeping for the product will be subject to extensive and ongoing regulatory compliance requirements. These requirements include submissions of safety and other post-marketing information and reports, as well as continued compliance with CGMPs for commercial manufacturing and good clinical practices, or GCPs, for any clinical trials that we conduct post-approval. In addition, any regulatory approvals covered under certain U.S. federal health care programs will trigger compliance with the Federal Physician Payment Sunshine Act reporting requirements and compliance with state marketing disclosure laws may also attach in certain jurisdictions. Later discovery of previously unknown problems with a product, including adverse events of unanticipated severity or frequency, or with our third-party manufacturers or manufacturing processes, or failure to comply with regulatory requirements, may result in, among other things:

- restrictions on the marketing or manufacturing of the product, withdrawal of the product from the market, or voluntary or mandatory product recalls;
- warning letters, 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;
- investigations or inspections by government entities, including FDA or foreign health authorities; and
- injunctions, fines, corporate integrity agreements, or the imposition of other civil penalties or criminal penalties.

We cannot predict the likelihood, nature or extent of government regulation that may arise from future legislation or administrative action, either in the United States or abroad. If we are slow or unable to adapt to changes in existing requirements or the adoption of new requirements or policies, or if we are not able to maintain regulatory compliance, we may lose any marketing approval that we may have obtained and we may not achieve or sustain profitability, which would adversely affect our business.

Risks Related to Our Financial Position and Capital Requirements

We have incurred net operating losses since our inception and anticipate that we will continue to incur substantial operating losses for the foreseeable future. We may never achieve or sustain profitability, which would depress the market price of our common stock, and could cause you to lose all or a part of your investment.

We have incurred net losses from operations from our inception through December 31, 2017 of approximately $215.7 million. We do not know whether or when we will become profitable. To date, we have not commercialized any products or generated any revenues from product sales. Our losses have resulted principally from costs incurred in development and discovery activities. We anticipate that our operating losses will substantially increase over the next several years as we execute our plan to expand our discovery, research, development and potential commercialization activities. We believe that our existing cash, cash equivalents, short-term investments and long-term investments including the cash proceeds received from our follow-on public offering during the first quarter of 2018, will be sufficient to fund our operations and our capital expenditures for at least the next two years. If our cash is insufficient to meet future operating requirements, we will have to raise additional funds. If we are unable to obtain additional funds on terms favorable to us or at all, we may be required to cease or reduce our operating activities or sell or license to third-parties some or all of our intellectual property. If we raise additional funds by selling additional shares of our capital stock, the ownership interests of our stockholders will be diluted. If we need to raise additional funds through the sale or license of our intellectual property, we may be unable to do so on terms favorable to us, if at all. In addition, if we do not continue to meet our diligence obligations under our license agreements for our product candidates that we have in-licensed, including SL-401, SL-801, and SL-701, we will lose our rights to develop and commercialize those product candidates.

If we do not successfully develop and obtain regulatory approval for our existing and future product candidates and effectively manufacture, market and sell any product candidates that are approved, we may never generate product sales, and even if we do generate product sales, we may never achieve or sustain profitability on a quarterly or annual basis. Our failure to become and remain profitable would 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 also could cause you to lose all or a part of your investment.

We will require additional financing to achieve our goals, and a failure to obtain this capital when needed could force us to delay, limit, reduce or terminate our product development or commercialization efforts.

Since our inception, most of our resources have been dedicated to the discovery, acquisition and preclinical and clinical development of our product candidates. We have expended and believe that we will continue to expend substantial resources for the development of SL-401, SL-801, and SL-701, and may expend additional resources on other product candidates and drug discovery and acquisition efforts. These expenditures will include costs associated with general administration, facilities, research and development, acquiring new technologies, manufacturing product candidates, conducting preclinical experiments and clinical trials, obtaining regulatory approvals, commercializing any products approved for sale, and costs associated with operating as a public company.

We have no significant current source of revenue to sustain our present activities, and we do not expect to generate revenue until, and unless, we obtain approval from the FDA or other regulatory authorities, and we successfully commercialize one or more of our compounds. As the outcome of our ongoing and future clinical trials is highly uncertain, our estimates of clinical trial costs necessary to successfully complete the development and commercialization of our product candidates may differ significantly from our actual costs. In addition, other unanticipated costs may arise. As a result of these and other factors currently unknown to us, we may need to seek additional funds sooner than planned, through public or private equity, debt financings or other sources, such as strategic partnerships and alliances and licensing arrangements. In addition, we may seek additional capital due to favorable market conditions or strategic considerations even if we believe we have sufficient funds for our current or future operating plans.

Our future capital requirements depend on many factors, including:

- the number and characteristics of the product candidates we pursue;
- the scope, progress, results and costs of researching and developing our product candidates, and conducting preclinical and clinical trials;
- the ability of our product candidates to progress through clinical development successfully;
- the timing of, and the costs involved in, seeking regulatory approvals for our product candidates;

- the cost of commercialization activities if any of our product candidates are approved for sale, including marketing, sales and distribution costs;
- the cost associated with securing and establishing commercialization and manufacturing capabilities for our product candidates and any products we successfully commercialize;
- our ability to establish and maintain strategic partnerships, licensing or other arrangements and the economic and other terms of such agreements;
- the costs involved in preparing, filing, prosecuting, maintaining, defending and enforcing patent claims, including litigation costs and the outcome of such litigation;
- the timing, receipt and amount of sales of, or royalties on, our future products, if any;
- our need and ability to hire additional management and scientific, medical, and sales and marketing personnel;
- the effect of competing technological and market developments; and
- our need to implement additional internal systems and infrastructure, including financial and reporting systems.

Additional funds may not be available 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:

- delay, limit, reduce or terminate preclinical studies, clinical trials or other research and development activities for one or more of our product candidates;
- delay, limit, reduce or terminate manufacturing of our product candidates; or
- delay, limit, reduce or terminate our establishment of sales and marketing capabilities or other activities that may be necessary to commercialize our product candidates and ensure their acceptance by third-party payors and the market.

We will need to raise additional funds to complete our clinical trials and achieve positive cash flow.

Raising additional capital may cause dilution to our existing stockholders, restrict our operations or require us to relinquish rights to our technologies or product candidates.

We will likely seek to raise additional capital through a combination of private and public equity offerings, debt financings, strategic partnerships and alliances and licensing arrangements. To the extent that we raise additional capital through the sale of equity or convertible debt securities, the ownership interests of existing stockholders will be diluted, and the terms may include liquidation or other preferences that adversely affect stockholder rights. Debt financing, if available, may involve agreements that include covenants limiting or restricting our ability to take certain actions, such as incurring debt, making capital expenditures or declaring dividends. If we raise additional funds through strategic partnerships and alliances and licensing arrangements with third-parties, we may have to relinquish valuable rights to our technologies or product candidates, or grant licenses on terms that are not favorable to us. If we are unable to raise additional funds through equity or debt financing when needed, we may be required to delay, limit, reduce or terminate our product development or commercialization efforts or grant rights to develop and market product candidates that we would otherwise prefer to develop and market ourselves.

Unstable market and economic conditions may have serious adverse consequences on our business, financial condition and stock price.

There can be no assurance that deterioration in credit and financial markets and confidence in economic conditions will not occur. Our general business strategy may be adversely affected by an economic downturn, a volatile business environment or an unpredictable and unstable market. If equity and credit markets deteriorate, it may make any necessary equity, debt, or other financing more difficult to secure, more costly, more dilutive, and less favorable to existing shareholders. Failure to secure any necessary financing in a timely manner and on favorable terms could have a material adverse effect on our growth strategy, financial performance and stock price and could require us to delay or abandon our business and clinical development plans. In addition, there is a risk that one or more of our current service providers, manufacturers and other partners may not survive these difficult economic times, which could directly affect

our ability to attain our operating goals on schedule and on budget. There is a possibility that our stock price may decline, due in part to the volatility of the stock market and the general economic downturn.

Risks Related to Our Business and Industry

We are a clinical stage company with no approved products, which makes it difficult to assess our future viability.

We are a clinical stage biopharmaceutical company with a limited operating history. We have not yet demonstrated an ability to successfully overcome many of the risks and uncertainties frequently encountered by companies in new and rapidly evolving fields, particularly in the biopharmaceutical area. For example, to execute our business plan, we will need to successfully:

- execute product candidate development activities;
- obtain required regulatory approvals for the development and commercialization of our product candidates;
- maintain, defend, leverage and expand our intellectual property portfolio;
- build, deploy, and maintain sales, distribution and marketing capabilities, either on our own or in collaboration with strategic partners should our products obtain market approval;
- gain market and third-party payor acceptance for our products should they obtain market approval;
- develop and maintain CGMP compliant manufacturing and distribution capabilities sufficient to support the intended scope of our preclinical and clinical development plans and the potential commercial demand for our product(s);
- complete required process characterization and validation activities to support any planned regulatory submission, which historically has included manufacture of at least 3 consecutive successful process validation batches for drug substance and at least 3 consecutive successful process validation batches for drug product;
- develop and maintain any strategic relationships we elect to enter into;
- satisfy our obligations under our license and other agreements; and
- manage our spending as costs and expenses increase due to drug discovery, preclinical development, clinical trials, regulatory approvals, manufacturing and commercialization.

If we are unsuccessful in accomplishing these objectives, we may not be able to develop product candidates, raise capital, expand our business or continue our operations.

We face substantial competition, which may result in others discovering, developing or commercializing products before, or more successfully, than we do.

Our future success depends on our ability to demonstrate and maintain a competitive advantage with respect to the design, development and commercialization of product candidates. Our competitors may succeed in developing competing products before we do for the same indications we are pursuing, obtaining regulatory approval for products or gaining acceptance for the same markets that we plan to target. If we are not “first to market” with one of our product candidates, our competitive position could be compromised because it may be more difficult for us to obtain marketing approval for that product candidate and successfully market that product candidate as a competitor. Even if we are “first to market” with one or more of our product candidates, a competitor could develop an alternative therapy for our approved indication(s) that demonstrates a superior efficacy and/or safety profile relative to our approved product(s).

We expect any product candidate that we commercialize will compete with products from other companies in the biotechnology and pharmaceutical industries. For example, there are a number of biopharmaceutical companies focused on developing therapeutics that may potentially compete including Astellas Pharma U.S., Inc., Bionomics Limited, Boehringer Ingelheim GmbH, Geron Corp., GlaxoSmithKline plc, Macrogenics Inc., Micromet, Inc. (an Amgen, Inc. company), OncoMed Pharmaceuticals, Inc., Pfizer Inc., Roche Holding AG, Sanofi U.S. LLC, Stemcentrx, Inc., Sumitomo Dainippon Pharma Co. Ltd., Verastem, Inc., and others. Additionally, there are a number of companies working to develop new treatments, which may compete with SL-401 and SL-801, including AbbVie, Agios, Inc., Ambit Biosciences Corporation (now a Daiichi Sankyo company), Amgen, Astex Pharmaceuticals

(now an Otsuka Pharmaceutical company), Celator Pharmaceuticals, Inc., Celgene Corporation, Cellectis, Cyclacel Pharmaceuticals, Inc., Eisai Co. Ltd., Genmab, Genzyme Corporation (now a Sanofi company) Immunogen, Janssen Pharmaceutical Companies of Johnson & Johnson, Karyopharm Therapeutics, Inc., MustangBio, Inc., Novartis AG, Seattle Genetics, Inc., Sunesis Pharmaceuticals, Inc., and Xencor, among others. There are also a number of drugs used for the treatment of brain cancer that may compete with SL-701, including, Avastin® (Roche Holding AG), Gliadel® (Eisai Co. Ltd.), and Temodar® (Merck & Co., Inc.). There are a number of companies working to develop brain cancer therapeutics with programs in clinical testing, including Agenus Inc., Bristol-Myers Squibb, Inc., Cortice Biosciences, Inc., Celldex Therapeutics, Inc., CytRx Corporation, GenSpera, Inc., GlaxoSmithKline plc., ImmunoCellular Therapeutics, Ltd., Northwest Biotherapeutics, Inc., Novartis AG, Roche Holding AG, and others.

Many of our competitors have substantially greater commercial infrastructures and financial, technical and personnel resources than we have. In addition, many are farther along in their clinical development programs. We may not be able to compete unless we successfully:

- design and develop products that address an unmet medical need or demonstrate a superior benefit/risk profile to other products in the market;
- conduct successful preclinical and clinical trials;
- attract qualified scientific, medical, sales and marketing and commercial personnel;
- obtain patent and/or other proprietary protection for our processes and product candidates;
- obtain required regulatory approvals; and
- collaborate with others in the design, development and commercialization of new products.

Established competitors may invest heavily to quickly discover and develop novel compounds that could make our product candidates obsolete. In addition, any new product that competes with an approved product must demonstrate compelling advantages in efficacy, convenience, tolerability and safety in order to overcome price competition and to be commercially successful. If we are not able to compete effectively against our current and future competitors, our business will not grow and our financial condition and operations will suffer.

If we fail to attract and keep senior management and key scientific and marketing personnel, we may be unable to successfully develop our product candidates, conduct our clinical trials and commercialize our product candidates.

Our success depends in part on our continued ability to attract, retain and motivate highly qualified management, clinical and scientific personnel. We are highly dependent upon our senior management as well as other employees, consultants and scientific and medical collaborators. As of March 16, 2018, we had 37 full-time employees, which may make us more reliant on our individual employees than companies with a greater number of employees. Although none of these individuals has informed us to date that he or she intends to retire or resign in the near future, the loss of services of any of these individuals or one or more of our other members of senior management could delay or prevent the successful development of our product pipeline, completion of our ongoing and future clinical trials or the commercialization and successful marketing launch of our product candidates.

Although we have not historically experienced unique difficulties attracting and retaining qualified employees, we could experience such problems in the future. For example, competition for qualified personnel in the biotechnology and pharmaceuticals field is intense. We will need to hire additional personnel as we expand our clinical development and commercial activities. We may not be able to attract and retain quality personnel on acceptable terms.

In addition, we rely on consultants and advisors, including scientific and clinical advisors, to assist us in formulating our research and development and commercialization strategy. Our consultants and advisors may also be engaged with employers other than us and may have commitments under consulting or advisory contracts with other entities that may limit their availability to us.

If our employees or third parties acting on our behalf commit fraud or other misconduct, including noncompliance with regulatory standards and requirements, our business may experience serious adverse consequences.

We are exposed to the risk of fraud or other misconduct by employees and third parties acting on our behalf. Misconduct by employees or third parties could include intentional failures to comply with FDA regulations, to provide accurate information to the FDA, to comply with manufacturing standards we have established, to comply with federal and state healthcare fraud and abuse laws

and regulations, to report financial information or data accurately or to disclose unauthorized activities to us. In particular, sales, marketing and business arrangements in the healthcare industry are subject to extensive laws and regulations intended to prevent fraud, kickbacks, self-dealing and other abusive practices. These laws and regulations may restrict or prohibit a wide range of pricing, discounting, marketing and promotion, sales commission, customer incentive programs and other business arrangements. Employee misconduct could also involve the improper use of information obtained in the course of clinical trials, which could result in regulatory sanctions and serious harm to our reputation. We have adopted a Code of Business Conduct and Ethics, but it is not always possible to identify and deter employee and third-party misconduct, and the precautions we take to detect and prevent this activity may not be effective in controlling unknown or unmanaged risks or losses or in protecting us from governmental investigations or other actions or lawsuits stemming from a failure to be in compliance with such laws or regulations. If any such actions are instituted against us, and we are not successful in defending ourselves or asserting our rights, those actions could have a significant impact on our business, including the imposition of significant fines or other sanctions.

We may encounter difficulties in managing our growth and expanding our operations successfully.

As we seek to advance our product candidates through clinical trials, we will need to expand our development, regulatory, manufacturing, marketing and sales capabilities or contract with third-parties to provide these capabilities for us. As our operations expand, we expect that we will need to identify, commence and manage additional relationships with various strategic partners, qualified suppliers, manufacturers and other third-parties. Future growth will impose significant added responsibilities on members of management. Our future financial performance and our ability to commercialize our product candidates and to compete effectively will depend, in part, on our ability to manage any future growth effectively. To that end, we must be able to manage our development efforts and clinical trials effectively and hire, train and integrate additional management, administrative and sales and marketing personnel. The hiring, training and integration of new employees may be more difficult, costly and/or time-consuming for us because we have fewer resources than a larger organization. We may not be able to accomplish these tasks, or to accomplish them in a timely fashion, and our failure to accomplish any of them could prevent us from successfully growing our company.

If product liability lawsuits are brought against us, we may incur substantial liabilities and may be required to limit commercialization of our product candidates.

We face an inherent risk of product liability as a result of the clinical testing of our product candidates and will face an even greater risk if we commercialize any products. For example, we may be sued if any product we develop allegedly causes or contributes to an injury or is found to be otherwise defective during product testing, clinical study, clinical use, manufacturing, marketing or sale. Any such product liability claims may include allegations of defects in manufacturing, defects in design, a failure to warn of dangers inherent in the product, negligence, strict liability and a breach of warranties. Fraud-based claims as well as claims made pursuant to under state consumer protection acts are also a possibility. If we cannot successfully defend ourselves against product liability claims, we may incur substantial liabilities or be required to limit commercialization of our product candidates. Even successful defense would require significant financial and management resources. Regardless of the merits or eventual outcome, liability claims may result in:

- decreased demand for our product candidates or products that we may develop;
- injury to our reputation;
- withdrawal of clinical trial participants and inability to enroll future clinical trial participants;
- costs to defend the related litigation;
- a diversion of management's time and our resources;
- substantial monetary awards to trial participants or patients;
- product recalls, withdrawals or labeling, marketing or promotional restrictions;
- loss of revenue;
- the inability to commercialize our product candidates; and
- a decline in our stock price.

Our inability to obtain and retain sufficient product liability insurance at an acceptable cost to protect against potential product liability claims could prevent or inhibit the commercialization of products we develop. Although we maintain liability insurance, any claim that may be brought against us could result in a court judgment or settlement in an amount that is not covered, in whole or in part, by our insurance or that is in excess of the limits of our insurance coverage. Our insurance policies also have various exclusions, and we may be subject to a product liability claim for which we have no coverage. We will have to pay any amounts awarded by a court or negotiated in a settlement that exceed our coverage limitations or that are not covered by our insurance, and we may not have, or be able to obtain, sufficient capital to pay such amounts.

Our relationships with customers and third-party payors in the United States and elsewhere will be subject or administrative to applicable anti-kickback, fraud and abuse and other healthcare laws and regulations, which could expose us to civil, criminal sanctions, civil penalties, contractual damages, reputational harm and diminished profits and future earnings.

Healthcare providers, physicians and third-party payors in the United States and elsewhere play a primary role in the recommendation and prescription of any product candidates for which we obtain marketing approval. Our future arrangements with third-party payors and customers may expose us to broadly applicable fraud and abuse and other healthcare laws and regulations that may constrain the business or financial arrangements and relationships through which we market, sell and distribute our products for which we obtain marketing approval. Restrictions under applicable federal, state and foreign healthcare laws and regulations include the following:

- the federal healthcare anti-kickback statute prohibits, among other things, persons from knowingly and willfully soliciting, offering, receiving or providing remuneration, directly or indirectly, in cash or in kind, to induce or reward either the referral of an individual for, or the purchase, order or recommendation of, any good or service for which payment may be made under federal and state healthcare programs such as Medicare and Medicaid;

- the federal False Claims Act imposes criminal and civil penalties, against individuals or entities for knowingly presenting, or causing to be presented, to the federal government, claims for payment that are false or fraudulent or making a false statement to avoid, decrease or conceal an obligation to pay money to the federal government and also includes provisions allowing for private, civil whistleblower or "qui tam" actions;

- the federal Health Insurance Portability and Accountability Act of 1996 ("HIPAA"), as amended by the Health Information Technology for Economic and Clinical Health Act ("HITECH"), imposes criminal and civil liability for executing a scheme to defraud any healthcare benefit program. HIPAA and HITECH also regulate the use and disclosure of identifiable health information by healthcare providers, health plans and healthcare clearinghouses, and impose obligations, including mandatory contractual terms, with respect to safeguarding the privacy, security and transmission of identifiable health information as well as requiring notification of regulatory breaches. HIPAA and HITECH violations may prompt civil and criminal enforcement actions as well as enforcement by state attorneys general;

- the federal false statements statute prohibits knowingly and willfully falsifying, concealing or covering up a material fact or making any materially false statement in connection with the delivery of or payment for healthcare benefits, items or services;

- the federal transparency requirements under the Affordable Care Act, or ACA, commonly referred to as the Sunshine Act, requires manufacturers of drugs, devices, biologics and medical supplies to report to the Department of Health and Human Services information related to U.S.-licensed physician and teaching hospital payments and other transfers of value including research payments and ownership and investment interests;

- analogous state laws and regulations, such as state anti-kickback and false claims laws, may apply to sales or marketing arrangements and claims involving healthcare items or services reimbursed by non-governmental third-party payors, including private insurers, and some state laws require pharmaceutical companies to comply with the pharmaceutical industry's voluntary compliance guidelines and the relevant compliance guidance promulgated by the federal government in addition to requiring drug manufacturers to report information related to payments to physicians and other healthcare providers or marketing expenditures; and

- analogous anti-kickback, fraud and abuse and healthcare laws and regulations in foreign countries.

The ACA broadened the reach of the fraud and abuse laws by, among other things, amending the intent requirement of the federal Anti-Kickback Statute and the applicable criminal healthcare fraud statutes contained within 42 U.S.C. Section 1320a-7b. Pursuant to the statutory amendment, a person or entity no longer needs to have actual knowledge of this statute or specific intent to violate it in order to have committed a violation. In addition, the ACA 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 or the civil monetary penalties statute. Many states have adopted laws similar to the federal Anti-Kickback Statute, some of which apply to the referral of patients for healthcare items or services reimbursed by any source, not only the Medicare and Medicaid programs.

Efforts to ensure that our business arrangements with third-parties will comply with applicable healthcare laws and regulations will involve substantial costs. It is possible that governmental authorities will conclude that our business practices do not comply with current or future statutes, regulations or case law involving applicable fraud and abuse or other healthcare laws and regulations. If our operations are found to be in violation of any of these laws or any other governmental regulations that may apply to us, we may be

subject to significant civil, criminal and administrative penalties, damages, fines, exclusion from government funded healthcare programs, such as Medicare and Medicaid, and the curtailment or restructuring of our operations. If any of the physicians or other providers or entities with whom we expect to do business are found to be not in compliance with applicable laws, they may be subject to criminal, civil or administrative sanctions, including exclusions from government funded healthcare programs.

If we fail to comply with environmental, health and safety laws and regulations, we could become subject to fines or penalties or incur costs that could have a material adverse effect on the success of our business.

We and our suppliers are subject to numerous environmental, health and safety laws and regulations, including those governing laboratory procedures and the handling, use, storage, treatment and disposal of hazardous materials and wastes. Our operations involve the use of hazardous and flammable materials, including chemicals and biological materials. Our operations also release hazardous waste. We generally contract with third-parties for the disposal of these materials and wastes. We cannot eliminate the risk of release, contamination or injury from these materials. In the event of release, contamination or injury resulting from our use of hazardous materials, we could be held liable for any resulting damages, and any liability could exceed our resources. We also could incur significant costs associated with civil or criminal fines and penalties.

Although we maintain workers' compensation insurance to cover us for costs and expenses we may incur due to injuries to our employees resulting from the use of hazardous materials, this insurance may not provide adequate coverage against potential liabilities. We do not maintain insurance for environmental liability or toxic tort claims that may be asserted against us in connection with our storage or disposal of biological, hazardous or radioactive materials.

In addition, we and our suppliers may incur substantial costs in order to comply with current or future environmental, health and safety laws and regulations. These current or future laws and regulations may impair our research, development or production efforts. Failure to comply with these laws and regulations also may result in substantial fines, penalties or other sanctions.

Risks Related to Commercialization of Our Product Candidates

If we are unable to establish or implement our own sales, marketing and distribution capabilities in a timely manner or enter into licensing or collaboration agreements for these purposes, we may not be successful in commercializing our product candidates.

We currently have a relatively small number of employees and do not have a sales or marketing infrastructure. Should we receive regulatory approval of any product candidates, we would be opportunistic in seeking to either build our own commercial infrastructure to commercialize SL-401, SL-801, SL-701 or any other future product candidates if and when they are approved, or enter into contract research, contract sales, or licensing or collaboration agreements to assist in the future development and commercialization of such product candidates.

To develop internal sales, distribution and marketing capabilities, we will have to invest significant amounts of financial and management resources, some of which will be committed prior to knowing that SL-401, SL-801, and/or SL-701 will be approved. For product candidates for which we decide to perform sales, marketing and distribution functions ourselves, we could face a number of additional risks, including:

- our inability to recruit, train, and retain adequate numbers of effective sales and marketing personnel;
- the inability of sales personnel to obtain access to physicians or effectively promote our approved products to physicians and other providers;
- the lack of complementary drug products to be offered by sales personnel, which may put us at a competitive disadvantage relative to companies with more extensive product lines;
- unforeseen costs and expenses associated with creating an independent sales and marketing organization;
- our inability to build our own commercial infrastructure to manufacture, market and sell our product candidates;
- our inability to build and staff, or enter a partnership to support, a commercial distribution capability; and
- the addressable market for our product candidates may result in unsatisfactory revenue.

Where and when appropriate, we may elect to utilize contract sales forces or strategic partners to assist in the commercialization of our product candidates. If we enter into arrangements with third-parties to perform sales, marketing and distribution services for our products, the resulting revenues or the profitability from these revenues to us are likely to be lower than if we had sold, marketed and distributed our products ourselves. In addition, we may not be successful in entering into arrangements with third-parties to sell, market and distribute our product candidates or may be unable to do so on terms that are favorable to us. We may have limited control over such third-parties, and any of these third-parties may fail to devote the necessary resources and attention to sell, market and distribute our products effectively and may engage in conduct that subjects us to significant regulatory enforcement action. Should we commercialize our product candidates on our own and build our own sales and marketing organization, to do so, there is also a risk that our employees may engage in conduct that subjects us to significant regulatory enforcement action. The sale of pharmaceutical products is subject to numerous regulatory and legal restrictions in promotional statements that may be made regarding a product's benefits and risks in addition to certain restrictions and limitations on interactions with health care professionals. If we do not establish sales, marketing and distribution capabilities successfully and in compliance with legal and regulatory requirements, either on our own or in collaboration with third-parties, we will not be successful in commercializing our product candidates.

Our commercial success depends upon attaining significant market acceptance of our product candidates, if approved, including SL-401, SL-801, and SL-701, among physicians and other healthcare providers, patients, third-party payors and, in the cancer market, acceptance by the operators of major cancer clinics.

Even if SL-401, SL-801, SL-701 or any other product candidate that we may develop or acquire in the future obtains regulatory approval, the product may not gain market acceptance among physicians, third-party payors, patients and the medical community. For example, current cancer treatments such as chemotherapy and radiation therapy are well established in the medical community, and doctors may continue to rely on these treatments. The degree of market acceptance of any products for which we receive approval for commercial sale depends on a number of factors, including:

- the efficacy and safety of our products, as demonstrated in clinical trials, and the degree to which our products represent a clinically meaningful improvement in care as compared with other available therapies;
- the clinical indications for which our products are approved and any limiting contraindications, warnings, and precautions;
- acceptance by physicians, operators of major cancer clinics and patients of our products as a safe and effective treatment;
- the willingness of the target patient population to try new therapies and of physicians to prescribe these therapies;
- the potential and perceived advantages of our products over alternative treatments;
- the cost of treatment in relation to alternative treatments;
- the availability of adequate reimbursement and pricing by third-parties and government authorities;
- the continued projected growth of oncology drug markets;
- relative convenience and ease of administration, including access to drug administration equipment such as syringe pumps;
- the requirement for in-patient versus out-patient dosing;
- the prevalence and severity of adverse events and side effects; and
- the effectiveness of our sales and marketing efforts.

If our approved drugs fail to achieve market acceptance, we would not be able to generate significant revenue. In addition, there are no guarantees that any approved product will be effective, or gain market acceptance, in additional indications.

Even if we are able to commercialize any product candidates, the products may become subject to unfavorable pricing regulations, third-party reimbursement practices or healthcare reform initiatives, which would harm our business.

The regulations that govern marketing approvals, pricing and reimbursement for new drug products vary widely from country to country, and are subject to changes interpretation, application and new legislative proposals at any time. Some countries require approval of the sale price of a drug before it can be marketed. In many countries, the pricing review period begins after marketing or product licensing approval is granted. In some foreign markets, prescription pharmaceutical pricing remains subject to continuing governmental control even after initial approval is granted. As a result, we might obtain marketing approval for a product in a particular country, but then be subject to price regulations that delay our commercial launch of the product, possibly for lengthy time periods, and negatively impact the revenues we are able to generate from the sale of the product in that country. Adverse pricing limitations may hinder our ability to recoup our investment in one or more product candidates, even if our product candidates obtain marketing approval.

Our ability to commercialize any products successfully also will depend in part on the extent to which reimbursement for these products and related treatments will be available from government health administration authorities, private health insurers and other organizations. Government authorities and third-party payors, such as private health insurers and health maintenance organizations, decide which medications they will cover and how much they will pay for them. A primary trend in the U.S. healthcare industry and elsewhere is cost containment. Government authorities and third-party payors have attempted to control costs by limiting coverage and the amount of reimbursement for particular medications. Increasingly, third-party payors are requiring that drug companies provide them with predetermined discounts from list prices and are challenging the prices charged for medical products. We cannot be sure that reimbursement will be available for any product that we commercialize and, if reimbursement is available, the level of reimbursement. Reimbursement may impact the demand for, or the price of, any product candidate for which we obtain marketing approval. Obtaining reimbursement for our products may be particularly difficult because of the higher prices often associated with drugs administered under the supervision of a physician. If reimbursement is not available or is available only to limited levels, we may not be able to successfully commercialize any product candidate for which we obtain marketing approval.

There may be significant delays in obtaining reimbursement for newly approved drugs, and coverage may be more limited than the purposes for which the drug is approved by the FDA or similar regulatory authorities outside the United States. Moreover, eligibility for reimbursement does not imply that any drug will be paid for in all cases or at a rate that covers our costs, including research, development, manufacture, sale and distribution. Interim reimbursement levels for new drugs, if applicable, may also not be sufficient to cover our costs and may not be made permanent. Reimbursement rates may vary according to the use of the drug and the clinical setting in which it is used, may be based on reimbursement levels already set for lower cost drugs and may be incorporated into existing payments for other services. Net prices for drugs may be reduced by mandatory discounts or rebates required by government healthcare programs or private payors and by any future relaxation of laws that presently restrict imports of drugs from countries where they may be sold at lower prices than in the United States. Third-party payors often follow Medicare coverage policy and payment limitations in setting their own reimbursement policies. Our inability to promptly obtain coverage and profitable payment rates from both government-funded and private payors for any approved products that we develop could have a material adverse effect on our operating results, our ability to raise capital needed to commercialize products and our overall financial condition.

Healthcare policy changes may have a material adverse effect on us.

Our business may be affected by the efforts of government and third-party payors to contain or reduce the cost of healthcare through various means. For example, the Patient Protection and Affordable Care Act and the Health Care and Education Affordability Reconciliation Act of 2010 (collectively, the ACA), enacted in March 2010, substantially changed the way healthcare is financed by both governmental and private insurers, and significantly impacted the pharmaceutical industry. With regard to pharmaceutical products, among other things, the ACA is expected to expand and increase industry rebates for drugs covered under Medicaid programs and make changes to the coverage requirements under the Medicare Part D program. The Supreme Court upheld the ACA in the main challenge to the constitutionality of the statute in 2012. The Supreme Court also upheld federal subsidies for purchasers of insurance through federally facilitated exchanges in a decision released in June 2015. Any remaining legal challenges to the ACA are viewed generally as not significantly impacting the implementation of the law if the plaintiffs prevail. Legislative proposals such as expanding the Medicaid drug rebate program to the Medicare Part D program, providing authority for the government to negotiate drug prices under the Medicare Part D program and lowering reimbursement for drugs covered under the Medicare Part B program have been raised in Congress but have been met with strong opposition and have not been enacted so far. The administration can rely on its existing statutory authority to make policy changes that could have an impact on the drug industry. For example, the Medicare program has in the past proposed to test alternative payment methodologies for drugs covered under the Part B program and finalized a proposal to pay hospitals less for Part B-covered drugs purchased through the 340B Drug Pricing Program effective January 1, 2018.

Modifications to or repeal of all or certain provisions of the ACA have been attempted in Congress as a result of the outcome of the recent presidential and congressional elections, consistent with statements made by the incoming administration and members of

Congress during the presidential and congressional campaigns and following the election. In January 2017, Congress voted to adopt a budget resolution for fiscal year 2017, or the Budget Resolution, that authorizes the implementation of legislation that would repeal portions of the ACA. The Budget Resolution is not a law. However, it is widely viewed as the first step toward the passage of legislation that would repeal certain aspects of the ACA. Further, on January 20, 2017, President Trump signed an Executive Order directing federal agencies with authorities and responsibilities under the ACA to waive, defer, grant exemptions from, or delay the implementation of any provision of the ACA that would impose a fiscal or regulatory burden on states, individuals, healthcare providers, health insurers, or manufacturers of pharmaceuticals or medical devices. In March 2017, following the passage of the budget resolution for fiscal year 2017, the U.S. House of Representatives passed legislation known as the American Health Care Act of 2017, which, if enacted, would amend or repeal significant portions of the ACA. Attempts in the Senate in 2017 to pass similar ACA repeal legislation, including the Better Care Reconciliation Act of 2017, were unsuccessful. However, in December 2017, the Tax Cuts and Jobs Act was enacted, which includes a provision that effectively repeals the ACA's individual mandate by reducing the tax penalty for failing to maintain minimum essential coverage to zero. Regardless of whether or not the ACA is changed or modified by Congress or the Supreme Court, we expect both government and private health plans to continue to require healthcare providers, including healthcare providers that may one day purchase our products, to contain costs and demonstrate the value of the therapies they provide.

Our therapeutic product candidates for which we intend to seek approval as biological products may face competition sooner than expected.

With the enactment of the Biologics Price Competition and Innovation Act of 2009, or BPCIA, as part of the ACA, an abbreviated pathway for the approval of biosimilar and interchangeable biological products was created. The new abbreviated regulatory pathway establishes legal authority for the FDA to review and approve biosimilar biologics, including the possible designation of a biosimilar as "interchangeable." The FDA defines an interchangeable biosimilar as a product that, in terms of safety or diminished efficacy, presents no greater risk when switching between the biosimilar and its reference product than the risk of using the reference product alone. Under the BPCIA, an application for a biosimilar product cannot be submitted to the FDA until four years, or approved by the FDA until 12 years, after the original brand product identified as the reference product was approved under a BLA. The new law is complex and is only beginning to be interpreted by the FDA and the courts. For example, in June 2017, the United States Supreme Court, in Sandoz, Inc. v. Amgen Inc., issued an opinion potentially impacting the previously understood effective market exclusivity period. As a result of its relatively recent passage and implementation, the BPCIA's ultimate impact, implementation and meaning are subject to uncertainty. While it is uncertain when any such processes may be fully adopted by the FDA, any such processes could have a material adverse effect on the future commercial prospects for our biological products.

We believe that if any of our product candidates, such as SL-401, were to receive marketing approval by the FDA as a biological product under a BLA, such an approved product(s) should qualify for the 12-year period of exclusivity. However, there is a risk that the U.S. Congress could amend the BPCIA to significantly shorten this exclusivity period potentially creating the opportunity for generic competition sooner than anticipated. Moreover, the extent to which a biosimilar, once approved, will be substituted for any one of our reference products in a way that is similar to traditional generic substitution for non-biological products is not yet clear, and will depend on a number of marketplace and regulatory factors that are still developing. In addition, a competitor could decide to forego the biosimilar route and submit a full BLA after completing its own preclinical studies and clinical trials. In such cases, any exclusivity to which we may be eligible under the BPCIA would not prevent the competitor from marketing its product as soon as it is approved.

Risks Related to Our Dependence on Third-Parties

Third-parties have conducted initial clinical trials of our product candidates in the past, and our ability to influence the design and conduct of such trials was limited. Our current and future corporate-sponsored trials will also require us to rely on various third-parties. Any failure by a third-party to meet its obligations with respect to the clinical and regulatory development of our product candidates may delay or impair our ability to obtain regulatory approval for our products.

We are currently advancing our clinical stage product candidates through multiple corporate-sponsored clinical trials under corporate-sponsored INDs. Previously, we had not sponsored any INDs or any clinical trials relating to SL-401, SL-801, or SL-701. Instead, faculty members at academic institutions conducted and sponsored all INDs and clinical trials relating to our drug candidates. Because the completed trials relating to our drug candidates were investigator-sponsored, we did not control the design or conduct of the previous trials, and it is possible that the FDA will not view these previous trials as providing adequate support for future clinical trials or regulatory filings, whether controlled by us or third-parties, for one or more reasons, including elements of the design or execution of the previous trials or safety concerns or other trial results.

In addition, we have relied on contractual arrangements with academic institutions and investigators that provide us certain information rights with respect to the completed investigator-sponsored trials, including access to and the ability to use and reference

the data, including for our own regulatory filings, resulting from the completed trials. If these obligations are breached by the investigators or institutions, or if the data prove to be inadequate then our ability to conduct our planned corporate-sponsored clinical trials may be adversely affected. Additionally, the FDA may disagree with our interpretation of the adequacy of preclinical, manufacturing, or clinical data from these clinical trials. If so, the FDA may require us to obtain and submit additional preclinical, manufacturing, or clinical data relating to our planned trials and/or may not accept such additional data as adequate for our regulatory filings.

We rely on, and expect to continue to rely on, third-parties to monitor, support, conduct and/or oversee clinical trials of our product candidates and, in some cases, to maintain regulatory files for our product candidates. If we are not able to maintain or secure agreements with such third-parties on acceptable terms to monitor, support, conduct and/or oversee these clinical trials, if these third-parties do not perform their services as required, or if these third-parties fail to timely transfer any regulatory information held by them to us, we may not be able to obtain regulatory approval for, or commercialize, our product candidates.

We rely on academic institutions, CROs, hospitals, clinics and other third-party collaborators who are outside our direct control to monitor, support, conduct and/or oversee preclinical and clinical studies of our product candidates. As a result, we have less control over the timing and cost of these studies and the ability to recruit trial subjects than if we had conducted these trials wholly by ourselves. In our corporate sponsored trials of SL-401, SL-801, and SL-701, we have continued to engage various third-parties. If we are unable to maintain or enter into agreements with these third-parties on acceptable terms, or if any such engagement is terminated, we may be unable to enroll patients on a timely basis or otherwise conduct our trials in the manner we anticipate. In addition, there is no guarantee that these third-parties will devote adequate time and resources to our studies or perform as required by contract or in accordance with regulatory requirements. If these third-parties fail to meet expected deadlines, fail to adhere to protocols or fail to act in accordance with regulatory requirements or our agreements with them, or if they otherwise perform in a substandard manner or in a way that compromises the quality or accuracy of their activities or the data they obtain, then clinical trials of our product candidates may be extended, delayed or terminated, and as a result we may not be able to commercialize our product candidates.

We may not be successful in establishing and maintaining strategic partnerships, which could adversely affect our ability to develop and commercialize products.

We may seek to enter into strategic partnerships in the future, including alliances with other biotechnology or pharmaceutical companies, to enhance and accelerate the development and commercialization of our products in territories outside the United States. We face significant competition in seeking appropriate strategic partners and the negotiation process is time-consuming and complex. Moreover, we may not be successful in our efforts to establish a strategic partnership or other alternative arrangements for any future product candidates and programs because our research and development pipeline may be insufficient, our product candidates and programs may be deemed to be at too early of a stage of development for collaborative effort and/or third-parties may not view our product candidates and programs as having the requisite potential to demonstrate safety and efficacy. Even if we are successful in our efforts to establish strategic partnerships, the terms that we agree upon may not be favorable to us and we may not be able to maintain such strategic partnerships if, for example, development or approval of a product candidate is delayed or sales of an approved product are disappointing.

If we ultimately determine that entering into strategic partnerships is in our best interest but either fail to enter into, are delayed in entering into or fail to maintain such strategic partnerships:

- the development of certain of our current or future product candidates may be terminated or delayed;
- our cash expenditures related to development of certain of our current or future product candidates would increase significantly and we may need to seek additional financing;
- we may be required to hire additional employees or otherwise develop expertise, such as sales and marketing expertise, for which we have not budgeted;
- we will bear all of the risk related to the development of any such product candidates;
- the competitiveness of any product candidate that is commercialized could be reduced; and
- with respect to our platform technology, StemScreen®, we may not realize its potential as a means of identifying and validating new cancer therapies.

We rely on third-party manufacturers to produce and supply our clinical and preclinical product candidates and we intend to rely on third-party manufacturers to produce commercial supplies of any approved products. Any failure by a third-party manufacturer to produce supplies for us may delay or impair our ability to initiate or complete our clinical trials, commercialize our products or continue to sell any products we commercialize.

We do not currently own or operate any manufacturing facilities, and we lack sufficient internal staff and infrastructure to produce clinical and preclinical product candidate supplies ourselves. As a result, we work with third-party CMOs to produce SL-401, SL-801, and SL-701 in acceptable quality and quantity for our ongoing and future clinical trials. If we are unable to maintain such third-party manufacturing sources, or fail to do so on commercially reasonable terms or on a timely basis, we may not be able to successfully produce, develop, and market SL-401, SL-801, and/or SL-701 or may be delayed in doing so. We purchase and plan to purchase immunostimulants used with SL-701 from third-parties. Whereas GM-CSF and Imiquimod are commercially available products, poly-ICLC (Hiltonol®) is a development stage candidate and not commercially available. We do not have a right to manufacture poly-ICLC directly or through third-party CMOs and are wholly dependent on a third-party manufacturer of poly-ICLC for clinical supply. This third-party manufacturer currently has a limited supply and may be unable to provide adequate poly-ICLC to us in the future.

We also expect to rely upon third-parties to produce drug substance and drug product required for the clinical trials and commercialization of our other product candidates in preclinical development. If we are unable to arrange for third-party manufacturing sources, or to do so on commercially reasonable terms or on a timely basis, we may not be able to complete development of such other product candidates or market them. Reliance on third-party manufacturers entails risks to which we would not be subject if we manufactured product candidates ourselves, including reliance on the third-party for regulatory compliance and quality assurance, the possibility of breach of the manufacturing agreement by the third-party because of factors beyond our control (including a failure to synthesize and manufacture our product candidates in accordance with our product specifications) and the possibility of termination or nonrenewal of the agreement by the third-party, based on its own business priorities, at a time that is costly or damaging to us. We will be dependent on the ability of these third-party manufacturers to produce adequate supplies of drug product to support our clinical development programs and future commercialization of our product candidates. In addition, the FDA and other regulatory authorities require that our product candidates be manufactured according to CGMP and similar foreign standards. Any failure by our third-party manufacturers to comply with CGMP or failure to scale up manufacturing processes, including any failure to deliver sufficient quantities of product candidates of acceptable quality in a timely manner, could lead to a delay in, or failure to obtain, regulatory approval for trial initiation or marketing of any of our product candidates. In addition, such failure could be the basis for action by the FDA to withdraw approvals for product candidates previously granted to us and for other regulatory action, including recall or seizure, fines, imposition of operating restrictions, total or partial suspension of production or injunctions.

We rely on our third-party manufacturers to purchase from third-party suppliers the materials necessary to produce our product candidates for our clinical studies. There are a small number of suppliers for certain capital equipment and materials that we use to manufacture and test our drugs. Such suppliers may not sell these materials to our manufacturers at the times we need them or on commercially reasonable terms. We do not have 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. Although we generally do not begin a clinical trial unless we believe we have a sufficient supply of a product candidate to complete the clinical trial, any significant delay in the supply of a product candidate or the material components thereof for a clinical trial, including an ongoing clinical trial, due to the need to replace a third-party manufacturer could considerably delay completion of our clinical studies, product testing and potential regulatory approval of 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 impair our ability to generate revenues from the sale of our product candidates.

We are working with our contract manufacturers to optimize the manufacturing processes for drug substance and drug product of our product candidates so that these product candidates may be routinely produced in adequate quantities of adequate quality, and at an acceptable cost, to support our clinical trials and ultimate commercialization. Our manufacturers may not be able to control batch-to- batch variability below an acceptable threshold, increasing the risk of batch failures, which could cause significant delays and increased costs to our programs. Our manufacturers may not be able to manufacture our product candidates at a cost or in quantities or in a timely manner necessary to develop and commercialize them. If we successfully commercialize any of our drugs, we may be required to establish or access large-scale commercial manufacturing capabilities. In addition, assuming that our drug development pipeline increases and matures, we will have a greater need for clinical trial and commercial manufacturing capacity. To meet our projected needs for commercial manufacturing, third-parties with whom we currently work may need to increase their scale of production and/or we will need to secure additional suppliers.

Because of our reliance on contract manufacturers, we may choose to maintain a higher inventory of drug product and/or drug substance for any of our product candidates or approved products than would be necessary if we had direct control of the manufacturing assets.

We rely on a single third-party to manufacture and supply our drug substance and a single third-party to manufacture and supply our drug product for each of our product candidates. Any problems experienced by our third-party manufacturers or their vendors could result in a delay or interruption in the supply of our product candidate to us until the third-party manufacturer or its vendor cures the problem or until we locate and qualify an alternative source of manufacturing and supply.

The manufacturers of our product candidates require specialized equipment and utilize complicated production processes that would be difficult, time consuming and costly to duplicate. Thus, we only have one third-party manufacturer for each of our product candidates. Because of this arrangement, there is a greater risk that issues in execution or changes in business focus and/or product risk assessments at a third-party manufacturer could cause delays in the clinical development or manufacture of a product candidate than if we used more than one third-party manufacturer for each product candidate. For each of our product candidates, we currently rely on third-party manufacturers to purchase from their third-party vendors the materials necessary to manufacture our product candidates for our clinical studies. Any prolonged disruption in our third-party manufacturers vendor's ability to supply materials for our manufacturing could have a significant negative impact on our ability to manufacture products on our own and would cause us to seek additional third-party manufacturing contracts, thereby increasing our development costs and timelines and any commercialization costs. In addition, our third-party manufacturers may experience problems not related to their vendors that could also have a significant negative impact on our ability to manufacture products on our own and would cause us to seek additional third-party manufacturing contracts, thereby increasing our development costs and timelines and any commercialization costs. Moreover, third-party manufacturers and third-party laboratories performing analytical and other testing could receive inspection findings from regulatory authorities that require investigation and remediation and could result in business interruptions affecting the production of our product candidates. We may face losses related to the supply of drug substances, drug product, adjuvants and other components of the product due to third-party distribution and storage of such product. We may suffer losses due to third-party manufacturer shortages or supply shortages of their vendors. We may suffer losses as a result of business interruptions that exceed coverage under our manufacturer's insurance policies. Events beyond our control, such as natural disasters, fire, sabotage or business accidents could have a significant negative impact on our operations by disrupting our product candidate development and commercialization efforts until our third-party manufacturer can repair its facility or we can put in place alternate third-party contract manufacturers to assume this manufacturing role, which we may not be able to do on reasonable terms, if at all. In addition, if we are required to change manufacturers for any reason, we will be required to verify that the new manufacturer maintains facilities and procedures that comply with quality standards and with all applicable regulations and guidelines and that they can successfully transfer our manufacturing processes to produce product of equivalent quality and quantity. FDA approval of any new manufacturer would also be required. The delays associated with the verification of a new manufacturer or the reverification of an existing manufacturer could negatively affect our ability to develop product candidates or produce approved products in a timely manner. Any delay or interruption in our clinical studies or in the development, validation and commercialization of our product candidates could negatively affect our business.

To the extent we elect to enter into licensing or collaboration agreements to develop and potentially commercialize our product candidates, our dependence on such relationships may adversely affect our business.

Our global commercialization strategy for certain of our product candidates may depend on our ability to enter into agreements with collaborators to obtain assistance and funding for the development and potential commercialization of these product candidates. Supporting diligence activities conducted by potential collaborators and negotiating the financial and other terms of a collaboration agreement are long and complex processes with uncertain results. Even if we are successful in entering into one or more collaboration agreements, collaborations may involve greater uncertainty for us, as we have less control over certain aspects of our collaborative programs than we do over our proprietary development and commercialization programs. We may determine that continuing to collaborate under the terms provided is not in our best interest, and we may terminate such collaboration. Our collaborators could delay or terminate their agreements, and our products subject to collaborative arrangements may never be successfully commercialized.

Further, our future collaborators may develop alternative products or pursue alternative technologies either on their own or in collaboration with others, including our competitors, and the priorities or focus of our collaborators may shift such that our programs receive less attention or resources than we would like, or they may be terminated altogether. Any such actions by our collaborators may adversely affect our business prospects and ability to earn revenues. In addition, we could have disputes with our future collaborators, such as the interpretation of terms in our agreements. Any such disagreements could lead to delays in the development or commercialization of any potential products or could result in time-consuming and expensive litigation or arbitration, which may not be resolved in our favor.

Even with respect to certain other programs that we intend to commercialize ourselves, we may enter into agreements with collaborators to share in the burden of conducting clinical trials, manufacturing and marketing our product candidates or products. In addition, our ability to apply our proprietary technologies to develop proprietary compounds will depend on our ability to establish and maintain licensing arrangements or other collaborative arrangements with the holders of proprietary rights to such compounds. We may not be able to establish such arrangements on favorable terms or at all, and our future collaborative arrangements may not be successful.

Risks Related to Our Intellectual Property Rights

We could be unsuccessful in obtaining adequate patent protection for one or more of our product candidates.

Our commercial success will depend in part on obtaining and maintaining patent protection and trade secret protection in the U.S. and other countries with respect to our product candidates or any future product candidate that we may license or acquire and the methods we use to manufacture them, as well as successfully defending these patents and trade secrets against third-party challenges. We seek to protect our proprietary position by filing patent applications in the United States and abroad related to our novel technologies and product candidates, and by maintenance of our trade secrets through proper procedures. We will only be able to protect our technologies from unauthorized use by third parties to the extent that valid and enforceable patents or trade secrets cover them in the market they are being used or developed. We cannot be certain that patents will be issued, or that issued or allowed patents will not later be found to be invalid and/or unenforceable. The patent prosecution process is expensive and time-consuming, and we may not be able to file and prosecute all necessary or desirable patent applications at a reasonable cost or in a timely manner. It is also possible that we will fail to identify any patentable aspects of our research and development output and methodology, and, even if we do, an opportunity to obtain patent protection may have passed. Given the uncertain and time-consuming process of filing patent applications and prosecuting them, it is possible that our product(s) or process(es) originally covered by the scope of the patent application may have changed or been modified, leaving our product(s) or process(es) without patent protection.

The patent position of biotechnology and pharmaceutical companies is generally highly uncertain, involves complex legal and factual questions, and has in recent years been the subject of much litigation. In addition, no consistent policy regarding the breadth of claims allowed in pharmaceutical or biotechnology patents has emerged to date in the U.S. The patent situation outside the U.S. is even more uncertain. The laws of foreign countries may not protect our rights to the same extent as the laws of the U.S., and we may fail to seek or obtain patent protection in all major markets. For example, European patent law restricts the patentability of methods of treatment of the human body more than U.S. law does. Publications of discoveries in the scientific literature often lag behind the actual discoveries, and patent applications in the U.S. and other jurisdictions are typically not published until 18 months after a first filing, or in some cases not at all. Therefore, we cannot know with certainty whether we or our licensors were the first to make the inventions claimed in patents or pending patent applications that we own or licensed, or that we or our licensors were the first to file for patent protection of such inventions. In the event that a third party has also filed a U.S. patent application relating to our product candidates or a similar invention, depending upon the priority dates claimed by the competing parties, we may have to participate in interference proceedings declared by the United States Patent and Trademark Office, or USPTO, to determine priority of invention in the U.S. The costs of these proceedings could be substantial and it is possible that our efforts to establish priority of invention would be unsuccessful, resulting in a material adverse effect on our U.S. patent position. As a result, the issuance, scope, validity, enforceability and commercial value of our patent rights are highly uncertain. Our pending and future patent applications may not result in patents being issued which protect our technology or products, in whole or in part, or which effectively prevent others from commercializing competitive technologies and products. Changes in either the patent laws or interpretation of the patent laws in the U.S. and other countries may diminish the value of our patents or narrow the scope of our patent protection. For example, the federal courts of the U.S. have taken an increasingly dim view of the patent eligibility of certain subject matter, such as naturally occurring nucleic acid sequences, amino acid sequences and certain methods of utilizing same, which include their detection in a biological sample and diagnostic conclusions arising from their detection. Such subject matter, which had long been a staple of the biotechnology and biopharmaceutical industry to protect their discoveries, is now considered, with few exceptions, ineligible in the first instance for protection under the patent laws of the U.S. Accordingly, we cannot predict the breadth of claims that may be allowed or enforced in our patents or in those licensed from a third-party.

Recent patent reform legislation could increase the uncertainties and costs surrounding the prosecution of our patent applications and the enforcement or defense of our issued patents. On September 16, 2011, the Leahy-Smith America Invents Act, or the Leahy-Smith Act, was signed into law. The Leahy-Smith Act includes a number of significant changes to United States patent law. These include changes to transition from a “first-to-invent” system to a “first-to-file” system and to the way issued patents are challenged. The formation of the Patent Trial and Appeal Board now provides a quicker and less expensive process for challenging issued patents. The USPTO recently developed new regulations and procedures to govern administration of the Leahy-Smith Act, and many of the substantive changes to patent law associated with the Leahy-Smith Act, and in particular, the first inventor-to-file provisions, only became effective on March 16, 2013. Accordingly, it is not clear what, if any, impact the Leahy-Smith Act will have on the operation of our business. However, the Leahy-Smith Act and its implementation could increase the uncertainties and costs surrounding the

prosecution of our patent applications and the enforcement or defense of our issued patents, all of which could have a material adverse effect on our business and financial condition.

Moreover, we may be subject to a third-party pre-issuance submission of prior art to the USPTO, or become involved in opposition, derivation, reexamination, *inter partes* review, post-grant review or interference proceedings challenging our patent rights or the patent rights of others. The costs of these proceedings could be substantial, and it is possible that our efforts to establish priority of invention would be unsuccessful, resulting in a material adverse effect on our U.S. patent position. An adverse determination in any such submission, patent office trial, proceeding or litigation could reduce the scope of, render unenforceable, or invalidate, our patent rights, allow third parties to commercialize our technology or products and compete directly with us, without payment to us, or result in our inability to manufacture or commercialize products without infringing third-party patent rights. In addition, if the breadth or strength of protection provided by our patents and patent applications is threatened, it could dissuade companies from collaborating with us to license, develop or commercialize current or future product candidates.

Even if our patent applications issue as patents, they may not issue in a form that will provide us with any meaningful protection, prevent competitors from competing with us or otherwise provide us with any competitive advantage. Our competitors may be able to circumvent our owned or licensed patents by developing similar or alternative technologies or products in a non-infringing manner.

Our patents and patent applications may not be sufficient to protect our products and product candidates from commercial competition. For example, we cannot obtain a composition of matter patent and are limited in the types of claims that we can obtain for SL-401 due to earlier published prior art. We have however obtained U.S. and foreign patents for certain methods of using SL-401 to treat AML, BPDCN, and myelodysplastic syndrome, or MDS. In addition, we have filed additional U.S. and foreign patent applications for the method of using SL-401 to treat AML, MDS, BPDCN, and other diseases although there can be no assurances that such patents will issue. Failure to obtain patents directed to all approved uses of SL-401 may enable a competitor to market SL-401 for such approved but unpatented indication(s), which could lead to price erosion for sales of SL-401. With respect to SL-701, although we have licensed an issued U.S. patent directed to the composition of matter for the mutant immunogenic IL-13Rα2 peptide, we currently do not have any foreign composition of matter patent protection. We do, however, have foreign pending patent applications, as well as an issued patent in Australia, that would cover certain uses of this peptide. While we have a non-exclusive license to issued U.S. patents directed to methods of use for the EphA2 peptide, we currently do not have any composition of matter patent protection, although we do have rights to foreign pending patent applications that seek to cover certain uses of this peptide. While we have filed U.S. and foreign patent applications directed to methods of use of a new survivin mutant peptide for use in SL-701, we currently do not have any composition-of-matter patent protection. With respect to SL-801, we have licensed composition of matter patents issued in the U.S. and abroad directed to the SL-801 compound. While we have patent applications pending in the U.S. and Canada directed to our StemScreen® technology, we currently have no issued patents covering StemScreen®. Although we have various patent applications pending in the U.S. and abroad that we anticipate may result in additional protection for SL-401, SL-801, SL-701 and StemScreen®, there can be no assurance that any of these applications will result in an issued patent, or that if they issue, they will provide additional meaningful protection for these assets. Our inability to obtain adequate patent protection for our product candidates or platform technology could adversely affect our business.

Issued patents covering one or more of our product candidates could be found invalid or unenforceable if challenged in court.

If we were to initiate legal proceedings against a third-party to enforce a patent covering one of our product candidates, the defendant could counterclaim that our patent is invalid and/or unenforceable. In patent litigation in the United States, defendant counterclaims alleging invalidity and/or unenforceability are commonplace. Grounds for a validity challenge could be an alleged failure to meet any of several statutory requirements, for example, lack of patentable subject matter, novelty, obviousness, written description or non-enablement. Grounds for an unenforceability assertion could be an allegation that someone connected with prosecution of the patent withheld relevant information from the USPTO, or made a misleading statement, during prosecution. The outcome following legal assertions of invalidity and unenforceability during patent litigation is unpredictable. Furthermore, any claims asserted against accused infringers could provoke those parties to petition the USPTO to institute inter partes review against the asserted patents, which may lead to a finding that all or some of the claims of the patent are invalid. With respect to the validity question, for example, we cannot be certain that there is no invalidating prior art, of which we and the patent examiner were unaware during prosecution. If a defendant were to prevail on a legal assertion of invalidity and/or unenforceability, we would lose at least part, and perhaps all, of the patent protection on one or more of our product candidates or certain aspects of our platform technology, StemScreen®. Such a loss of patent protection could have a material adverse impact on our business. Furthermore, adverse results on U.S. patents may affect related patents in our global portfolio.

The issuance of a patent does not foreclose challenges to its inventorship, scope, validity or enforceability. Therefore, our owned and licensed patents may be challenged in the courts or patent offices in the United States and abroad. Such challenges may result in loss of exclusivity or in patent claims being narrowed, invalidated or held unenforceable, in whole or in part, which could limit our ability

to stop others from using or commercializing similar or identical technology and products, or limit the duration of the patent protection of our technology and products. Given the amount of time required for the development, testing and regulatory review of new product candidates, patents protecting such product candidates might expire before or shortly after such product candidates are commercialized. As a result, our owned and licensed patent portfolio may not provide us with sufficient rights to exclude others from commercializing products similar or identical to ours.

Claims that our product candidates or StemScreen®, or the sale or use of our products or technology infringe the patent rights of third-parties could result in costly litigation or could require substantial time and money to resolve, even if litigation is avoided.

We cannot guarantee that our product candidates, the use of our product candidates, or our platform technology, StemScreen®, do not infringe third-party patents or other intellectual property. Third-parties might allege that we are infringing their patent rights or that we have misappropriated their trade secrets. Such third-parties might resort to litigation against us. The basis of such litigation could be existing patents or patents that issue in the future. Our failure to successfully defend against any claims that our product candidates or platform technology infringe the rights of third-parties could also adversely affect our business. Regardless of the outcome of any litigation, defending the litigation may be expensive, time-consuming and distracting to management. For example, we are aware of a third-party European patent with certain claims directed to one of the peptides used in SL-701. We may need to seek a license with respect to one or more of these third-party patents in order to commercialize our products. No assurance can be given that any such licenses will be available, or that they will be available on commercially acceptable terms. Failure to obtain any required licenses could restrict our ability to commercialize our products in certain territories or subject us to patent infringement litigation, could result in us having to cease commercialization of our products and/or subject us to money damages in such territories.

It is also possible that we have failed to identify relevant patents or applications. Patent applications covering our products or platform technology could have been filed by others without our knowledge. Additionally, pending patent applications which have been published can, subject to certain limitations, be later amended in a manner that could cover our platform technologies, our products or the use of our products.

In order to avoid or settle potential claims with respect to any patent rights of third-parties, we may choose or be required to seek a license from a third-party and be required to pay license fees or royalties or both. These licenses may not be available on acceptable terms, or at all. Even if we or any future strategic partners were able to obtain a license, the rights may be nonexclusive, which could result in our competitors gaining access to the same intellectual property. Ultimately, we could be prevented from commercializing one or more of our product candidates, or be forced to cease some aspect of our business operations, if, as a result of actual or threatened patent infringement claims, we are unable to enter into licenses on acceptable terms. Patent litigation could also expose us to significant monetary damages. This could harm our business significantly.

Defending against claims of patent infringement or misappropriation of trade secrets could be costly and time consuming, regardless of the outcome. Thus, even if we were to ultimately prevail, or to settle at an early-stage, such litigation could burden us with substantial unanticipated costs. In addition, litigation or threatened litigation could result in significant demands on the time and attention of our management team, distracting them from the pursuit of other Company business.

Unfavorable outcomes in intellectual property litigation could limit our research and development activities and/or our ability to commercialize certain products.

If third-parties successfully assert intellectual property rights against us, we might be barred from using certain aspects of our platform technology or barred from developing and commercializing certain products. Prohibitions against using certain technologies, or prohibitions against commercializing certain products, could be imposed by a court or by a settlement agreement between us and a patent owner. In addition, if we are unsuccessful in defending against allegations of patent infringement or misappropriation of trade secrets, we may be forced to pay substantial damage awards to the plaintiff. There is inevitable uncertainty in any litigation, including intellectual property litigation. There can be no assurance that we would prevail in any intellectual property litigation, even if the case against us is weak or flawed. If litigation leads to an outcome unfavorable to us, we may be required to obtain a license from the patent owner, in order to continue our research and development programs or to market our product(s). It is possible that the necessary license will not be available to us on commercially acceptable terms, or at all. This could limit our research and development activities, our ability to commercialize certain products, or both.

Most of our competitors are larger than we are and have substantially greater resources. They are, therefore, likely to be able to sustain the costs of complex patent litigation longer than we could. In addition, the uncertainties associated with litigation could have a material adverse effect on our ability to raise the funds necessary to continue our clinical trials, continue our internal research programs, in-license needed technology, or enter into strategic partnerships that would help us bring our product candidates to market. Such litigation or proceedings could substantially increase our operating losses and reduce the resources available for development

activities or any future sales, marketing or distribution activities. We may not have sufficient financial or other resources to conduct such litigation or proceedings adequately.

In addition, any future patent litigation, interference or other administrative proceedings will result in additional expense and distraction of our personnel. An adverse outcome in such litigation or proceedings may expose us or any future strategic partners to loss of our proprietary position, expose us to significant liabilities, or require us to seek licenses that may not be available on commercially acceptable terms, if at all.

Intellectual property litigation may lead to unfavorable publicity that harms our reputation and causes the market price of our common stock to decline.

During the course of any patent litigation, there could be public announcements of the results of hearings, rulings on motions, and other interim proceedings in the litigation. If securities analysts or investors regard these announcements as negative, the perceived value of our product candidates, platform technology, programs, or intellectual property could be diminished. Accordingly, the market price of our common stock may decline.

SL-401, SL-801, SL-701, as well as some of our other product candidates and our platform technologies, are protected by intellectual property licensed from third parties, including academic institutions. If the licensors terminate the licenses, or fail to prosecute, maintain, enforce, and/or defend the licensed patents and patent applications, our competitive position, market share, and business prospects would be harmed.

We are a party to several license agreements relating to certain patents and patent applications owned by third-parties, upon which certain aspects of our business depend. In particular, we hold an exclusive license from Scott and White Memorial Hospital, or Scott and White, for SL-401 and SL-501, and we hold three licenses, including an exclusive license and two non-exclusive licenses, from the University of Pittsburgh relating to SL-701. Our license agreement with Scott and White survives, unless earlier terminated, until the later of the expiration of the last to expire licensed patent or the date on which we owe no further payments to Scott and White. Our exclusive and our non-exclusive patent license agreements with the University of Pittsburgh survive, unless earlier terminated, until the expiration of the last to expire licensed patent, and our non-exclusive license with the University of Pittsburgh to use and reference certain clinical trial data and information survives for a term of twenty years unless earlier terminated. We hold an exclusive license from CanBas, Ltd. for SL-801 in all worldwide territories other than Japan, Korea, Taiwan, and China. The agreement with CanBas, Ltd. survives until the later of ten years following the first commercial sale of each product in each country; the date upon which there are no more valid claims; or the expiration or termination of the last regulatory exclusivity period, after which our license becomes fully paid, irrevocable, perpetual, non-exclusive and royalty-free. We also hold licenses from academic institutions relating to intellectual property underlying other product candidates and our StemScreen® platform technology. We expect to enter into additional license agreements as part of the development of our business.

We depend on our licensors to protect the proprietary rights covering our product candidates and we have limited, if any, control over the amount or timing of resources that they devote on our behalf, or the priority they place on, maintaining patent rights and prosecuting patent applications to our advantage. Moreover, we have limited, if any, control over the strategies and arguments employed in the maintenance of patent rights and the prosecution of patent applications to our advantage. Our current or future licensors may not successfully prosecute certain patent applications under which we are licensed and on which our business depends. Even if patents issue from these applications, our licensors may fail to maintain these patents, may decide not to pursue litigation against third-party infringers, may fail to prove infringement, or may fail to defend against counterclaims of patent invalidity or unenforceability. Moreover, and possibly unbeknownst to us, our licensors may experience serious difficulties related to their overall business or financial stability, and they may be unwilling or unable to continue to expend the financial resources required to maintain and prosecute these patents and patent applications. While we intend to take actions reasonably necessary to enforce our patent rights, we depend, in part, on our licensors to protect a substantial portion of our proprietary rights and to inform us of the status of those protections and efforts thereto.

Our licensors may also be notified of alleged infringement and be sued for infringement of third-party patents or other proprietary rights. We may have limited, if any, control or involvement over the defense of these claims, and our licensors could be subject to injunctions and temporary or permanent exclusionary orders in the U.S. or other countries. Our licensors are not obligated to defend or assist in our defense against third-party claims of infringement. We have limited, if any, control over the amount or timing of resources, if any, that our licensors devote on our behalf or the priority they place on defense of such third-party claims of infringement.

In addition, in spite of our best efforts, our licensors might conclude that we have materially breached our license agreements and might therefore seek to terminate the license agreements, thereby removing our ability to obtain regulatory approval and to market products covered by these license agreements. Our licensors may also seek to terminate the license agreements if we fail to satisfy our

diligence obligations and/or meet specified milestones or upon insolvency. From time to time, we have had to request extensions of our development obligations contained in some of our license agreements, and we may need to seek further extensions in the future. Although we have obtained such extensions in the past, there can be no assurance that our licensors will continue to extend the development timelines or other milestones contained in our license agreements. If these in-licenses are terminated, or if the underlying patents fail to provide the intended market exclusivity, we could lose our rights to develop and commercialize the product candidates governed by the licenses, and competitors would have the freedom to seek regulatory approval of, and to market, products identical to ours. This could have a material adverse effect on our competitive business position and our business prospects.

We could be unsuccessful in obtaining patent protection on one or more components of our platform technology.

We believe that an important factor in our competitive position relative to other companies in the field of targeted-oncology therapeutics is our proprietary innovative platform technology, StemScreen®. We believe that this platform is useful for identifying new potential product candidates. We have pending U.S. and Canadian patent applications for StemScreen®, however, there is no guarantee that any of such pending patent applications will result in issued patents, and, even if patents eventually issue, there is no certainty that the issued claims will have adequate scope to preserve our competitive position. In addition, by practicing our technology in jurisdictions where we do not have patent protection, third-parties could substantially weaken our competitive position in oncology research and development.

Confidentiality agreements with employees and third-parties may not prevent unauthorized disclosure of trade secrets and other proprietary information.

In addition to patents, we rely on trade secrets, technical know-how, and proprietary information concerning our business strategy in order to protect our competitive position in the field of oncology. In the course of our research and development activities and our business activities, we often rely on confidentiality agreements to protect our proprietary information. Such confidentiality agreements are used, for example, when we talk to vendors of laboratory or clinical development services or potential strategic partners. In addition, each of our employees is required to sign a confidentiality agreement upon joining our company. We take steps to protect our proprietary information, and our confidentiality agreements are carefully drafted to protect our proprietary interests. Nevertheless, there can be no guarantee that an employee or an outside party will not make an unauthorized disclosure of our proprietary confidential information. This might happen intentionally or inadvertently, and we may not be able to obtain adequate remedies for such breaches. It is possible that a competitor will make use of such information, and that our competitive position will be compromised, in spite of any legal action we might take against persons making such unauthorized disclosures.

Trade secrets are difficult to protect. Although we use reasonable efforts to protect our trade secrets, our employees, consultants, contractors, or outside scientific collaborators might intentionally or inadvertently disclose our trade secret information to competitors.

Enforcing a claim that a third-party illegally obtained and is using any of our trade secrets is expensive and time consuming, and the outcome is unpredictable. In addition, courts outside the United States sometimes are less willing than U.S. courts to protect trade secrets. Moreover, if any of our trade secrets were to be lawfully obtained or independently developed by a competitor, we would have no right to prevent them, or those to whom they communicate it, from using that technology or information to compete with us. If any of our trade secrets were to be disclosed to or independently developed by a competitor, our competitive position would be harmed.

Our research and development strategic partners may have rights to publish data and other information to which we have rights. In addition, we sometimes engage individuals or entities to conduct research relevant to our business. The ability of these individuals or entities to publish or otherwise publicly disclose data and other information generated during the course of their research is subject to certain contractual limitations. These contractual provisions may be insufficient or inadequate to protect our confidential information. If we do not apply for patent protection prior to such publication, or if we cannot otherwise maintain the confidentiality of our proprietary technology and other confidential information, then our ability to obtain patent protection or to protect our trade secret information may be jeopardized.

Intellectual property rights do not necessarily address all potential threats to our competitive advantage.

The degree of future protection afforded by our intellectual property rights is uncertain because intellectual property rights have limitations, and may not adequately protect our business, or permit us to maintain our competitive advantage. The following examples are illustrative:

- Others may be able to make compounds that are the same as or similar to our product candidates but that are not covered by the claims of the patents that we own or have exclusively licensed;

- We or our licensors or any future strategic partners might not have been the first to make the inventions covered by the issued patent or pending patent application that we own or have exclusively licensed;
- We or our licensors or any future strategic partners might not have been the first to file patent applications covering certain of our inventions;
- Others may independently develop similar or alternative technologies or duplicate any of our technologies without infringing our intellectual property rights;
- It is possible that our pending patent applications will not lead to issued patents;
- The scope of our issued patents may not extend to competitive products developed or produced by others;
- Issued patents that we own or have exclusively licensed may not provide us with any competitive advantages, or may be held invalid or unenforceable, as a result of legal challenges by our competitors;
- Our competitors might conduct research and development activities in countries where we do not have patent rights, or where the applicable laws provide a safe harbor exemption from infringement liability for certain research purposes, and then use the information learned from such activities to develop competitive products for sale in our major commercial markets;
- We may not develop additional proprietary technologies that are patentable; and
- The intellectual property rights of others may have an adverse effect on our business.

Changes in U.S. patent law could diminish the value of patents in general, thereby impairing our ability to protect our product candidates.

As is the case with other biopharmaceutical companies, our success is heavily dependent on intellectual property, particularly patents. Obtaining and enforcing patents in the biopharma industry involve both technological and legal complexities and is costly, time-consuming and inherently uncertain. In addition, in recent years, Congress has passed patent-reform legislation providing new or revised limitations on attaining, maintaining and enforcing patent rights in the U.S. Further, the Supreme Court has issued several decisions in patent cases in recent years, which either narrow the scope of patent protection or weaken the rights of patent owners in certain situations. In addition to increasing uncertainty with regard to our ability to obtain patents in the future, this combination of events has created uncertainty with respect to the value of patents, once obtained. Depending on decisions by the U.S. Congress, the federal courts, and the USPTO, the laws and regulations governing patents could change in unpredictable ways that could hinder our ability to obtain new patents or to enforce our existing patents and patents that we might obtain in the future.

Risks Related to Our Common Stock

The market price of our common stock may be highly volatile and our stockholders could incur substantial losses.

The market price of our common stock may be highly volatile, and could be subject to wide fluctuations in response to various factors, some of which are beyond our control. Since our initial public offering which occurred in January 2013, the price of our common stock has ranged from $3.88 per share to $47.25 per share. The stock market in general and the market for biopharmaceutical 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:

- results from or delays of clinical trials of our product candidates, as well as results of regulatory reviews relating to the approval of our product candidates;
- our decision to initiate a clinical trial, not to initiate a clinical trial or to terminate an existing clinical trial;
- our dependence on third-parties, including clinical research organizations and contract manufacturing organizations, trial sites, clinical trial sponsors and clinical investigators;
- our ability to commercialize our product candidates, if approved;

- the results of our efforts to discover, develop, acquire or in-license additional product candidates or products;
- new products, product candidates or new uses for existing products or technologies introduced or announced by our competitors and the timing of these introductions or announcements;
- regulatory or legal developments in the United States and other countries;
- our ability to maintain the license agreements for our product candidates;
- developments or disputes concerning patent applications, issued patents or other proprietary rights;
- the recruitment or departure of key scientific or management personnel;
- the level of expenses related to any of our product candidates or clinical development programs;
- actual or anticipated changes in estimates as to financial results, development timelines or recommendations by securities analysts;
- variations in our financial results or those of companies that are perceived to be similar to us;
- sales of common stock by us or our stockholders in the future, as well as the overall trading volume of our common stock;
- changes in the structure of healthcare payment systems and product pricing restrictions;
- market conditions in the pharmaceutical and biotechnology sectors;
- general economic, industry and market conditions and other factors that may be unrelated to our operating performance or the operating performance of our competitors, including changes in market valuations of similar companies; and
- the other factors described in this "Risk Factors" section.

We are an "emerging growth company" and the reduced disclosure requirements applicable to emerging growth companies could make our common stock less attractive to investors.

We are an "emerging growth company," as defined in the Jumpstart Our Business Startups Act of 2012, or the JOBS Act, and we have and intend to continue to take advantage of certain exemptions from various reporting requirements that are applicable to other public companies that are not "emerging growth companies" including not being required to comply with the auditor attestation requirements of Section 404(b) of the Sarbanes-Oxley Act, and exemptions from the requirements of holding a nonbinding advisory vote on executive compensation and shareholder approval of any golden parachute payments not previously approved. We cannot predict if investors will find our common stock less attractive because we have and may continue to rely on these exemptions. If some investors find our common stock less attractive as a result, there may be a less active trading market for our common stock and our stock price may be more volatile. We may take advantage of these reporting exemptions until we are no longer an "emerging growth company." We will remain an "emerging growth company" until the earliest of (i) the last day of the fiscal year in which we have total annual gross revenues of $1 billion or more; (ii) December 31, 2018; (iii) the date on which we have issued more than $1 billion in nonconvertible debt during the previous three years; or (iv) the date on which we are deemed to be a large accelerated filer under the rules of the Securities and Exchange Commission, or SEC.

Our executive officers, directors and principal stockholders maintain the ability to exert substantial influence over all matters submitted to stockholders for approval.

Our executive officers, directors and principal stockholders beneficially own shares representing approximately 31.1% of our outstanding capital stock. As a result, if these stockholders were to choose to act together, they would be able to exert substantial influence over all matters submitted to our stockholders for approval, as well as our management and affairs. For example, these persons, if they choose to act together, would exert substantial influence over the election of directors and approval of any merger, consolidation or sale of all or substantially all of our assets. This concentration of voting power could delay or prevent an acquisition of our company on terms that other stockholders may desire.

Provisions in our corporate charter documents and under Delaware law could make an acquisition of us, which may be beneficial to our stockholders, more difficult and may prevent attempts by our stockholders to replace or remove our current management.

Provisions in our corporate charter and our bylaws may discourage, delay or prevent a merger, acquisition or other change in control of us that stockholders may consider favorable, including transactions in which they might otherwise receive a premium for their shares. These provisions could also limit the price that investors might be willing to pay in the future for shares of our common stock, thereby depressing the market price of our common stock. Among other things, these provisions:

- establish a classified board of directors such that not all members of the board are elected at one time;
- allow the authorized number of our directors to be changed only by resolution of our board of directors;
- limit the manner in which stockholders can remove directors from the board;
- establish advance notice requirements for stockholder proposals that can be acted on at stockholder meetings and nominations to our board of directors;
- require that stockholder actions must be effected at a duly called stockholder meeting and prohibit actions by our stockholders by written consent;
- limit who may call special stockholder meetings and the matters transacted at such meetings;
- authorize our board of directors to issue preferred stock without stockholder approval, which could be used to institute a "poison pill" that would work to dilute the stock ownership of a potential hostile acquirer, effectively preventing acquisitions that have not been approved by our board of directors; and
- require the approval of the holders of at least 75% of the votes that all our stockholders would be entitled to cast to amend or repeal certain provisions of our charter or bylaws.

Moreover, because we are incorporated in Delaware, we are governed by the provisions of Section 203 of the Delaware General Corporation Law, which prohibits a person who owns in excess of 15% of our outstanding voting stock from merging or combining with us for a period of three years after the date of the transaction in which the person acquired in excess of 15% of our outstanding voting stock, unless the merger or combination is approved in a prescribed manner. Any provision in our corporate charter or our bylaws or Delaware law that has the effect of delaying or deterring a change in control could limit the opportunity for our stockholders to receive a premium for their shares of our common stock, and could also affect the price that some investors are willing to pay for our common stock.

We incur significant costs as a result of operating as a public company, and our management is required to devote substantial time to compliance initiatives.

As a public company, we incur and will continue to incur significant legal, accounting and other expenses. We are subject to the reporting and other requirements of the Securities Exchange Act of 1934, as amended, or the Exchange Act, the Sarbanes Oxley Act of 2002, or the Sarbanes Oxley Act, and the Dodd Frank Wall Street Reform and Protection Act, as well as rules subsequently adopted by the SEC and the NASDAQ Stock Market, or NASDAQ. These rules and regulations require, among other things, that we file annual, quarterly and current reports with respect to our business and financial condition and establish and maintain effective disclosure and financial controls and corporate governance practices. Changes in these rules and regulations can create uncertainty for public companies, increasing legal and financial compliance costs and making some activities more time consuming. Our management and other personnel devote a substantial amount of time to these compliance initiatives.

If we fail to maintain an effective system of internal control over financial reporting, we may not be able to accurately report our financial results or prevent fraud. As a result, stockholders could lose confidence in our financial and other public reporting, which would harm our business and the trading price of our common stock.

Effective internal controls over financial reporting are necessary for us to provide reliable financial reports and, together with adequate disclosure controls and procedures, are designed to prevent fraud. Any failure to implement required new or improved controls, or difficulties encountered in their implementation, could cause us to fail to meet our reporting obligations. In addition, any testing by us conducted in connection with Section 404 of the Sarbanes-Oxley Act, or the subsequent testing by our independent registered public accounting firm, may reveal deficiencies in our internal controls over financial reporting that are deemed to be material weaknesses or

that may require prospective or retroactive changes to our financial statements or identify other areas for further attention or improvement. Inferior internal controls could also cause investors to lose confidence in our reported financial information, which could have a negative effect on the trading price of our common stock.

We are required to disclose changes made in our internal controls and procedures on a quarterly basis and our management is required to assess the effectiveness of these controls annually. However, for as long as we are an "emerging growth company" under the JOBS Act, our independent registered public accounting firm will not be required to attest to the effectiveness of our internal controls over financial reporting pursuant to Section 404. We could be an emerging growth company until December 31, 2018. An independent assessment of the effectiveness of our internal controls could detect problems that our management's assessment might not. Undetected material weaknesses in our internal controls could lead to financial statement restatements and require us to incur the expense of remediation.

We do not expect to pay dividends on our capital stock in the foreseeable future.

We have never declared or paid cash dividends on our capital stock. We currently intend to retain all of our future earnings, if any, to finance the growth and development of our business, and we do not anticipate paying any cash dividends on our capital stock in the foreseeable future. In addition, the terms of any future debt agreements may preclude us from paying dividends. As a result, capital appreciation, if any, of our common stock will be your sole source of gain for the foreseeable future.

If securities or industry analysts do not publish research or publish inaccurate or unfavorable research about our business, our stock price and trading volume could decline.

The trading market for our common stock will depend in part on the research and reports that securities or industry analysts publish about us or our business. If one or more of the analysts who cover us downgrade our stock or publish inaccurate or unfavorable research about our business, our stock price would likely decline. If one or more of these analysts cease coverage of our company or fail to publish reports on us regularly, demand for our stock could decrease, which might cause our stock price and trading volume to decline.

Item 1B. Unresolved Staff Comments

None.

Item 2. Properties

Our corporate and executive office is located in New York, New York. Our New York facility consists of subleased space at 750 Lexington Avenue, Eleventh Floor, New York, New York 10022.

Item 3. Legal Proceedings

On March 15, 2018, the United States District Court for the Southern District of New York granted the defendants' motion to dismiss in its entirety a consolidated shareholder action against the Company, its directors, certain of its officers, and Jefferies LLC. All claims in the Amended Complaint are to be dismissed with prejudice unless Plaintiffs file for leave to amend their complaint within thirty days of March 15, 2018.(i)

(i) In February 2017, four putative class action lawsuits were filed against the Company and certain of its officers and directors in the Southern District of New York, alleging violations of the Exchange Act and Rule 10b-5 promulgated thereunder and violations of Section 11 and 15 of the Securities Act of 1933, or the Securities Act, arising from our January 2017 follow-on public offering. Each of lawsuits was premised upon allegations that the defendants made false and misleading statements and/or omissions by failing to earlier disclose that a cancer patient in a Stemline clinical trial of SL-401 who experienced the side effect of CLS died on January 18, 2017. Additionally, the complaints alleged that, as a result of the foregoing, certain of the defendants' statements about Stemline's business, operations, and prospects were materially false and misleading and/or lacked a reasonable basis. In April 2017, the United States District Court for the Southern District of New York consolidated these four shareholder actions into a single action, and appointed three purported individual investors in the Company as Lead Plaintiff to represent the proposed class. This class appointed Pomerantz LLP and the Rosen Law Firm as Co-Lead Counsel. On June 26, 2017, lead Plaintiffs filed an amended complaint in the consolidated action, naming as defendants, the Company, its directors, certain of its officers, and Jefferies LLC, and alleging violations of the Exchange Act and Rule 10b-5 promulgated thereunder during the period from January 20, 2017 through February 1, 2017, as well as violations of the Securities Act arising from our January 2017 follow-on public offering. On March 15, 2018, the United States District Court for the Southern District of New York granted the defendants' motion to dismiss the consolidated action in its entirety. All claims in the Amended Complaint are to be dismissed with prejudice unless Plaintiffs file for leave to amend their complaint within thirty days of March 15, 2018.

On May 2, 2017, a shareholder derivative litigation was filed in New York Supreme Court in New York County against all of our directors and certain of our officers in litigation captioned Hancock v. Bergstein. We are named only as a nominal defendant. The suit alleges that the officers and directors breached their fiduciary duties to the Company, unjustly enriched themselves, wasted corporate assets, abused their control, and grossly mismanaged the Company. The claims are based on allegations that the defendants engaged in improper conduct by failing to disclose in connection with its follow-on public offering of stock on January 20, 2017, that a cancer patient in a Stemline clinical trial died on January 18, 2017. It is alleged that the non-disclosure of that adverse event in the follow-on public offering has led us to incur losses, including defense and investigation costs, and allowed the defendants to reap substantial financial rewards in the form of bonuses and other compensation.

We intend to vigorously defend against these actions. However, there is no assurance that we will be successful in our defense or that insurance will be available or adequate to fund any settlement or judgment or the litigation costs of the actions. We are unable to predict the outcome or reasonably estimate a range of possible loss at this time on March 15, 2018.

Item 4. Mine Safety Disclosures

Not applicable.

Part II

Item 5. Market for the Registrant's Common Equity, Related Stockholder Matters and Issuer Purchases of Equity Securities

Market Information

Our common stock is listed on the NASDAQ Capital Market and trades under the symbol "STML" and has been publicly traded since January 31, 2013. Prior to that time, there was no public market for our common stock. The following table sets forth the high and low sales prices for our common stock as reported on the NASDAQ Capital Market for the periods indicated.

	High	Low
2016		
First Quarter	$ 6.50	$ 3.88
Second Quarter	9.66	4.62
Third Quarter	11.29	6.40
Fourth Quarter	14.60	10.08
2017		
First Quarter	$ 14.25	$ 5.50
Second Quarter	9.65	7.30
Third Quarter	11.70	7.30
Fourth Quarter	16.00	10.20

Holders

The number of record holders of our common stock as of March 16, 2018, was 137. This number does not include beneficial owners whose shares are held by nominees in street name.

Dividends

We have never declared or paid any cash dividends on our common stock and do not anticipate paying any cash dividends in the foreseeable future. Any future determination to pay dividends will be at the discretion of our board of directors.

Securities Authorized for Issuance under Equity Compensation Plans

The following table contains information about our equity compensation plans as of December 31, 2017.

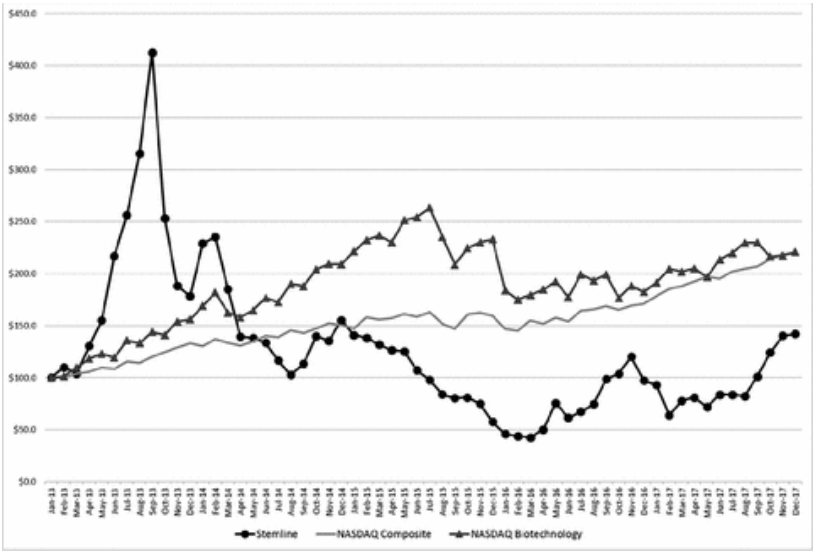
Equity Compensation Plan Information

Plan Category	Number of securities to be issued upon exercise of outstanding options and restricted stock (a)	Weighted-average exercise price of outstanding options (b)	Number of securities remaining available for future issuance under equity compensation plans (excluding securities reflected in column (a)) (c)
Equity compensation plans approved by security holders			
Options	3,174,964	$ 8.74	1,089,975
Restricted stock	1,724,837	N/A	—
Equity compensation plans not approved by security holders	—	—	—
Total	4,899,801	—	1,089,975

Common Stock Performance Graph

The following graph compares the cumulative total stockholder return on our common stock for the period from January 28, 2013 through December 31, 2017, with the cumulative total return over such period on (i) the U.S. Index of The NASDAQ Stock Market and (ii) the Biotechnology Index of The NASDAQ Stock Market. The graph assumes an investment of $100 on January 28, 2013, in our common stock (at the closing market price) and in each of the indices listed above, and assumes the reinvestment of dividends.

COMPARISON OF 5 YEARS CUMULATIVE TOTAL RETURN*
Among Stemline Therapeutics, Inc., the NASDAQ Composite Index
and the NASDAQ Biotechnology Index



* $100 invested on January 28, 2013 in stock or index, including reinvestment of dividends.
Fiscal year ending December 31.

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Item 6. Selected Financial Data

You should read the following selected financial data together with our financial statements and the related notes appearing at the end of this Form 10-K and the "Management's Discussion and Analysis of Financial Condition and Results of Operations" section of this Form 10-K. We have derived the financial information from our audited financial statements. Our historical results for any prior period are not necessarily indicative of results to be expected in any future period.

	Year Ended December 31,				
	2017	2016	2015	2014	2013
Statement of operations data:					
Grant Revenue	$ 898,199	$ 1,041,354	$ 654,160	$ 335,287	$ 71,000
Operating expenses:					
Research and development	$ 50,242,386	$ 27,869,921	$ 29,458,676	$ 21,240,599	$ 16,178,744
General and administrative	19,214,207	12,056,890	8,828,843	8,084,580	7,871,719
Total operating expenses	69,456,593	39,926,811	38,287,519	29,325,179	24,050,463
Loss from operations	(68,558,394)	(38,885,457)	(37,633,359)	(28,989,892)	(23,979,463)
Other (expense) income	(6,330)	11,438	1,609	3,607	280,687
Interest expense	—	—	—	—	(516,871)
Interest income	736,330	545,718	387,889	156,310	19,136
Net loss before income taxes	$ (67,828,394)	$ (38,328,301)	$ (37,243,861)	$ (28,829,975)	$ (24,196,511)
Income tax benefit	—	25,296	—	—	—
Net loss	$ (67,828,394)	$ (38,303,005)	$ (37,243,861)	$ (28,829,975)	$ (24,196,511)
Net (loss) / income attributable to common stockholders per common share:					
Basic and Diluted	$ (2.94)	$ (2.15)	$ (2.15)	$ (2.23)	$ (2.35)
Weighted average number of common shares:					
Basic and Diluted	23,056,928	17,804,681	17,289,021	12,936,741	10,317,351

	As of December 31,				
	2017	2016	2015	2014	2013
Balance sheet data:					
Cash and cash equivalents	$ 4,795,098	$ 10,316,064	$ 13,376,196	$ 25,007,217	$ 44,200,420
Total assets	$ 67,006,168	$ 68,119,098	$ 98,215,623	$ 60,494,992	$ 85,281,196
Other liabilities	$ 96,826	$ 142,200	$ 648,190	$ 607,999	$ 643,000
Accumulated deficit	$ (204,275,690)	$ (135,742,607)	$ (97,439,602)	$ (60,195,741)	$ (31,365,766)
Total stockholders' equity	$ 47,070,429	$ 57,723,085	$ 88,111,956	$ 55,413,151	$ 79,624,388

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

Unless the context requires otherwise, references in this report to "Stemline," "Company," "we," "us" and "our" refer to Stemline Therapeutics, Inc.

The following discussion and analysis contains forward-looking statements about our plans and expectations of what may happen in the future. Forward-looking statements are based on a number of assumptions and estimates that are inherently subject to significant risks and uncertainties, and our results could differ materially from the results anticipated by our forward-looking statements as a result of many known or unknown factors, including, but not limited to, those factors discussed in "Item 1A. Risk Factors." See also the "Special Cautionary Notice Regarding Forward-Looking Statements" set forth at the beginning of this report.

You should read the following discussion and analysis in conjunction with "Item 6. Selected financial Data," "Item 8. Financial Statements and Supplementary Data," and our financial statements beginning on page F-1 of this report.

Overview

We are a clinical stage biopharmaceutical company focused on discovering, acquiring, developing and potentially commercializing innovative oncology therapeutics that target difficult to treat cancers. Our clinical pipeline product candidates include: SL-401, SL-801, and SL-701.

SL-401

SL-401 is a novel targeted therapy directed to the interleukin-3 receptor-α, or CD123, a target expressed on a wide range of malignancies. SL-401 has completed a pivotal Phase 2 trial in patients with blastic plasmacytoid dendritic cell neoplasm, or BPDCN. SL-401 is also being assessed in additional indications including Phase 1/2 trials in patients with high-risk myeloproliferative neoplasms, or MPNs, focused on chronic myelomonocytic leukemia, or CMML, and myelofibrosis, or MF, patients with acute myeloid leukemia, or AML, and a trial in combination with other agents in a Phase 1 trial in patients with relapsed/refractory multiple myeloma. In August 2016, SL-401 was granted Breakthrough Therapy Designation, or BTD, from the U.S. Food and Drug Administration, or FDA, for the treatment of BPDCN.

On October 31, 2017, we announced that the pivotal Phase 2 trial of SL-401 in BPDCN met its primary endpoint. The pivotal Phase 2 trial in BPDCN is an open label, non-randomized, single arm clinical trial. We believe this trial is the largest multicenter prospective study ever conducted in BPDCN. The trial enrolled 45 BPDCN (32 first line, 13 relapsed/refractory) patients and consisted of 3 Stages: Stage 1 (lead-in, dose escalation), Stage 2 (expansion), and Stage 3 (pivotal, confirmatory). To ensure ongoing patient access to SL-401, we are currently enrolling both first-line and relapsed/refractory BPDCN patients in an additional cohort, Stage 4. We believe the results of this trial could support pursuing marketing approval and we plan to include such results as part of a Biologics License Application, or BLA, that seeks U.S. marketing approval. We anticipate such a filing could be completed in the first half of 2018. If successful, we project marketing approval could be attained in the second half of 2018, or soon thereafter. Later this year, we anticipate feedback from the European Medicines Agency, or EMA, regarding a potential regulatory filing.

SL-801

SL-801 is a structurally novel, oral, small molecule, reversible inhibitor of Exportin-1, or XPO1, a nuclear transport protein implicated in a variety of malignancies. SL-801 has demonstrated preclinical in vitro and in vivo antitumor activity against a wide array of solid and hematologic cancers. SL-801's potential ability to reversibly bind XPO1 may offer the possibility to mitigate side effects and help optimize the therapeutic index. We are currently enrolling patients with advanced solid tumors in a Phase 1 dose escalation trial of single agent SL-801.

SL-701

SL-701 is an immunotherapy designed to direct the immune system to attack targets present on certain malignancies, including brain cancer. SL-701 is comprised of 3 short synthetic peptides that correspond to epitopes of targets including IL-13Rα2, EphA2, and survivin, present on brain cancer. We advanced SL-701 into a corporate sponsored Phase 2 trial in adult patients with second-line glioblastoma. In Stage 1 of this trial, SL-701 was administered as a single agent with the immunostimulants GM-CSF and Imiquimod. In Stage 2 of the trial, SL-701 was administered, with the immunostimulant poly-ICLC in combination with bevacizumab. Both stages of the trial have completed dosing and patients are being followed for outcomes including survival.

Financings

We have devoted substantially all of our resources to develop our product candidates, manufacture our product candidates, build our intellectual property portfolio, business plan, raising capital, and provide general and administrative support for these operations. We have generated minimal revenues to date, have not generated any revenue from product sales, and have funded our operations primarily through public and private sales of common stock and private sales of convertible preferred stock to our investors. From inception through December 31, 2017, we have received net proceeds of $213.9 million from the sale of common stock, $12.5 million from the sale of convertible preferred stock and $0.9 million from the issuance of convertible debt. The convertible preferred stock was retired in March 2010 and the convertible debt was converted into common stock in April 2013.

On January 26, 2018, we completed a fourth follow-on public offering, selling 3,700,000 shares at an offering price of $14 per share. Additionally, the underwriters exercised in full their over-allotment option to purchase an additional 555,000 shares at an offering price of $14 per share. Aggregate gross proceeds from the follow-on public offering, including the exercise of the over-allotment option, were $59.6 million, and net proceeds received after underwriting fees and offering expenses were approximately $55.5 million.

We have never been profitable and our net loss from operations was $67.8 million for the year ended December 31, 2017, $38.3 million for the year ended December 31, 2016 and $37.2 million for the year ended December 31, 2015. We expect to incur significant expenses and increasing operating losses for the foreseeable future. We expect our expenses to increase in connection with our ongoing activities, particularly as we advance our product candidates through preclinical activities and clinical trials to seek regulatory approval and, if approved, commercialize such product candidates. Accordingly, we may need additional financing to support our continuing operations. We will seek to fund our operations through public or private equity or debt financings or other sources. Adequate additional financing may not be available to us on acceptable terms, or at all. Our failure to raise capital as and when needed would have a negative impact on our financial condition and our ability to pursue our business strategy. We will need to generate significant revenues to achieve profitability, and we may never do so.

Litigation

On March 15, 2018, the United States District Court for the Southern District of New York granted the defendants' motion to dismiss in its entirety a consolidated shareholder action against the Company, its directors, certain of its officers, and Jefferies LLC. All claims in the Amended Complaint are to be dismissed with prejudice unless Plaintiffs file for leave to amend their complaint within thirty days of March 15, 2018.(i)

(i) In February 2017, four putative class action lawsuits were filed against the Company and certain of its officers and directors in the Southern District of New York, alleging violations of the Exchange Act and Rule 10b-5 promulgated thereunder and violations of Section 11 and 15 of the Securities Act of 1933, or the Securities Act, arising from our January 2017 follow-on public offering. Each of lawsuits was premised upon allegations that the defendants made false and misleading statements and/or omissions by failing to earlier disclose that a cancer patient in a Stemline clinical trial of SL-401 who experienced the side effect of CLS died on January 18, 2017. Additionally, the complaints alleged that, as a result of the foregoing, certain of the defendants' statements about Stemline's business, operations, and prospects were materially false and misleading and/or lacked a reasonable basis. In April 2017, the United States District Court for the Southern District of New York consolidated these four shareholder actions into a single action, and appointed three purported individual investors in the Company as Lead Plaintiff to represent the proposed class. This class appointed Pomerantz LLP and the Rosen Law Firm as Co-Lead Counsel. On June 26, 2017, lead Plaintiffs filed an amended complaint in the consolidated action, naming as defendants, the Company, its directors, certain of its officers, and Jefferies LLC, and alleging violations of the Exchange Act and Rule 10b-5 promulgated thereunder during the period from January 20, 2017 through February 1, 2017, as well as violations of the Securities Act arising from our January 2017 follow-on public offering. On March 15, 2018, the United States District Court for the Southern District of New York granted the defendants' motion to dismiss the consolidated action in its entirety. All claims in the Amended Complaint are to be dismissed with prejudice unless Plaintiffs file for leave to amend their complaint within thirty days of March 15, 2018.

On May 2, 2017, a shareholder derivative litigation was filed in New York Supreme Court in New York County against all of our directors and certain of our officers in litigation captioned Hancock v. Bergstein. We are named only as a nominal defendant. The suit alleges that the officers and directors breached their fiduciary duties to the Company, unjustly enriched themselves, wasted corporate assets, abused their control, and grossly mismanaged the Company. The claims are based on allegations that the defendants engaged in improper conduct by failing to disclose in connection with its follow-on public offering of stock on January 20, 2017, that a cancer patient in a Stemline clinical trial died on January 18, 2017. It is alleged that the non-disclosure of that adverse event in the follow-on public offering has led us to incur losses, including defense and investigation costs, and allowed the defendants to reap substantial financial rewards in the form of bonuses and other compensation.

We intend to vigorously defend against these actions. However, there is no assurance that we will be successful in our defense or that insurance will be available or adequate to fund any settlement or judgment or the litigation costs of the actions. We are unable to predict the outcome or reasonably estimate a range of possible loss at this time on March 15, 2018.

Financial Operations Overview

Revenue

We have not generated any revenue from product sales and we have generated minimal revenues to date, all relating to a $3.0 million research funding received to date from the Leukemia and Lymphoma Society, or LLS, where we recognized revenue of $0.9 million during 2017, $1.0 million during 2016 and $0.7 million during 2015. In the future, we may generate revenue from product sales, contingent on marketing approval for one of our product candidates and market acceptance of that product. In addition, to the extent we enter into licensing or collaboration arrangements, we may have additional sources of revenue.

If we fail to complete the development of our product candidates in a timely manner or obtain regulatory approval for them, our ability to generate future revenue, and our results of operations and financial position, would be materially adversely affected.

Research and Development Expenses

The following table shows our research and development expenses for the years ended December 31, 2017, 2016 and 2015:

	Year Ended December 31,		
	2017	2016	2015
SL-401	$ 33,855,807	$ 14,161,925	$ 13,750,593
SL-801	2,764,694	2,387,036	2,611,514
SL-701	1,211,241	2,536,927	3,156,116
Personnel expenses	10,276,948	8,022,193	8,923,118
Other expenses	2,133,696	761,840	1,017,335
Total research and development expenses	$ 50,242,386	$ 27,869,921	$ 29,458,676

Research and development expenses consist of costs associated with the development of our product candidates and our platform technology, which include:

- clinical trial costs;
- chemistry, manufacturing and controls, or CMC, related costs, particularly as they relate to process characterization and validation expenses for SL-401 as required to support BLA submission requirements
- nonclinical costs;
- regulatory costs including BLA related expenses;
- employee-related expenses, including salaries, benefits, travel and stock-based compensation expense, and consultant costs;
- costs associated with work contracted and conducted by third-party contract research organizations, or CROs, contract manufacturing organizations, or CMOs, academic institutions and consultants; and
- license fees and milestone payments related to in-licensed products and technology.

We expense research and development costs to operations as incurred. We account for nonrefundable advance payments for goods and services that will be used in future research and development activities as expenses when the services have been performed or when the goods have been received, rather than when the payments are made.

We use our employee and infrastructure resources across multiple research and development projects. We do not allocate employee-related expenses or depreciation to any particular project. The components of our research and development costs are described in more detail in "Results of Operations."

We anticipate that our research and development expenses will trend higher in future periods as we seek to complete development of our most advanced product candidates, SL-401, SL-801, and SL-701, prepare to commercialize SL-401, and continue to develop our other product candidates and our platform technology. We anticipate that the majority of our research and development expense will be devoted to the development of SL-401, SL-801, and SL-701.

The successful development of our product candidates is highly uncertain. As of this time, other than as discussed above, we cannot reasonably estimate or know the nature, timing and estimated costs of the efforts that will be necessary to complete development of our product candidates or the period, if any, in which material net cash inflows from our product candidates may commence. This is due to the numerous risks and uncertainties associated with developing drugs, including the uncertainty of:

- the scope, enrollment, rate of progress and costs of our planned, as well as any additional, clinical trials and other research and development activities;
- timing and results of future clinical trials;
- the potential benefits of our product candidates over other therapies;
- the potential safety risks of our product candidate compared to other therapies;
- the costs, timing and outcome of regulatory submissions and approvals;
- our ability to market and commercialize, either on our own or with strategic partners, and achieve market acceptance for any of our product candidates that we are developing or may develop in the future;
- our ability to manufacture, at a reasonable expense, adequate supplies or our product candidates for use in planned and future clinical trials and/or commercial distribution in the event of a successful regulatory approval; and
- the costs of preparing, filing, prosecuting, defending and enforcing patents and other intellectual property.

A change in the outcome of any of these or similar variables with respect to the development of a product candidate could mean a significant change in the costs and timing associated with the development of that product candidate. For example, if the FDA or other regulatory authority were to require us to conduct clinical trials beyond those which we currently anticipate will be required for the completion of clinical development of a product candidate, we could be required to expend significant additional financial resources and time on the completion of clinical development. A similar result could occur if we experience significant delays in the progress of, including enrollment in, any clinical trials.

General and Administrative Expenses

General and administrative expenses consist primarily of salaries and related costs for personnel, including stock-based compensation expense. The primary functions included in our general and administrative expenses are legal, finance, human resources, investor relations, commercial operations and business development departments. Other general and administrative expenses include facility costs, insurance expense and professional fees for legal, business development, consulting and accounting services.

We anticipate that our general and administrative expenses will be higher in future periods due to the build out of a commercial infrastructure and regulatory compliance systems to support a potential commercial product launch for SL-401 if an FDA or foreign equivalent health authority approval for marketing is obtained.

Interest Income

Interest income consists of interest earned on our cash, cash equivalents, short-term investments and long-term investments. Given the current interest rate environment and that our primary investment is in 100% U.S. Treasury and Agency securities and related money market funds coupled with FDIC-insured bank certificates of deposits, we expect interest income to be minimal in future years.

Critical Accounting Policies and Estimates

To understand our financial statements, it is important to understand our critical accounting policies and estimates. We prepare our financial statements in accordance with generally accepted accounting principles, or GAAP. The preparation of financial statements also requires us to make estimates and assumptions that affect the reported amounts of assets, liabilities, costs and expenses and related disclosures. We base our estimates on historical experience and on various other assumptions that we believe to be reasonable under the circumstances. Actual results could differ significantly from the estimates made by our management. To the extent that there are differences between our estimates and actual results, our future financial statement presentation, financial condition, results of operations and cash flows will be affected. We believe that the accounting policies discussed below are critical to understanding our historical and future performance, as these policies relate to the more significant areas involving management's judgments and estimates.

Our significant accounting policies are described in more detail in the notes to our financial statements appearing at the end of this Form 10-K. However, we believe that the following accounting policies are the most critical to aid you in fully understanding and evaluating our financial condition and results of operations.

Accrued Research and Development Expenses

As part of the process of preparing our financial statements, we are required to estimate our accrued expenses. This process involves reviewing quotations and contracts, identifying services that have been performed on our behalf and estimating the level of service performed and the associated cost incurred for the service when we have not yet been invoiced or otherwise notified of the actual cost. The majority of our service providers invoice us monthly in arrears for services performed or when contractual milestones are met. We make estimates of our accrued expenses as of each balance sheet date in our financial statements based on facts and circumstances known to us at that time. We periodically confirm the accuracy of our estimates with the service providers and make adjustments if necessary. The significant estimates in our accrued research and development expenses include fees paid to CROs and CMOs, consultants and other third-party organizations in connection with research and development and administrative activities for which we have not yet been invoiced.

We base our expenses related to CROs and CMOs on our estimates of the services received and efforts expended pursuant to quotes and contracts with CROs and CMOs that conduct research and development on our behalf. The financial terms of these agreements are subject to negotiation, vary from contract to contract and may result in uneven payment flows. There may be instances in which payments made to our vendors will exceed the level of services provided and result in a prepayment of the research and development expense. In accruing service fees, we estimate the time period over which services will be performed and the level of effort to be expended in each period. If the actual timing of the performance of services or the level of effort varies from our estimate, we may adjust the accrual or prepaid accordingly. Although we do not expect our estimates to be materially different from amounts actually incurred, our understanding of the status and timing of services performed relative to the actual status and timing of services performed may vary and could result in us reporting amounts that are too high or too low in any particular period.

Income Taxes

We use the liability method of accounting for income taxes as set forth in the authoritative guidance for income taxes. Under this method, we recognize deferred tax liabilities and assets for the expected future tax consequences of temporary differences between the respective carrying amounts and tax bases of our assets and liabilities.

We continue to assess our ability to realize our deferred tax assets, which primarily consist of net operating losses, or NOL, carry-forwards. In assessing our ability to realize these deferred tax assets, management considers whether it is more likely than not that some portion or all of the deferred tax assets will be realized. We establish valuation allowances when necessary to reduce deferred tax assets to the amounts expected to be realized. The factors used to assess the likelihood of realization include our latest forecast of future taxable income and available tax planning strategies that could be implemented to realize the net deferred tax assets. As of December 31, 2017, 2016 and 2015, our deferred tax assets had full valuation allowances on them as we did not have sufficient positive evidence to recognize such deferred tax assets.

The Internal Revenue Code of 1986, as amended (the "Code"), provides for a limitation of the annual use of net operating losses and other tax attributes (such as research and development tax credit carryforwards) following certain ownership changes (as defined by the Code) that could limit our ability to utilize these carryforwards. At this time, we have not completed a study to assess whether an ownership change under section 382 of the Code has occurred, or whether there have been multiple ownership changes since our formation, due to the costs and complexities associated with such a study. We may have experienced various ownership changes, as defined by the Code, as a result of past financing transactions. Accordingly, our ability to utilize the aforementioned carryforwards may be limited. Additionally, U.S. tax laws limit the time during which these carryforwards may be applied against future taxes. Therefore, we may not be able to take full advantage of these carryforwards for federal or state income tax purposes.

If any of our products are approved for commercial sale and we start to realize profitability, we may determine that there is sufficient positive evidence to support a reversal of, or decrease in, the valuation allowance on our deferred tax assets. If we were to reverse all or some part of our valuation allowance, our financial statements in the period of reversal would likely reflect an increase in assets on our balance sheet and a corresponding tax benefit to our statement of operations in the amount of the reversal.

As of December 31, 2017, we had net operating losses of $122.8 million for federal and $127.3 million for state purposes and research and development credits of $31.6 million which expire in 2023 through 2037.

We have applied Accounting Standards Codification (ASC) 740-10, *Accounting for Uncertainty in Income Taxes — an Interpretation of FASB Statement No. 109*, on January 1, 2007. We analyzed our tax position in all jurisdictions where we are required to file an income tax return and concluded that we do not have any material unrecognized tax benefits. We file U.S. income tax returns as well as tax returns for any state jurisdiction in which we are authorized to conduct business. Our policy is to recognize interest and penalties accrued on any unrecognized tax benefit within the provision for income taxes on the statement of operations. We have no interest or penalties accrued for any unrecognized tax benefits for any periods presented.

Our annual provision for income taxes and the determination of the resulting deferred tax assets and liabilities involve a significant amount of management judgment. Management's judgments, assumptions and estimates relative to the current provision for income taxes take into account current tax laws, our interpretation of current tax laws and possible outcomes of current and future audits conducted by foreign and domestic tax authorities. We operate within federal, state and international taxing jurisdictions and are subject to audit in these jurisdictions. These audits can involve complex issues that may require an extended period of time to resolve.

The Company is still in the process of analyzing the impact to the Company of the Tax Act. Where the Company has been able to make reasonable estimates of the effects for which its analysis is not yet complete, the Company has recorded provisional amounts.

Stock-Based Compensation

We account for stock-based compensation in accordance with the provisions of ASC 718, *Compensation—Stock Compensation*. Accordingly, compensation costs related to equity instruments granted are recognized over the requisite service periods of the awards on a straight-line basis at the grant-date fair value calculated by either using a Black-Scholes option pricing model for stock option valuations or the closing stock price on date of grant for restricted stock. Additionally, under the provisions of ASC 718, the Company accounts for forfeited awards as incurred and on the date the forfeiture or cancellation occurs.

For stock options and restricted stock granted as consideration for services rendered by non-employees, we recognize expense in accordance with the requirements of ASC Topic 505-50, *Equity Based Payments to Non-Employees*. Non-employee option and restricted stock grants that do not vest immediately upon grant are recorded as an expense over the vesting period of the underlying stock options and restricted shares. At the end of each financial reporting period prior to vesting, the value of stock options, as calculated using the Black-Scholes option-pricing model, is re-measured using the fair value of our common stock and the non-cash expense recognized during the period is adjusted accordingly. Restricted stock is also re-valued based upon the closing stock price at the end of each financial reporting period. Since the fair market value of options and restricted stock granted to non-employees is subject to change in the future, the amount of the future expense will include fair value re-measurements until the stock options and restricted stock are fully vested.

Revenue Recognition

We have not yet generated any revenue from product sales. Our sole source of revenue is grant revenue related to $3.0 million of research grants received to date from the Leukemia and Lymphoma Society. Grant payments received prior to our performance of work required by the terms of the research grant are recorded as deferred revenue and recognized as grant revenue once work is performed and qualifying costs are incurred.

Results of Operations

Comparison of Years Ended December 31, 2017 and 2016

Research and development expense. Research and development expense was $50.2 million for the year ended December 31, 2017, compared with $27.9 million for the year ended December 31, 2016, which represents an increase of $22.3 million. The higher costs were primarily driven by an increase of $14.4 million in manufacturing expense to support clinical trials and a BLA filing for SL-401 relating to the potential commercialization of SL-401. We also incurred $5.1 million in regulatory expenses to support a potential BLA filing for SL-401. Additionally, we incurred $2.2 million in higher cash and non-cash stock-based compensation expense resulting from an increase in headcount.

General and administrative expense. General and administrative expenses were $19.2 million for the year ended December 31, 2017, compared with $12.1 million for the year ended December 31, 2016, which represents an increase of $7.1 million. The higher costs were primarily attributable to $3.2 million of legal fees and by $2.3 million in pre-launch expenses in support of preparing for potential commercialization of SL-401, if marketing approval from the FDA is obtained. Also driving the increase in costs was $1.7 million of cash compensation and non-cash stock-based compensation expense.

Interest income. Interest income was $0.7 million for the year ended December 31, 2017, compared with $0.5 million for the year ended December 31, 2016. The increase in income of $0.2 million is primarily due to higher average cash and investment balances during 2017 versus the prior year.

Comparison of Years Ended December 31, 2016 and 2015

Research and development expense. Research and development expense was $27.9 million for the year ended December 31, 2016, compared with $29.5 million for the year ended December 31, 2015, which represents a decrease of $1.6 million. The lower costs were partially due to a $0.5 million decrease in clinical trial expenses for SL-701 resulting from the study attaining full patient enrollment during the third quarter of 2016.

General and administrative expense. General and administrative expenses were $12.1 million for the year ended December 31, 2016, compared with $8.8 million for the year ended December 31, 2015, which represents an increase of $3.3 million. The higher costs were primarily attributable to an increase in non-cash stock-based compensation expense and increased headcount.

Interest income. Interest income was $0.5 million for the year ended December 31, 2016, compared with $0.4 for the year ended December 31, 2015. The increase in income of $0.1 million is due to a change in the composition of our investment portfolio to include higher yielding FDIC insured certificates of deposit coupled with higher market interest rates.

Liquidity and Capital Resources

Sources of Liquidity

We have financed our operations to date primarily through proceeds from public sales of common stock via our 2013 IPO and subsequent follow-on offerings. To date, we have not generated any revenue from the sale of products. We have incurred losses and generated negative cash flows from operations since inception.

Since inception and through December 31, 2017, we received net proceeds of $213.9 million primarily from the public sale of common stock from our 2013 IPO and three subsequent follow-on public offerings. On January 26, 2018, we completed a fourth follow-on public offering, selling 3,700,000 shares at an offering price of $14 per share. Additionally, the underwriters exercised in full their over-allotment option to purchase an additional 555,000 shares at an offering price of $14 per share. Aggregate gross proceeds from the follow-on public offering, including the exercise of the over-allotment option, were $59.6 million, and net proceeds received after underwriting fees and offering expenses were approximately $55.5 million.

As of December 31, 2017, our cash, cash equivalents and short and long-term investments totaled $66.2 million. We primarily invest our cash, cash equivalents, short-term investments and long-term investments in U.S. Treasury and Agency securities and related money market funds and FDIC-insured bank certificates of deposit, with the balance in commercial bank operating accounts. We believe that our existing cash, cash equivalents, short-term investments and long-term investments including cash proceeds received from our follow-on offering in January 2018, will be sufficient to fund our operations for at least the next two years.

Cash Flows

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

	Year Ended December 31,		
	2017	2016	2015
Net cash used in operating activities	$ (49,618,794)	$ (30,092,774)	$ (25,589,442)
Net cash (used in) provided by investing activities	(4,437,050)	26,676,870	(50,922,458)
Net cash provided by financing activities	48,534,878	355,772	64,880,879
Net decrease in cash and cash equivalents	$ (5,520,966)	$ (3,060,132)	$ (11,631,021)

Operating activities. The use of cash in all periods resulted primarily from our net losses adjusted for stock-based compensation expense, non-cash depreciation expense and changes in the components of working capital. The net cash used in operating activities in 2017 and 2016 primarily resulted from research and development expenses as we continued our clinical trial activities relating to SL-401, SL-801, and SL-701. Additional research and development costs also include CMC-related expenses for the manufacture of drug substance and drug product of our product candidates in development.

Investing activities. The net cash provided by and used in financing activities for 2017 and 2016 reflects purchases and redemptions of short-term and long-term investments within our U.S. Treasury-related investment and bank certificate of deposit portfolios, net of maturities.

Financing activities. The net cash provided by financing activities for 2017 resulted primarily from our January 2017 issuance and sale of 5,175,000 shares of our common stock which generated gross cash proceeds of $51.8 million ($48.2 million cash proceeds, net of expenses). The net cash provided by financing activities for 2017, 2016 and 2015 was also impacted by the issuance of stock related to the 2015 Employee Stock Purchase Plan and exercise of employee and consultant stock options. The net cash provided by financing activities for 2015 resulted primarily from our January 2015 issuance and sale of 4,353,877 shares of our common stock which generated gross cash proceeds of $68.6 million ($64.1 million cash proceeds, net of expenses).

Funding Requirements

All of our product candidates are still in clinical or preclinical development. We expect to continue to incur significant expenses and increased operating losses for the foreseeable future. We anticipate that our expenses will increase if and as we:

- continue the ongoing clinical trials, and initiate the planned clinical trials, of our product candidates;
- continue the research and development of our other product candidates;
- seek to identify additional product candidates;
- acquire or in-license other products and technologies;
- seek marketing approvals for our product candidates that successfully complete pre-market clinical trials;
- establish, either on our own or with strategic partners, a manufacturing, sales, marketing and distribution infrastructure to commercialize any products for which we may obtain marketing approval;
- continue to incur legal expenses relating to our ongoing shareholder class action lawsuits;
- maintain, leverage and expand our intellectual property portfolio; and
- add operational, financial and management information systems and personnel, including personnel to support our product development and future commercialization efforts.

Our existing cash and cash equivalents will enable us to fund our operating expenses and capital expenditure requirements for at least the next two years. We have based this estimate on assumptions that may prove to be wrong, and we could use our available capital resources sooner than we currently expect. Because of the numerous risks and uncertainties associated with the development and commercialization of our product candidates, and the extent to which we may enter into collaborations with third-parties for development and commercialization of our product candidates, we are unable to estimate the amounts of increased capital outlays and

operating expenses associated with completing the development of our current product candidates. Our future capital requirements will depend on many factors, including:

- the progress and results of the ongoing and future clinical trials of our product candidates;
- the scope, progress, results and costs of compound discovery, preclinical development, laboratory testing and clinical trials for our product candidates now or in the future;
- the extent to which we acquire or in-license other products and technologies;
- the costs, timing and outcome of regulatory review of our product candidates;
- the costs of future commercialization activities, including product sales promotion, marketing, manufacturing and distribution, for any of our product candidates for which we receive marketing approval;
- revenue, if any, received from commercial sales of our product candidates, should any of our product candidates receive marketing approval;
- the progress of our ongoing shareholder class action lawsuits;
- the costs of preparing, filing and prosecuting patent applications, maintaining and enforcing our intellectual property rights and defending intellectual property-related claims; and
- our ability to establish any future collaboration arrangements on favorable terms, if at all.

Until such time, if ever, as we can generate substantial product revenues, we expect to finance our cash needs through a combination of equity offerings, debt financings, collaborations, strategic alliances and licensing arrangements. We do not have any committed external source of funds. To the extent that we raise additional capital through the sale of equity or convertible debt securities, the ownership interest of our stockholders will be diluted, and the terms of these securities may include liquidation or other preferences that adversely affect the rights of a common stockholder. Debt financing, if available, may involve agreements that include covenants limiting or restricting our ability to take specific actions, such as incurring additional debt, making capital expenditures or declaring dividends. If we raise additional funds through collaborations, strategic alliances or licensing arrangements with third-parties, we may have to relinquish valuable rights to our technologies, future revenue streams, research programs or product candidates or grant licenses on terms that may not be favorable to us.

If we are unable to raise additional funds through equity or debt financings when needed, we may be required to delay, limit, reduce or terminate our product development or future commercialization efforts or grant rights to develop and market product candidates that we would otherwise prefer to develop and market ourselves.

Expected Cash Requirements for Contractual Obligations

The following table presents our expected cash requirements for contractual obligations for each of the following years subsequent to December 31, 2017:

Contractual Obligations and Commitments

The following table summarizes our contractual obligations at December 31, 2017:

	Total	Less than 1 year	1-3 years	3-5 years	More than 5 years
Operating lease obligations (1)	$ 1,674,000	$ 837,000	837,000	—	—
Clinical trial CRO obligations (2)	8,142,482	7,407,907	734,575	—	—
Bioprocessing Contract (3)	12,455,691	12,455,691	—	—	—
License agreements (4)	3,098,590	2,126,400	267,268	266,968	437,953
Regulatory Contracts (5)	2,687,284	2,687,284			
Commercial Contracts (6)	3,671,641	3,671,641			
Other commitments (7)	523,000	252,000	271,000	—	—
Total	$ 32,252,688	$ 29,437,923	$ 2,109,844	$ 266,968	$ 437,953

(1) Operating lease obligations reflects our lease agreement with respect to our corporate offices at 750 Lexington Avenue, New York, New York, for a monthly rent of $69,750. The term of this lease agreement is 42 months and it expires on December 31, 2019.

(2) We have agreements in place with various contract research organizations (CROs) to facilitate research, clinical and data management services in connection with our three clinical-stage product candidates: SL-401, SL-801, and SL-701.

(3) Includes commitments to our third party drug substance and drug product manufacturers. Also includes contractual obligations for stability testing on drug substance and drug product inventory.

(4) We have executed several license agreements. Other than the payments noted in the table above, milestone and royalty payments associated with licensing have not been included as management cannot reasonably estimate if or when they will occur. These agreements include the following:

- Under a research and license agreement with Scott and White Hospital for SL-401, we are required to pay royalties on annual sales of licensed products.
- Under three separate license agreements with the University of Pittsburgh, we are required to make aggregate development and regulatory milestone payments associated with SL-701 and pay royalties on net sales of licensed products.
- Under an exclusive patent and non-exclusive know-how license agreement with the Cambridge University Technical Services Limited, related to our StemScreen® platform technology, we are required to make milestone payments upon specified regulatory events and pay royalties on sales of licensed products.
- On December 26, 2014, we entered into a license agreement with CanBas, Ltd for SL-801. SL-801 is a small molecule, reversible inhibitor of XPO1. Under the terms of the agreement, CanBas has granted us an exclusive, royalty-bearing, worldwide (excluding Japan, Korea, China and Taiwan) license, under certain patent rights, know-how and materials to research, develop, make, have made, formulate, use, sell, offer to sell and import SL-801, and any products containing or comprising such compound in finished dosage pharmaceutical form, for the treatment of any disease or condition in humans. We are required to make milestone payments upon the achievement of various clinical development, regulatory and commercial milestones. Additionally, we are required to pay tiered royalties on net sales of licensed products.
- On December 31, 2017, the Company entered into a license agreement with UCB Biopharma Sprl ("UCB") for SL-901. SL-901 is a small molecule kinase inhibitor. UCB has granted the Company an exclusive, royalty-bearing, worldwide license, under certain patent rights, know-how and materials to research, develop, make, have made, formulate, use, sell, offer to sell and import SL-901, and any products containing or comprising such compound in finished dosage pharmaceutical form. The Company may be responsible, based on the achievement of specific clinical development, regulatory and sales-based commercial milestones, for certain payments to UCB of up to $111 million, largely consisting of approval and post-approval payments. Additionally, the Company may be obligated to pay UCB tiered royalties based on aggregate net sales of products.

(5) We have agreements in place with various regulatory consultants to assist us with our BLA filing for SL-401.

(6) We have an agreement in place with a marketing related agency to assist us in preparation for a potential commercial launch for SL-401 if approved by the FDA.

(7) Other commitments include certain sponsored research.

Certain contractual payment obligations will extend beyond five years until certain specified milestones are achieved. For purposes of this calculation, we have assumed that these payment obligations have only been made in the eighth year. However, these payments would continue each subsequent year until the specified milestones are achieved.

Off-Balance Sheet Arrangements

We did not have any off-balance sheet arrangements during the periods presented, and we do not currently have any relationships with any organizations or financial partnerships, such as structured finance or special purpose entities, that would have been established for the purpose of facilitating off-balance sheet arrangements or other contractually narrow or limited purposes.

Tax Loss Carryforwards

As of December 31, 2017, we had net operating losses of $122.8 million for federal and $127.3 million state purposes, which are available to reduce future taxable income. We also had federal tax credits of approximately $31.6 million, which may be used to offset future tax liabilities. The net operating loss and tax credit carryforwards will expire at various dates through 2037. Net operating loss and tax credit carryforwards are subject to review and possible adjustment by the Internal Revenue Service and state tax authorities and may become subject to an annual utilization limitation pursuant to the change in ownership rules of Internal Revenue Code Section 382 and 383. The amount of the annual limitation is determined based on the value of our company immediately prior to the ownership change. Subsequent ownership changes may further affect the limitation in future years. At December 31, 2017, we recorded a 100% valuation allowance against our net operating loss and tax credit carryforwards, as we believe it is more likely than not that the tax benefits will not be fully realized.

Recently Adopted Accounting Standards

See Note 2 to our financial statements for recently adopted accounting standards.

Jumpstart Our Business Startups Act of 2012

The JOBS Act permits an "emerging growth company," of which we are one, to take advantage of an extended transition period to comply with new or revised accounting standards applicable to public companies. We have "opted out" of this provision and, as a result, we will comply with new or revised accounting standards as required when they are adopted. This decision to opt out of the extended transition period under the JOBS Act is irrevocable.

Item 7A. Quantitative and Qualitative Disclosures about Market Risk

We are exposed to market risk related to changes in interest rates. We had cash, cash equivalents, short-term investments and long-term investments of $66.2 million as of December 31, 2017, $67.6 million as of December 31, 2016 and $97.5 million as of December 31, 2015, consisting of cash, U. S. Treasury and Agency securities and Treasury-related money market funds and FDIC-insured bank certificates of deposit. Our primary 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 primarily in Treasury-related debt securities and bank certificates of deposit. Our available for sale 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, an immediate 100 basis point change in interest rates would not have a material effect on the fair market value of our portfolio.

We contract with CROs and CMOs. We may be subject to fluctuations in foreign currency rates in connection with these agreements. Transactions denominated in currencies other than the functional currency are recorded based on exchange rates at the time such transactions arise. As of December 31, 2017, 2016 and 2015, all of our liabilities were denominated in our functional currency.

Item 8. Financial Statements and Supplementary Data

Our financial statements and the notes thereto, included in Part IV, Item 15(a), part 1, are incorporated by reference into this Item 8.

Item 9. Changes in and Disagreements with Accountants on Accounting and Financial Disclosure

None.

Item 9A. Controls and Procedures

Evaluation of Disclosure Controls and Procedures

Our management, with the participation of our Chief Executive Officer (principal executive officer) and Chief Accounting Officer (principal financial officer), evaluated the effectiveness of our disclosure controls and procedures as of December 31, 2017. The term "disclosure controls and procedures", as defined in Rules 13a-15I and 15d-15(e) under the Exchange Act, means controls and other procedures of a company that are designed to ensure that information required to be disclosed by a company in the reports that it files or submits under the Exchange Act is recorded, processed, summarized and reported, within the time periods specified in the SEC's rules and forms. Disclosure controls and procedures include, without limitation, controls and procedures designed to ensure that information required to be disclosed by a company in the reports that it files or submits under the Exchange Act is accumulated and communicated to the company's management, including its principal executive and principal financial officers, as appropriate to allow timely decisions regarding required disclosure. Management recognizes that any controls and procedures, no matter how well designed and operated, can provide only reasonable assurance of achieving their objectives and management necessarily applies its judgment in evaluating the cost-benefit relationship of possible controls and procedures. Based on the evaluation of our disclosure controls and procedures as of December 31, 2017, our Chief Executive Officer and Chief Accounting Officer concluded that, as of such date, our disclosure controls and procedures were effective.

Management's Report on Internal Control over Financial Reporting

Our management is responsible for establishing and maintaining adequate internal control over financial reporting for our company. Internal control over financial reporting is defined in Rule 13a-15(f) or 15d-15(f) promulgated under the Exchange Act as a process designed by, or under the supervision of, the company's principal executive and principal financial officers and effected by the company's 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 GAAP and includes those policies and procedures that: (i) pertain to the maintenance of records that, in reasonable detail, accurately and fairly reflect the transactions and dispositions of the assets of the company; (ii) 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

our company are being made only in accordance with authorizations of management and directors of the company; and (iii) provide reasonable assurance regarding prevention or timely detection of unauthorized acquisition, use, or disposition of our company's assets that could have a material effect on the financial statements.

Internal control over financial reporting is designed to provide reasonable assurance regarding the reliability of financial reporting and the preparation of financial statements prepared for external purposes in accordance with generally accepted accounting principles. 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.

Our management, with the participation of our Chief Executive Officer (principal executive officer) and Chief Accounting Officer (principal financial officer), assessed the effectiveness of our internal control over financial reporting as of December 31, 2017. In making this assessment, our management used the criteria set forth in the *Internal Control—Integrated Framework (2013)* issued by the Committee of Sponsoring Organizations of the Treadway Commission. Based on its assessment, management concluded that our internal control over financial reporting was effective as of December 31, 2017 based on those criteria.

Changes in Internal Control over Financial Reporting

No change in our internal control over financial reporting occurred during the quarter ended December 31, 2017 that has materially affected, or is reasonably likely to materially affect, our internal control over financial reporting.

Item 9B. Other Information

None.

Part III

Item 10. Directors, Executive Officers and Corporate Governance

The information required by this Item is incorporated herein by reference from our Proxy Statement for our 2018 Annual Meeting of Stockholders.

Item 11. Executive Compensation

The information required by this Item is incorporated herein by reference from our Proxy Statement for our 2018 Annual Meeting of Stockholders.

Item 12. Security Ownership of Certain Beneficial Owners and Management and Related Stockholder Matters

The information required by this Item is incorporated herein by reference from our Proxy Statement for our 2018 Annual Meeting of Stockholders.

Item 13. Certain Relationships and Related Transactions and Director Independence.

The information required by this Item is incorporated herein by reference from our Proxy Statement for our 2018 Annual Meeting of Stockholders.

Item 14. Principal Accounting Fees and Services.

The information required by this Item is incorporated herein by reference from our Proxy Statement for our 2018 Annual Meeting of Stockholders.

Part IV

Item 15. Exhibits, Financial Statements Schedules.

(a) 1. Financial Statements

The following financial statements of Stemline Therapeutics, Inc. are filed as part of this report.

INDEX TO EXHIBITS

2. Financial Statement Schedules

All schedules are omitted as the information required is inapplicable or the information is presented in the financial statements or the related notes.

3. Exhibits

Exhibit No.	Description
3.1	Restated Certificate of Incorporation of Stemline Therapeutics, Inc., filed as Exhibit 3.1 to Form 8-K on February 6, 2013 (File No.001-35619) and incorporated herein by reference.
3.2	Certificate of Amendment of Restated Certificate of Incorporation of Stemline Therapeutics, Inc., filed as Exhibit 3.2 to Form 10-Q on August 8, 2017 (File No. 001-35619) and incorporated herein by reference.
3.3	Amended and Restated Bylaws of Stemline Therapeutics, Inc., filed as Exhibit 3.2 to Form 8-K on February 6, 2013 (File No. 001-35619) and incorporated herein by reference.
3.4	Certificate of Amendment of Restated Certificate of Incorporation of Stemline Therapeutics, Inc., filed as Exhibit 3.3 to Form 10-Q on August 14, 2013 (File No. 001-35619) and incorporated herein by reference.
4.1	Specimen certificate evidencing shares of common stock, filed as Exhibit 4.1 to Form S-1/A on June 20, 2012 (File No. 333-180515) and incorporated herein by reference.
4.2	Form of Representative's Warrant Agreement, filed as Exhibit 4.2 to Form S-1/A on November 14, 2012 (File No. 333-180515) and incorporated herein by reference.
10.1†	Research and License Agreement by and among the Company, Scott and White Memorial Hospital, Scott, Sherwood and Brindley Foundation and Arthur E. Frankel, M.D., dated June 15, 2006; as amended by that certain First Amendment to Research and License Agreement dated December 9, 2008, that certain Second Amendment to Research and License Agreement dated March 17, 2010 and that certain Third Amendment to Research and License Agreement dated July 12, 2011, filed as Exhibit 10.1 to Form S-1/A on June 20, 2012 (File No. 333-180515) and incorporated herein by reference.

Exhibit No.	Description
10.2†	Exclusive License Agreement between the Company and the University of Pittsburgh, dated September 30, 2009, filed as Exhibit 10.2 to Form S-1/A on June 20, 2012 (File No. 333-180515) and incorporated herein by reference.
10.3†	Exclusive Patent and Non-Exclusive Know-How License Agreement between the Company and Cambridge University Technical Services Limited, commenced September 16, 2004, filed as Exhibit 10.3 to Form S-1/A on June 20, 2012 (File No. 333-180515) and incorporated herein by reference.
10.4†	Non-Exclusive License Agreement between the Company and the University of Pittsburgh, dated March 30, 2012, filed as Exhibit 10.4 to Form S-1/A on June 20, 2012 (File No. 333-180515) and incorporated herein by reference.
10.5†	Non-Exclusive License Agreement between the Company and the University of Pittsburgh, dated March 21, 2012, filed as Exhibit 10.5 to Form S-1 on April 2, 2012 (File No. 333-180515) and incorporated herein by reference.
10.6*	Employment Agreement, dated November 6, 2011, between the Registrant and Eric K. Rowinsky, M.D., filed as Exhibit 10.6 to Form S-1 on April 2, 2012 (File No. 333-180515) and incorporated herein by reference.
10.7*	Employment Agreement, dated June 15, 2012, between the Registrant and Ivan Bergstein, M.D., filed as Exhibit 10.8 to Form S-1/A on June 20, 2012 (File No. 333-180515) and incorporated herein by reference.
10.8*	Form of Indemnification Agreement between the Registrant and each director, filed as Exhibit 10.9 to Form S-1/A on June 20, 2012 (File No. 333-180515) and incorporated herein by reference.
10.9*	Amended and Restated 2004 Employee, Director and Consultant Stock Plan, filed as Exhibit 10.10 to Form S-1on April 2, 2012 (File No. 333-180515) and incorporated herein by reference.
10.10*	Form of Incentive Stock Option Agreement under Amended and Restated 2004 Employee, Director and Consultant Stock Plan, filed as Exhibit 10.11 to Form S-1 on April 2, 2012 (File No. 333-180515) and incorporated herein by reference.
10.11*	Form of Non-qualified Stock Option Agreement under Amended and Restated 2004 Employee, Director and Consultant Stock Plan, filed as Exhibit 10.12 to Form S-1 on April 2, 2012 (File No. 333-180515) and incorporated herein by reference.
10.12*	2012 Equity Incentive Plan, filed as Exhibit 10.13 to Form S-1/A on July 19, 2012 (File No. 333-180515) and incorporated herein by reference.
10.13*	Form of Incentive Stock Option Agreement under 2012 Equity Incentive Plan, filed as Exhibit 10.14 to Form S-1/A on July 19, 2012 (File No. 333-180515) and incorporated herein by reference.
10.14*	Form of Nonstatutory Stock Option Agreement under 2012 Equity Incentive Plan, filed as Exhibit 10.15 to Form S-1/A on July 19, 2012 (File No. 333-180515) and incorporated herein by reference.
10.15	Exclusive License Agreement between the Company and Dr. Ivan Bergstein M.D., effective as of December 1, 2003, filed as Exhibit 10.18 to Form S-1/A filed on June 20, 2012 (File No. 333-180515) and incorporated herein by reference.
10.16	Assignment Agreement between the Company and Ivan Bergstein, M.D., effective as of June 15, 2012, filed as Exhibit 10.20 to Form S-1/A on June 20, 2012 (File No. 333-180515) and incorporated herein by reference.
10.17*	Offer Letter between the Company and Eric L. Dobmeier, dated April 25, 2012, filed as Exhibit 10.21 to Form S-1/A on June 20, 2012 (File No. 333-180515) and incorporated herein by reference.
10.18*	Offer Letter between the Company and J. Kevin Buchi, dated March 2, 2012, filed as Exhibit 10.22 to Form S-1/A on June 20, 2012 (File No. 333-180515) and incorporated herein by reference.
10.19*	Offer Letter between the Company and Kenneth Zuerblis, dated March 8, 2012, filed as Exhibit 10.23 to Form S-1/A on June 20, 2012 (File No. 333-180515) and incorporated herein by reference.

Exhibit No.	Description
10.20	Amendment No. 1 to Assignment Agreement between the Company and Ivan Bergstein, M.D., dated as of November 7, 2012, filed as Exhibit 10.26 to Form S-1/A on November 14, 2012 (File No. 333-180515) and incorporated herein by reference.
10.21*	Employment Agreement between the Company and David G. Gionco, dated January 16, 2014, filed as Exhibit 10.1 to Form 8-K on January 23, 2014 (File No. 001-35619) and incorporated herein by reference.
10.22†	License Agreement by and between Stemline Therapeutics, Inc. and the CanBas Co., Ltd, dated December 26, 2014, filed as Exhibit 10.30 to Form 10-K on March 16, 2015 (File No. 001-35619) and incorporated herein by reference.
10.23*	Amendment No.1 to the 2012 Equity Incentive Plan, adopted March 13, 2015, filed as Exhibit 10.31 to Form 10-K on March 16, 2015 (File No. 001-35619) and incorporated herein by reference.
10.24*	Amendment No.1 to the Amended and Restated 2004 Employee, Director and Consultant Stock Plan, adopted March 13, 2015, filed as Exhibit 10.32 to Form 10-K on March 16, 2015 (File No. 001-35619) and incorporated herein by reference.
10.25*	Separation Agreement, dated October 27, 2015, between the Company and Eric Rowinsky, M.D, filed as Exhibit 10.1 to Form 8-K on October 29, 2015 (File No. 001-35619) and incorporated herein by reference.
10.26*	Employment Agreement between the Company and Kenneth Hoberman, dated January 7, 2016, filed as Exhibit 10.1 to Form 8-K on January 13, 2016 (File No. 001-35619) and incorporated herein by reference.
10.27	2016 Equity Incentive Plan, adopted May 25, 2016, filed as Appendix A to the Company's Definitive Proxy Statement on April 8, 2016 (File No. 001-35619) and incorporated herein by reference.
10.28	Amendment No. 1 to the 2016 Equity Incentive Plan, adopted June 20, 2017, filed as Exhibit A to the Company's Definitive Proxy Statement on May 1, 2017 (File No. 001-35619) and incorporated herein by reference.
21.1	List of subsidiaries of Stemline Therapeutics, Inc.
23.1	Consent of Ernst & Young LLP.
24.1	Power of Attorney (included on signature page).
31.1	Certificate of principal executive officer pursuant to Rule 13a-14(a)/15d-14(a), as adopted pursuant to Section 302 of the Sarbanes-Oxley Act of 2002.
31.2	Certificate of principal financial officer pursuant to Rule 13a-14(a)/15d-14(a), as adopted pursuant to Section 302 of the Sarbanes-Oxley Act of 2002.
32.1	Certificate of principal executive officer pursuant to 18 U.S.C. § 1350, as adopted pursuant to Section 906 of the Sarbanes-Oxley Act of 2002.
32.2	Certificate of principal financial officer pursuant to 18 U.S.C. § 1350, as adopted pursuant to Section 906 of the Sarbanes-Oxley Act of 2002.
101	The following financial information from Stemline Therapeutics, Inc.'s Annual Report on Form 10-K for the year ended December 31, 2016, formatted in XBRL (eXtensible Business Reporting Language): (i) Consolidated Balance Sheets, (ii) Consolidated Statements of Operations, (iii) Consolidated Statements of Stockholders' Equity, (iv) Consolidated Statements of Cash Flows, (v) the Notes to Consolidated Financial Statements.

† Confidential treatment has been granted with respect to the omitted portions of this exhibit. Confidential materials omitted and filed separately with the Securities and Exchange Commission.

* Management contract or compensatory plan, contract or agreement.

Item 16. Form 10-K Summary

None.

STEMLINE THERAPEUTICS, INC.
Index to Financial Statements

Contents

Report of Independent Registered Public Accounting Firm

To the Stockholders and the Board of Directors of Stemline Therapeutics, Inc.

Opinion on the Financial Statements

We have audited the accompanying balance sheets of Stemline Therapeutics, Inc. (the Company) as of December 31, 2017 and 2016, the related statements of operations, comprehensive loss, stockholders' equity (deficit) and cash flows for each of the three years in the period ended December 31, 2017, and the related notes (collectively referred to as the "financial statements"). In our opinion, the financial statements present fairly, in all material respects, the financial position of the Company at December 31, 2017 and 2016, and the results of its operations and its cash flows for each of the three years in the period ended December 31, 2017, in conformity with U.S. generally accepted accounting principles.

Basis for Opinion

These financial statements are the responsibility of the Company's management. Our responsibility is to express an opinion on the Company's financial statements based on our 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 financial statements are free of material misstatement, whether due to error or fraud. The Company is not required to have, nor were we engaged to perform, an audit of its internal control over financial reporting. As part of our audits we are required to obtain an understanding of internal control over financial reporting but not for the purpose of expressing an opinion on the effectiveness of the Company's internal control over financial reporting. Accordingly, we express no such opinion.

Our audits included performing procedures to assess the risks of material misstatement of the financial statements, whether due to error or fraud, and performing procedures that respond to those risks. Such procedures included examining, on a test basis, evidence regarding the amounts and disclosures in the financial statements. Our audits also included evaluating the accounting principles used and significant estimates made by management, as well as evaluating the overall presentation of the financial statements. We believe that our audits provide a reasonable basis for our opinion.

/s/ Ernst & Young LLP

We have served as the Company's auditor since 2011.
Stamford, Connecticut
March 16, 2018

STEMLINE THERAPEUTICS, INC.
Balance Sheets

	December 31, 2017	December 31, 2016
Assets		
Current assets:		
Cash and cash equivalents	$ 4,795,098	$ 10,316,064
Short-term investments	46,924,612	36,562,900
Prepaid expenses and other current assets	469,067	290,747
Total current assets	52,188,777	47,169,711
Property and equipment, net	136,672	22,531
Long-term investments	14,468,414	20,714,551
Other Assets	212,305	212,305
Total assets	$ 67,006,168	$ 68,119,098
Liabilities and stockholders' equity		
Current liabilities:		
Accounts payable and accrued expenses	$ 19,742,087	$ 9,284,514
Current portion of deferred grant revenue	—	898,199
Other current liabilities	96,826	71,100
Total current liabilities	19,838,913	10,253,813
Other Liabilities	96,826	142,200
Total liabilities	19,935,739	10,396,013
Commitments and contingencies (Note 13)		
Stockholders' equity:		
Preferred stock $0.0001 par value, 5,000,000 shares authorized, none issued and outstanding at December 31, 2017 and 2016	—	—
Common stock $0.0001 par value, 53,750,000 shares authorized at December 31, 2017 and 33,750,000 shares authorized at December 31, 2016. 25,313,595 shares issued and outstanding at December 31, 2017 and 19,219,223 shares issued and outstanding at December 31, 2016	2,531	1,922
Additional paid-in capital	251,489,546	193,563,572
Accumulated other comprehensive loss	(145,958)	(99,802)
Accumulated deficit	(204,275,690)	(135,742,607)
Total stockholders' equity	47,070,429	57,723,085
Total liabilities and stockholders' equity	$ 67,006,168	$ 68,119,098

See accompanying notes.

STEMLINE THERAPEUTICS, INC.
Statements of Operations

	Year Ended December 31,		
	2017	2016	2015
Revenues:			
Grant revenue	$ 898,199	$ 1,041,354	$ 654,160
Operating expenses:			
Research and development	50,242,386	27,869,921	29,458,676
General and administrative	19,214,207	12,056,890	8,828,843
Total operating expenses	69,456,593	39,926,811	38,287,519
Loss from operations	(68,558,394)	(38,885,457)	(37,633,359)
Other (expense) income	(6,330)	11,438	1,609
Interest income	736,330	545,718	387,889
Net loss before income taxes	(67,828,394)	(38,328,301)	(37,243,861)
Income tax benefit	—	25,296	—
Net loss	$ (67,828,394)	$ (38,303,005)	$ (37,243,861)
Net loss per common share:			
Basic and Diluted	$ (2.94)	$ (2.15)	$ (2.15)
Weighted-average shares outstanding:			
Basic and Diluted	23,056,928	17,804,681	17,289,021

See accompanying notes.

STEMLINE THERAPEUTICS, INC.
Statements of Comprehensive Loss

	Year Ended December 31		
	2017	2016	2015
Net loss	$ (67,828,394)	$ (38,303,005)	$ (37,243,861)
Other comprehensive loss:			
Unrealized gain (loss) on investments, net of tax	(52,308)	65,326	(155,081)
Reclassification adjustment for gain on investments included in net loss	6,152	(11,438)	(1,609)
Other comprehensive (loss) gain	(46,156)	53,888	(156,690)
Comprehensive loss	$ (67,874,550)	$ (38,249,117)	$ (37,400,551)

STEMLINE THERAPEUTICS, INC.
Statements of Stockholders' Equity (Deficit)

	Common Stock		Additional Paid-in Capital	Accumulated Other Comprehensive Income (Loss)	Earnings (Deficit)	Total Stockholders' Equity (Deficit)
	Shares	Capital				
Balance, December 31, 2014	13,277,269	$ 1,329	$ 115,604,563	$ 3,000	$ (60,195,741)	$ 55,413,151
Restricted stock grants	399,742	40	(40)	—	—	—
Stock award — outside services	12,100	1	147,377	—	—	147,378
Forfeiture of restricted stock grants	(13,976)	(1)	1	—	—	—
Stock-based compensation	—	—	5,015,584	—	—	5,015,584
ESPP compensation expense	—	—	55,515	—	—	55,515
Issuance of common stock in connection with the ESPP	7,070	1	49,489	—	—	49,490
Issuance of common stock in connection with the exercise of stock options	198,938	20	724,997	—	—	725,017
Issuance of common stock in connection with the follow-on public offering	4,353,877	435	64,105,937	—	—	64,106,372
Net loss	—	—	—	—	(37,243,861)	(37,243,861)
Other comprehensive income (loss)	—	—	—	(156,690)	—	(156,690)
Balance, December 31, 2015	18,235,020	$ 1,825	$ 185,703,423	$ (153,690)	$ (97,439,602)	$ 88,111,956
Restricted stock grants	920,444	91	(91)	—	—	—
Forfeiture of restricted stock grants	(39,997)	(4)	4	—	—	—
Stock-based compensation	—	—	7,431,126	—	—	7,431,126
ESPP compensation expense	—	—	73,348	—	—	73,348
Issuance of common stock in connection with the ESPP	15,970	1	91,575	—	—	91,576
Issuance of common stock in connection with the exercise of stock options	87,786	9	264,187	—	—	264,196
Net loss	—	—	—	—	(38,303,005)	(38,303,005)
Other comprehensive income, net of tax	—	—	—	53,888	—	53,888
Balance, December 31, 2016	19,219,223	$ 1,922	$ 193,563,572	$ (99,802)	$ (135,742,607)	$ 57,723,085
Restricted stock grants	914,950	91	(91)	—	—	—
Forfeiture of restricted stock grants	(42,048)	(4)	4	—	—	—
Stock-based compensation	—	—	8,618,520	—	—	8,618,520
ESPP compensation expense	—	—	68,496	—	—	68,496
Adoption of accounting standard update related to stock compensation accounting (ASU 2016-09)	—	—	704,689	—	(704,689)	—
Issuance of common stock in connection with ESPP	22,518	2	150,922	—	—	150,924
Issuance of common stock in connection with the exercise of stock options	23,952	2	141,738	—	—	141,740
Issuance of common stock in connection with follow-on public offering	5,175,000	518	48,241,696	—	—	48,242,214
Net loss	—	—	—	—	(67,828,394)	(67, 828,394)
Other comprehensive income, net of tax	—	—	—	(46,156)	—	(46,156)
Balance, December 31, 2017	25,313,595	$ 2,531	$ 251,489,546	$ (145,958)	$ (204,275,690)	$ 47,070,429

See accompanying notes.

STEMLINE THERAPEUTICS, INC.
Statements of Cash Flows

	Year Ended December 31,		
	2017	2016	2015
Cash flows from operating activities			
Net loss	$ (67,828,394)	$ (38,303,005)	$ (37,243,861)
Adjustments to reconcile net loss to net cash used in operating activities:			
Depreciation	14,641	80,259	153,797
Stock-based compensation expense	8,618,520	7,431,126	5,162,962
Employee Stock Purchase Plan compensation expense	68,496	73,348	55,515
Amortization of premium paid on marketable securities	140,385	221,049	277,009
Net loss (gain) on sale of marketable securities	6,152	(11,438)	(1,609)
Income tax benefit from other comprehensive income	—	(25,296)	—
Changes in operating assets and liabilities:			
Prepaid expenses and other current assets	(178,320)	361,142	984,919
Other Assets	—	(212,305)	—
Accounts payable and accrued expenses	10,457,573	651,641	4,981,635
Other current liabilities	25,726	71,100	—
Deferred grant revenue	(898,199)	(541,354)	8,950
Other liabilities	(45,374)	110,959	31,241
Net cash used in operating activities	(49,618,794)	(30,092,774)	(25,589,442)
Cash flows from investing activities			
Purchase of furniture and fixtures	(128,782)	(7,129)	(19,458)
Purchase of marketable securities	(61,893,074)	(26,464,749)	(105,565,837)
Sale and maturities of marketable securities	57,584,806	53,148,748	54,662,837
Net cash (used in) provided by investing activities	(4,437,050)	26,676,870	(50,922,458)
Cash flows from financing activities			
Proceeds from issuance of common stock from follow-on public offering, net	48,242,214	—	64,106,372
Proceeds from issuance of common stock from ESPP	150,924	91,576	49,490
Proceeds from exercise of stock options	141,740	264,196	725,017
Net cash provided by financing activities	48,534,878	355,772	64,880,879
Net decrease in cash and cash equivalents	(5,520,966)	(3,060,132)	(11,631,021)
Cash and cash equivalents at beginning of period	10,316,064	13,376,196	25,007,217
Cash and cash equivalents at end of period	$ 4,795,098	$ 10,316,064	$ 13,376,196

See accompanying notes.

STEMLINE THERAPEUTICS, INC.
Notes To Financial Statements
December 31, 2017

1. Organization and Basis of Presentation

Organization

Stemline Therapeutics, Inc. (the "Company") is a clinical-stage biopharmaceutical company focused on discovering, acquiring, developing and potentially commercializing proprietary oncology therapeutics. The Company's activities to date have primarily consisted of advancing its clinical stage programs, expanding and strengthening its intellectual property portfolio, identifying and acquiring additional product and technology rights and raising capital. The Company was incorporated in Delaware on August 8, 2003 and has its principal office in New York, New York.

The Company has incurred losses from operations since inception of $215.7 million. Since its inception, most of its resources have been dedicated to the discovery, acquisition and preclinical and clinical development of its product candidates. In particular, it has expended and will continue to expend substantial resources for the foreseeable future developing its clinical candidates, SL-401, SL-801, and SL-701 as well as its preclinical product candidates and drug discovery and acquisition efforts. These expenditures include costs associated with general and administrative costs, facilities costs, research and development, acquiring new technologies, manufacturing product candidates, conducting preclinical experiments and clinical trials and obtaining regulatory approvals, as well as commercializing any products approved for sale. The Company anticipates incurring additional losses until such time, if ever, that it can generate significant sales of its products currently in development. The Company expects its research and development expenses to increase significantly in connection with its ongoing and planned clinical trials and related manufacturing efforts, as well as pre-launch and launch activities should SL-401 be approved. As a result, the Company expects to continue to incur significant and increasing operating losses for the foreseeable future. If adequate funds are not available to the Company on a timely basis, or at all, the Company may be required to terminate or delay clinical trials or other development activities for SL-401, SL-801, or SL-701, or for one or more indications for which it is developing SL-401, SL-801, or SL-701, or delay its establishment of sales and marketing capabilities or other activities that may be necessary to commercialize SL-401, SL-801, or SL-701, if the Company obtains marketing approval.

Use of Estimates

The preparation of financial statements in conformity with generally accepted accounting principles in the U.S. (U.S. GAAP) requires management to make estimates and assumptions that affect the reported financial position at the date of the financial statements and the reported results of operations during the reporting period. Such estimates and assumptions affect the reported amounts of assets, liabilities, revenues and expenses and disclosure of contingent assets and liabilities in the financial statements and accompanying notes. Actual results could differ from those estimates.

Reclassifications

Certain reclassifications totaling $73,348 and $55,515 have been made to the Statement of Cash Flows for the period ended December 31, 2016 and December 31, 2015, respectively, to conform to the employee stock purchase plan compensation expense presentation in the Statement of Cash Flows for the period ended December 31, 2017. In addition, reclassifications totaling $91,576 and $49,490 have been made to the Statement of Cash Flows for the period ended December 31, 2016 and December 31, 2015, respectively, to conform to the proceeds from issuance of common stock from ESPP presentation in the Statement of Cash Flows for the period ended December 31, 2017. These reclassifications to adjust stock-based compensation expense and proceeds from issuance of common stock from ESPP had no impact on previously reported cash used in operations and cash provided by investing activities in the Statement of Cash Flows.

2. Summary of Significant Accounting Policies

Cash Equivalents

Cash equivalents consist of highly liquid instruments purchased with an original maturity of three months or less. At December 31, 2017 and 2016, cash equivalents consisted of deposits in financial institutions and money market mutual funds invested in U.S. treasury securities. The Company maintains its cash deposits and cash equivalents with well-known and stable financial institutions.

Concentration of Credit Risk

Financial instruments that potentially subject the Company to significant concentrations of credit risk consist primarily of cash equivalents. The Company invests its excess cash in major U.S. banks and financial institutions, and its deposits, at times, exceed federally insured limits. The Company has not experienced any losses from credit risks.

Investments

The Company's investments are considered to be available-for-sale and are carried at fair value. Unrealized gains and losses, if any, are reported as a separate component of stockholders' equity (deficit) and are not reflected in the statements of operations until a sale transaction occurs or when declines in fair value are deemed to be other-than-temporary ("OTT"). The cost of investments classified as available-for-sale are adjusted for the amortization of premiums and accretion of discounts to maturity and recorded in other expense and other income, respectively. Realized gains and losses, if any, are determined using the specific identification method and are included in other income and other expense, respectively. Investments with original maturities beyond 90 days at the date of purchase and which mature at, or less than twelve months from, the balance sheet date are classified as current. Investments with a maturity beyond twelve months from the balance sheet date are classified as long-term.

Fair Value of Financial Instruments

The Company's financial instruments consist principally of cash and cash equivalents, investments, other current assets, accounts payable and accrued liabilities. Cash and cash equivalents, and short and long-term investments are carried at fair value (see Note 7). Financial instruments including other current assets, accounts payable and accrued liabilities are carried at cost, which approximate fair value given their short-term nature.

Other-Than-Temporary Impairment Losses on Investments

The Company regularly monitors its available-for-sale portfolio to evaluate the necessity of recording impairment losses for OTT declines in the fair value of investments. Management makes this determination through the consideration of various factors such as management's intent and ability to retain an investment for a period of time sufficient to allow for any anticipated recovery in market value. OTT impairment losses result in a permanent reduction of the cost basis of an investment. For the years ended December 31, 2017 and 2016, the Company did not realize any investment losses due to OTT declines in fair value.

Furniture and Fixtures

Furniture and fixtures are stated at cost, less accumulated depreciation. Depreciation is provided over the estimated useful lives of the respective assets, which are between three to seven years, using the straight-line method.

Impairment of Long-Lived Assets

The Company reviews long-lived assets, (the Company's furniture and fixtures), for impairment whenever events or changes in business circumstances indicate that the carrying amount of the assets may not be fully recoverable. An impairment loss would be recognized when estimated undiscounted future cash flows expected to result from the use of the asset and its eventual disposition are less than its carrying amount. The impairment loss, if recognized, would be based on the excess of the carrying value of the impaired asset over its respective fair value. The Company purchased fixed assets during 2017, 2016 and 2015. For the years ended December 31, 2017, 2016 and 2015, the Company did not realize any impairment losses.

Grant Revenue Recognition

The Company has not yet generated any revenue from product sales and it has generated minimal revenues to date, all relating to $3.0 million in research grants received from the Leukemia and Lymphoma Society, or LLS. This research grant was awarded to the Company to support funding some of the costs for the ongoing SL-401 clinical trials. Grant payments received prior to the Company's performance of work required by the terms of the research grant are recorded as deferred revenue and recognized as grant revenue once work is performed and qualifying costs are incurred. The Company has recognized approximately $0.9 million, $1.0 million and $0.7 million of revenue related to the LLS grant for the years ended December 31, 2017, 2016 and 2015, respectively, which reflect twelve months of revenue recognized, respectively, on a straight-line basis, based on the Company's best estimates of the timing of work to be performed and qualifying costs incurred.

Accrued Clinical Development Costs

Outside research costs are a component of research and development expense. These expenses include fees paid to contract research organizations ("CROs") and other service providers that conduct certain clinical and product development activities on behalf of the Company. Depending upon the timing of payments to the service providers, the Company recognizes prepaid expenses or accrued expenses related to these costs. These accrued or prepaid expenses are based on management's estimates of the work performed under service agreements, milestones achieved and experience with similar contracts. The Company monitors each of these factors and adjusts estimates accordingly.

Research and Development Costs

Research and development costs are comprised primarily of costs for personnel, including salaries and benefits; clinical studies administered by third-party investigators and managed by Stemline personnel; materials and supplies to support the Company's clinical programs; contracted research; manufacturing; related consulting arrangements; costs related to upfront and milestone payments under license agreements; and other expenses incurred to sustain the Company's overall research and development programs. Internal research and development costs are expensed as incurred. Third-party research and development costs are expensed as the contracted work is performed. In certain circumstances, the Company is required to make advance payments to vendors for goods or services that will be received in the future for use in research and development activities. In such circumstances, the advance payments are deferred and are expensed when activities have been performed or when the goods have been received.

Income Taxes

Income taxes are accounted for under the asset and liability method. Deferred tax assets and liabilities are recognized for the future tax consequences attributable to the differences between the financial statement carrying amounts of existing assets and liabilities and the respective tax bases and operating loss and tax credit carryforwards. Deferred tax assets and liabilities are measured using enacted tax rates expected to apply to taxable income in the years in which those temporary differences are expected to be recovered or settled. The effect on deferred tax assets and liabilities of a change in tax rates is recognized in income in the period that includes the enactment date.

The asset and liability method requires that deferred tax assets and liabilities be recorded without consideration as to the ability to realize them. The deferred tax asset primarily includes net operating loss and tax credit carryforwards, accrued expenses not currently deductible and the cumulative temporary differences related to certain research and patent costs, which have been charged to expense in the accompanying statements of operations but have been recorded as assets for income tax purposes. The portion of any deferred tax asset for which it is more likely than not that a tax benefit will not be realized must then be offset by recording a valuation allowance. A valuation allowance has been established against all of the deferred tax assets (see Note 12), as it is more likely than not that these assets will not be realized given the history of operating losses.

The Company recognizes the tax benefit from an uncertain tax position only if it is more likely than not to be sustained upon examination based on the technical merits of the position. The amount of the accrual for which an exposure exists is measured as the largest amount of benefit determined on a cumulative probability basis that the Company believes is more likely than not to be realized upon ultimate settlement of the position.

In December 2017, the SEC staff issued Staff Accounting Bulletin No. 118 ("SAB 118") to address the application of U.S. GAAP in situation where a company does not have the necessary information available , prepared, or analyzed in reasonable detail to complete the accounting for certain income tax effects of the Tax Cuts and Jobs Act of 2017. It allows companies to record provisional amounts during a measurement period, which is not to extend beyond one year. Consistent with SAB 118, the Company was able to make reasonable estimates and recorded provisional amounts related to the remeasurement of the deferred tax asset in the fourth quarter 2017 consolidated financial statements. The recorded amount may require further adjustments due to evolving analysis and interpretations of law, including issuance by the IRS and other regulatory bodies, state tax conformity to federal changes, as well as interpretations of how accounting for income taxes should be applied.

Stock-Based Compensation

The Company accounts for stock-based compensation in accordance with the provisions of ASC 718, *Compensation—Stock Compensation*. Accordingly, compensation costs related to equity instruments granted are recognized over the requisite service periods of the awards on a straight-line basis at the grant-date fair value calculated using a Black-Scholes option pricing model for stock options and the closing stock price on date of grant for restricted stock. Additionally, under the provisions of ASC 718, the Company accounts for forfeited awards as incurred and on the date the forfeiture or cancellation occurs.

For stock options granted as consideration for services rendered by non-employees, the Company recognizes expense in accordance with the requirements of ASC Topic 505-50, *Equity Based Payments to Non-Employees*. Non-employee option grants that do not vest immediately upon grant are recorded as an expense over the vesting period of the underlying stock options. At the end of each financial reporting period prior to vesting, the value of these options, as calculated using the Black-Scholes option-pricing model, is re-measured using the fair value of the Company's common stock and the non-cash stock-based compensation expense recognized during the period is adjusted accordingly. Since the fair market value of options granted to non-employees is subject to change in the future, the amount of the future expense will include fair value re-measurements until the stock options are fully vested.

The Company's policy upon exercise of stock options is that shares will be issued as new shares drawing on the Company's 2016 Stock Equity Incentive Plan (the "2016 Plan") share pool that was adopted by the board of directors and approved by the stockholders in May 2016.

In the event a modification is made to an equity award after the grant date, the Company records a change in stock-based compensation expense equal to the incremental fair value of the equity award immediately subsequent to the modification as compared to the fair value of the equity award immediately preceding the modification. During 2015 and 2016, the Company modified certain outstanding equity award held by employees and certain Directors. These modifications resulted in incremental compensation cost of $0, $0.8 million, and $0.3 million for the year ended December 31, 2017, 2016, and 2015, respectively.

Segment Information

The Company reports segment information in accordance with applicable guidance on segment disclosures. The Company has one reportable segment.

Recent Accounting Pronouncements

In May 2014, the Financial Accounting Standards Board ("FASB"), issued a comprehensive new revenue recognition Accounting Standards Update, *Revenue From Contracts With Customers (Topic 606) (ASU2014-09).* ASU 2014-09 provides guidance to clarify the principles for recognizing revenue. This guidance includes the required steps to achieve the core principle that an entity should recognize revenue to depict the transfer of promised goods or services to customers in an amount that reflects the consideration to which the entity expects to be entitled in exchange for those goods or services. This guidance is effective for fiscal years and interim periods beginning after December 15, 2017. Early adoption is permitted for fiscal years and interim periods beginning after December 15, 2016. The Company expects to adopt this guidance on January 1, 2018 using the full retrospective method. The accounting standard will have no impact on the financial statements since the Company has no contracts with customers. Any future contracts with customers will be accounted for under the new guidance effective January 1, 2018.

In November 2015, the FASB issued a new Accounting Standards Update, *Balance Sheet Classification of Deferred Taxes (ASU 2015-17).* ASU 2015-17 requires all deferred tax assets and liabilities, and any related valuation allowance, to be classified as non-current on the balance sheet. The classification change for all deferred taxes as non-current simplifies entities' processes as it eliminates the need to separately identify the net current and net non-current deferred tax asset or liability in each jurisdiction and allocate valuation allowances. The Company adopted this accounting standard prospectively in the first quarter of fiscal 2017 and did not retrospectively adjust prior period. The adoption of this standard did not have any impact on the Company's financial statements due to full valuation allowance recorded on the Company's deferred taxes.

In January 2016, the FASB issued a new Accounting Standards Update, *Recognition and Measurement of Financial Assets and Financial Liabilities (ASU 2016-01).* ASU 2016-01 amends the guidance in U.S. GAAP on the classification and measurement of financial instruments. Although the ASU retains many current requirements, it significantly revises an entity's accounting related to (1) the classification and measurement of investments in equity securities and (2) the presentation of certain fair value changes for financial liabilities measured at fair value. The ASU also amends certain disclosure requirements associated with the fair value of financial instruments. The new standard is effective for fiscal years and interim periods within those fiscal years beginning after December 15, 2017, with early adoption permitted for certain changes. The Company is currently evaluating the potential effects the new standard will have of the Company's financial statements and related disclosures.

In February 2016, the FASB issued a new Accounting Standards Update*, Leases (ASU 2016-02),* ASU 2016-02 is aimed at making leasing activities more transparent and comparable and requires most leases be recognized by lessees on the balance sheets as a right-of-use asset and corresponding lease liability, regardless of whether they are classified as finance or operating leases. The new standard is effective for annual reporting periods beginning after December 15, 2018, including interim periods within that reporting period, with early adoption permitted. The Company is currently evaluating the impact of the new pronouncement on the Company's financial statements and related disclosures.

In August 2016, the FASB issued ASU 2016-15, *Statement of Cash Flows (Topic 230), Classification of Certain Cash Receipts and Cash Payments*. ASU 2016-15 clarifies how entities should classify certain cash receipts and cash payments on the statement of cash flows and amends certain disclosure requirements of ASC 230. The guidance will generally be applied retrospectively and is effective for public business entities for fiscal years beginning after December 15, 2017, and interim periods within those years. For all other entities, it is effective for fiscal years beginning after December 15, 2018, and interim periods within fiscal years beginning after December 15, 2019. Early adoption is permitted, including adoption in an interim period. If an entity early adopts the guidance in an interim period, any adjustments should be reflected as of the beginning of the fiscal year that includes that interim period. An entity that elects early adoption must adopt all of the guidance in the same period. The Company is currently evaluating the impact of the new pronouncement on the Company's financial statements and related disclosures.

As of January 1, 2017, we adopted ASU No. 2016-09, *Improvements to Employee Share-Based Payment Accounting*, or ASU 2016-09, which amends Accounting Standards Codification, or ASC, *Topic 718, Compensation — Stock Compensation*, and is effective for fiscal years beginning after December 15, 2016, and interim periods within those fiscal years. ASU 2016-09 simplifies several aspects of the accounting for share-based payment transactions, including the accounting for forfeitures, income tax consequences, classification of awards as either equity or liabilities, and classification on the statement of cash flows. Certain amendments will be applied using a modified retrospective transition method by means of a cumulative-effect adjustment to equity as of January 1, 2017, while other amendments will be applied retrospectively, prospectively or using either a prospective or a retrospective transition method. Upon adoption on January 1, 2017, we are beginning to account for forfeitures as they occur rather than estimate a forfeiture rate and have recorded a cumulative-effect adjustment to equity of $0.7 million on the date of initial adoption. In periods subsequent to adoption, a higher expense will be recognized earlier during the respective vesting periods of stock-based awards that are not forfeited. As a result of the valuation allowance against our deferred tax assets, there was no net adjustment to retained earnings for the change in accounting for unrecognized windfall tax benefits.

On December 22, 2017, the Tax Cuts and Jobs Act (the "TCJ Act") was enacted into law. The TCJ Act provides for significant changes to the U.S. Internal Revenue Code of 1986, as amended (the "Code"), that impact corporate taxation requirements, such as the reduction of the federal tax rate for corporations from 35% to 21% and changes or limitations to certain tax deductions. The reduction in the corporate tax rate under the TCJ Act will also require a one-time revaluation of certain tax-related assets to reflect their value at the lower corporate tax rate of 21%. As such, the Company currently calculates a reduction in the value of these assets of approximately $25M, which is fully offset by a valuation allowance and has no impact on the income tax provision. The company is still evaluating the full impact of the TCJ Act but due to a full valuation against deferred taxes, the company estimates no impact to the income tax provision.

3. Net Loss per Common Share

The Company accounts for and discloses net loss per share using the treasury stock method. Net loss per common share, or basic loss per share, is computed by dividing net loss by the weighted-average number of common shares outstanding. Since the Company is in a net loss for all periods presented, diluted net loss per share is not presented since the common stock equivalents would have an anti-dilutive effect on the per share calculation.

The following table sets forth the computation of basic and diluted net loss per share for the periods indicated:

	Year Ended December 31,		
	2017	2016	2015
Basic and Diluted loss per common share calculation:			
Net loss attributable to common shareholders — basic and diluted	$ (67,828,394)	$ (38,303,005)	$ (37,243,861)
Basic and diluted weighted-average common shares	23,056,928	17,804,681	17,289,021
Basic and diluted net loss per share	$ (2.94)	$ (2.15)	$ (2.15)

The difference between basic and diluted weighted-average common shares generally results from the assumption that dilutive stock options outstanding were exercised, dilutive restricted stock has vested and outstanding warrants are issued. For the years ended December 31, 2017, 2016 and 2015, the Company reported a loss from operations and therefore, all potentially dilutive stock options, restricted stock, and outstanding warrants as of such date were excluded from the computation of diluted net loss per share as their effect would have been

anti-dilutive. The total shares of stock options, restricted stock, and outstanding warrants that could potentially dilute earnings per share in the future, but which were not included in the calculation of diluted net loss per share because their effect would have been anti-dilutive were as follows:

	Year Ended December 31		
	2017	2016	2015
Unvested restricted stock	1,724,837	1,268,092	553,045
Options outstanding	3,174,964	3,090,752	2,121,726
Warrants	99,529	99,529	99,529
Total	4,999,330	4,458,373	2,774,300

4. Marketable Investments

The following table summarizes the Company's cash equivalents and available for sale securities:

	December 31, 2017			
	Amortized Cost	Gross Unrealized Gains	Gross Unrealized Losses	Estimated Fair Value
Cash:				
Cash from operating accounts	$ 3,088,659	$ —	$ —	$ 3,088,659
Cash equivalents:				
Money market funds	1,706,439	—	—	1,706,439
Short-term investments:				
Fixed-income treasury portfolio:				
Fannie Mae	13,631,041	—	(31,396)	13,599,645
Federal farm credit bank	1,249,823	—	(1,869)	1,247,954
Federal home loan bank	5,923,497	—	(14,197)	5,909,300
Freddie Mac	5,409,227	—	(19,182)	5,390,045
U.S. Treasury Securities	6,282,231	—	(12,578)	6,269,653
Other	1,019,302	—	(612)	1,018,690
Certificate of Deposits	13,489,241	84	—	13,489,325
Total Short-term investments	47,004,362	84	(79,834)	46,924,612
Long-term investments:				
Fixed-income treasury portfolio:				
Fannie Mae	—	—	—	—
Federal farm credit bank	—	—	—	—
Federal home loan bank	2,753,337	—	(15,529)	2,737,808
Freddie Mac	2,509,471	—	(13,635)	2,495,836
U.S. Treasury Securities	1,503,030	—	(11,748)	1,491,282
Certificate of Deposits	7,743,488	—	—	7,743,488
Total Long-term investments	14,509,326	—	(40,912)	14,468,414
Total	$ 66,308,786	$ 84	$ (120,746)	$ 66,188,124

	December 31, 2016			
	Amortized Cost	Gross Unrealized Gains	Gross Unrealized Losses	Estimated Fair Value
Cash:				
Cash from operating accounts	$ 851,667	$ —	$ —	$ 851,667
Cash equivalents:				
Money market funds	9,464,397	—	—	9,464,397
Short-term investments:				
Fixed-income treasury portfolio:				
Fannie Mae	2,108,607	—	(1,366)	2,107,241
Federal farm credit bank	4,357,111	830	(2,666)	4,355,275
Federal home loan bank	7,816,214	1,284	(3,400)	7,814,098
Freddie Mac	7,467,276	223	(1,003)	7,466,496
Certificate of Deposits	14,818,468	1,322	—	14,819,790
Total Short-term investments	36,567,676	3,659	(8,435)	36,562,900
Long-term investments:				
Fixed-income treasury portfolio:				
Fannie Mae	5,878,500	—	(29,885)	5,848,615
Federal farm credit bank	—	—	—	—
Federal home loan bank	1,900,546	—	(10,773)	1,889,773
Freddie Mac	7,933,745	—	(29,374)	7,904,371
Certificate of Deposits	5,071,490	302	—	5,071,792
Total Long-term investments	20,784,281	302	(70,032)	20,714,551
Total	$ 67,668,021	$ 3,961	$ (78,467)	$ 67,593,515

At December 31, 2017 and December 31, 2016, the remaining contractual maturities of available-for-sale investments classified as current on the balance sheet was less than 12 months, and remaining contractual maturities of available-for-sale investments classified as long-term were less than two years.

There were no available-for-sale securities in a continuous unrealized loss position for greater than twelve months at December 31, 2017 and December 31, 2016. The Company has the ability to hold such securities with an unrealized loss until its forecasted recovery. The Company determined that there was no material change in the credit risk of the above investments. As a result, the Company determined it did not hold any investments with an other-than-temporary impairment as of December 31, 2017.

5. Prepaid Expenses and Other Current Assets

Prepaid expenses and other current assets consist of the following at December 31, 2017 and 2016:

	December 31, 2017	December 31, 2016
Prepaid third-party vendor costs	$ 131,286	$ 208,196
Deferred registration fees	143,924	—
Prepaid insurance	44,065	48,048
Other receivable	149,792	34,503
Total	$ 469,067	$ 290,747

6. Property and Equipment, Net

Furniture and fixtures consist of the following at December 31, 2017 and 2016:

	December 31, 2017	December 31, 2016
Office furniture and fixtures	$ 519,675	$ 486,586
Leasehold improvements	82,694	—
Manufacturing equipment	13,000	—
Property and equipment	615,369	486,586
Less accumulated depreciation	(478,697)	(464,055)
Property and equipment, net	$ 136,672	$ 22,531

Depreciation expense was $14,641, $80,259 and $153,797 for the years ended December 31, 2017, 2016 and 2015, respectively.

7. Fair Value Measurements

Fair value is defined as the exchange price that would be received for an asset or paid to transfer a liability (an exit price) in the principal or most advantageous market for the asset or liability in an orderly transaction between market participants at the measurement date. Assets and liabilities that are measured at fair value are reported using a three-level fair value hierarchy that prioritizes the inputs used to measure fair value. This hierarchy maximizes the use of observable inputs and minimizes the use of unobservable inputs. The three levels of inputs used to measure fair value are as follows:

Level 1 — Quoted prices in active markets for identical assets or liabilities that the Company has the ability to access at the measurement date.

Level 2 — Inputs other than quoted prices in active markets that are observable for the asset or liability, either directly or indirectly.

Level 3 — Inputs that are unobservable for the asset or liability.

The following fair value hierarchy table presents information about each major category of the Company's financial assets and liability measured at fair value on a recurring basis as of December 31, 2017 and 2016:

	December 31, 2017			
	Quoted Prices in Active Markets for Identical Assets (Level 1)	Significant Other Observable Inputs (Level 2)	Significant Unobservable Inputs (Level 3)	Balance at December 31, 2017
Fixed-income treasury portfolio	$ 40,160,213	$ —	$ —	$ 40,160,213
Certificate of Deposits	—	21,232,813	—	21,232,813
Cash and cash equivalents	4,795,098	—	—	4,795,098
Total assets at fair value	$ 44,955,311	$ 21,232,813	$ —	$ 66,188,124

	December 31, 2016			
	Quoted Prices in Active Markets for Identical Assets (Level 1)	Significant Other Observable Inputs (Level 2)	Significant Unobservable Inputs (Level 3)	Balance at December 31, 2016
Fixed-income treasury portfolio	$ 37,385,869	$ —	$ —	$ 37,385,869
Certificate of Deposits	—	19,891,582	—	19,891,582
Cash and cash equivalents	10,316,064	—	—	10,316,064
Total assets at fair value	$ 47,701,933	$ 19,891,582	$ —	$ 67,593,515

There were no transfers between levels in the fair value hierarchy during any of the periods presented herein.

8. Accounts Payable and Accrued Expenses

Accounts payable and accrued expenses consist of the following:

	December 31,	
	2017	2016
Accrued research and development costs	$ 14,841,071	$ 6,792,947
Accrued compensation	2,940,039	1,975,037
Accrued legal	780,664	71,554
Other accrued liabilities	1,180,313	444,976
Total accounts payable and accrued expenses	$ 19,742,087	$ 9,284,514

9. Capital Structure

Initial Public Offering

On January 31, 2013, the Company completed its initial public offering (the "IPO"), selling 3,317,644 shares at an offering price of $10.00 per share. On January 29, 2013, the underwriters exercised in full their over-allotment option to purchase an additional 497,647 shares at an offering price of $10.00 per share. Aggregate gross proceeds from the IPO, including the exercise of the over-allotment option, were $38.2 million and net proceeds received after underwriting fees and offering expenses were approximately $32.3 million. Additionally, upon the closing of the IPO, certain transactions were triggered based on a successful completion of an IPO. Convertible debt of $1.4 million principal, plus accrued interest thereon, was converted into 166,769 shares of common stock. The Company recorded approximately $1.5 million of compensation expense related to certain bonuses and salary increases payable upon continued employment and the occurrence of a specified financing, including the consummation of an initial public offering. Finally, the Company recorded one-time compensation expense of approximately $1.4 million for certain options and restricted stock that fully vested upon the closing of the IPO.

Representative's Warrants

On October 1, 2012, the Company agreed to issue to the representative of the underwriters in the IPO warrants to purchase up to 99,529 shares of the Company's common stock in the event of a successful public offering. The warrants are exercisable for cash or on a cashless basis at a price per share equal to $15.00. The term of the warrants is five years and they expire on January 28, 2018. Based on a successful public offering in January of 2013, these warrants were issued and accounted for as a cost of issuance. The Company has determined, based upon a Black-Scholes model, that the fair value of the warrants on the date of IPO was $413,146. The Company has accounted for the fair value of the warrants as a cost of issuance of common stock from the IPO resulting in a charge directly to stockholder's equity.

Common Stock

As of December 31, 2017, the Company was authorized to issue 53,750,000 shares of common stock. Dividends on common stock will be paid when, and if, declared by the board of directors. Each holder of common stock is entitled to vote on all matters and is entitled to one vote for each share held. The Company will, at all times, reserve and keep available, out of its authorized but unissued shares of common stock, sufficient shares to effect the conversion of the shares of the stock options.

Follow-on Public Offerings

On May 16, 2013, the Company completed a follow-on public offering selling 4,137,931 shares at an offering price of $14.50 per share. On May 22, 2013, the underwriters exercised in full their over-allotment option to purchase an additional 620,689 shares at an offering price of $14.50 per share. Aggregate gross proceeds from the follow-on public offering, including the exercise of the over-allotment option, were $69.0 million, and net proceeds received after underwriting fees and offering expenses were approximately $64.5 million.

Also, on January 8, 2015, the Company completed a second follow-on public offering, selling 3,800,000 shares at an offering price of $15.75 per share. On February 10, 2015, the underwriters exercised in full their over-allotment option to purchase an additional 553,877 shares at an offering price of $15.75 per share. Aggregate gross proceeds from this follow-on public offering, including the exercise of the over-allotment option, were $68.6 million, and net proceeds received after underwriting fees and offering expenses were approximately $64.1 million.

In addition, on January 20, 2017, the Company completed a third follow-on public offering, selling 4,500,000 shares at an offering price of $10 per share. Additionally, the underwriters exercised in full their over-allotment option to purchase an additional 675,000 shares at an offering price of $10 per share. Aggregate gross proceeds from this follow-on public offering, including the exercise of the over-allotment option, were $51.8 million, and net proceeds received after underwriting fees and offering expenses were approximately $48.2 million.

On January 26, 2018, the Company completed a fourth follow-on public offering, selling 3,700,000 shares at an offering price of $14 per share. Additionally, the underwriters exercised in full their over-allotment option to purchase an additional 555,000 shares at an offering price of $14 per share. Aggregate gross proceeds from the follow-on public offering, including the exercise of the over-allotment option, were $59.6 million, and net proceeds received after underwriting fees and offering expenses were approximately $55.5 million.

The Company may seek additional capital through a combination of private and public equity offerings, debt financings, collaborations and strategic and licensing arrangements.

10. Grant Revenue

In October 2013, the Company entered into a contract relating to the Therapy Acceleration Program ("the TAP Agreement") with The Leukemia and Lymphoma Society ("LLS"). LLS is a national voluntary health agency which, among other activities, encourages and sponsors research relating to blood cancers to develop therapies to cure or mitigate these diseases. To further its mission, LLS provides research funding to entities that can demonstrate, after LLS's review process, that their proposed research projects have scientific promise to advance LLS's effort to find treatments and cures for blood cancers and their complications. Pursuant to the TAP Agreement, LLS agreed to provide funding to the Company not to exceed $3.5 million to fund the Company's development program related to the Company's preclinical and clinical product development activities. Through December 31, 2017, the Company has received $3.0 million based on milestones achieved. The Company could receive the additional $0.5 million based on the completion of a milestone event. The Company has recognized approximately $0.9 million, $1.0 million and $0.7 million of revenue related to the LLS grant for the years ended December 31, 2017, 2016, and 2015, respectively, which reflects revenue recognized, respectively, on a straight-line basis, based on the Company's best estimates of work performed and qualifying costs incurred. The TAP agreement terminates when there are no longer any payment obligations.

11. Stock-Based Compensation

The 2016 Plan was adopted by the board of directors and approved by the stockholders in May 2016. The 2016 Plan authorizes the Company to grant up to 1,812,932 shares of common stock to eligible employees, directors, and non-employee consultants and advisors to the Company. Under the provisions of the 2016 Plan, no option will have a term in excess of 10 years.

The Company's 2012 Stock Equity Incentive Plan (the "2012 Plan"), which was adopted by the board of directors and approved by the stockholders in July 2012, became effective immediately prior to the closing of the Company's initial public offering. In addition, the Company's 2004 Stock Option and Grant Plan (the "2004 Plan") was terminated effective immediately prior to the closing of the Company's initial public offering. The 2012 Plan authorized the Company to grant up to 1,663,727 shares of common stock to eligible employees, directors, and non-employee consultants and advisors to the Company in the form of options to purchase common stock of the Company at a price not less than the estimated fair value at the date of grant. Under the provisions of the 2012 Plan, no option will have a term in excess of 10 years. With the adoption of the 2016 plan, all authorized but un-issued shares, totaling 12,932, under the 2012 plan were converted to the 2016 plan. All future awards will be granted out of the 2016 plan.

As of December 31, 2017, there were 1,089,975 shares of common stock available for future grants under the 2016 Plan.

Total compensation cost that has been charged against operations related to the above plans was approximately $8.6 million, $7.4 million and $5.2 million for the years ended December 31, 2017, 2016 and 2015, respectively. The exercise of stock options and the vesting of restricted stock during the year ended December 31, 2017 generated an income tax deduction of approximately $4.2 million. In January 2017, the Company adopted ASU No. 2016-09, Compensation-Stock Compensation (Topic 718): Improvements to Share-Based Payment Accounting which changes the accounting for certain aspects of share-based payments to employees. One aspect of the standard requires that excess tax benefits and deficiencies that arise upon vesting or exercise of share-based payments be recognized as an income tax benefit and expense in the income statement. Previously, such amounts were recognized as an increase and decrease in additional paid-in capital. This aspect of the standard was adopted prospectively, and accordingly the benefit from income taxes for the year ended December 31, 2017, included $0.6 million, of excess tax benefits arising from share-based payments during the period of adoption. No income tax benefit was recognized in the statements of operations for share-based compensation arrangements for the years ended December 31, 2016 and 2015.

Additionally, the new standard requires that historical excess tax benefits that were not previously recognized because the related tax deduction had not reduced current taxes should be recognized on a modified retrospective basis as a cumulative-effect adjustment to retained earnings as of the beginning of the annual period of adoption. Consequently, the Company recognized gross deferred tax assets of approximately $14.8 million relating to excess tax benefits of stock-based compensation during the year of adoption, with a corresponding cumulative-effect adjustment to accumulated deficit.

The following table summarizes stock-based compensation related to the above plans by expense category for the years ended December 31, 2017, 2016 and 2015:

	Year Ended December 31,		
	2017	2016	2015
Research and development	$ 3,732,742	$ 3,186,783	$ 2,703,861
General and administrative	4,885,778	4,244,343	2,459,101
Total	$ 8,618,520	$ 7,431,126	$ 5,162,962

Stock Options

The Company grants stock options to employees, directors and non-employee consultants, with exercise prices equal to the closing price of the underlying shares of the Company's common stock on the date that the options are granted. Options granted have a term of 10 years from the grant date. Options granted to employees generally vest over a four-year period from date of grant or if vesting based on market condition, awards vest based on the derived service period which is the estimated period of time that would be required to satisfy the market condition. Options granted to directors vest in equal yearly installments over a three-year period from the date of grant. Options to directors are granted on an annual basis and represent compensation for services performed on the Board of Directors. Compensation cost for stock options granted to employees and directors is charged against operations using the straight-line attribution method between the grant date for the option and each vesting date. The Company estimates the fair value of stock options on the grant date by applying the Black-Scholes option pricing valuation model. The application of this valuation model involves assumptions that are highly subjective, judgmental and sensitive in the determination of compensation cost.

The weighted-average key assumptions used in determining the fair value of options granted for the years ended December 31, 2017, 2016 and 2015, are as follows:

	Year Ended December 31,		
	2017	2016	2015
Risk-free interest rate	1.92%	1.56%	1.86%
Expected volatility	75.76%	73.53%	77.92%
Dividend yield	—	—	—
Expected life	6.07 years	6.03 years	6.15 years

Due to the lack of trading history, the Company's computation of stock-price volatility is based on the volatility rates of comparable publicly held companies over a period equal to the estimated useful life of the options granted by the Company. The Company's computation of expected life was determined using the "simplified" method which is the midpoint between the vesting date and the end of the contractual term. The Company believes that it does not have sufficient reliable exercise data in order to justify the use of a method other than the "simplified" method of estimating the expected exercise term of employee stock option grants. The Company has paid no dividends to stockholders. The risk-free interest rate is based on the zero-coupon U.S. Treasury yield at the date of grant for a term equivalent to the expected term of the option.

For the years ended December 31, 2017, 2016 and 2015 the Company issued 23,952 shares, 87,786 shares and 198,938 shares of the Company's common stock, respectively, upon the exercise of outstanding stock options and received proceeds of approximately $141,740, $264,196 and $725,017, respectively. For the years ended December 31, 2017, the company received a tax benefit of $0.6 million from the exercise of stock options, and for the years ended December 31, 2016 and December 31, 2015, the Company realized no tax benefit from the exercise of stock options. As of December 31, 2017, there was approximately $5.1 million of unrecognized compensation cost related to unamortized stock option compensation which is expected to be recognized over a remaining weighted-average period of approximately 1.43 years.

The Company's stock options outstanding at December 31, 2017, 2016 and 2015 and changes during the years ended December 31, 2017, 2016 and 2015 are presented below:

	Options	Weighted-Average Exercise Price	Weighted-Average Remaining Contractual Life	Aggregate Intrinsic Value
Outstanding at December 31, 2014	1,643,532	$ 8.64		
Options granted	702,094	13.74		
Options exercised	(198,938)	3.64		
Options forfeited	(24,962)	13.22		
Outstanding at December 31, 2015	2,121,726	$ 10.74		
Options granted	1,132,972	5.29		
Options exercised	(87,786)	3.01		
Options forfeited	(76,160)	18.17		
Outstanding at December 31, 2016	3,090,752	$ 8.78		
Options granted	131,000	8.40		
Options exercised	(23,952)	5.92		
Options forfeited	(22,836)	15.51		
Outstanding at December 31, 2017	3,174,964	$ 8.74	6.25	$ 24,508,881
Options exercisable at December 31, 2017	1,912,882	$ 9.22	5.04	$ 14,507,809

The aggregate intrinsic value in the previous table reflects the total pretax intrinsic value (the difference between the Company's closing stock price on the last trading day of the period and the exercise price of the options, multiplied by the number of in-the-money stock options) that would have been received by the option holders had all option holders exercised their options on December 31, 2017. The intrinsic value of the Company's stock options changes based on the closing price of the Company's common stock. The total intrinsic value of options exercised (the difference in the market price of the Company's common stock on the exercise date and the price paid by the optionee to exercise the option) was approximately $0.2 million, $0.7 million and $1.4 million for the years ended December 31, 2017, 2016 and 2015, respectively.

Restricted Stock

The Company grants restricted stock to its employees, directors, and non-employee consultants. Restricted stock is recorded as deferred compensation and charged against income on a straight-line basis over the vesting period, which ranges from immediate to four years in duration. If vesting of the award is based on a market condition, awards vest based on the derived service period which is the estimated period of time that would be required to satisfy the market condition. Restricted stock to directors is granted on a yearly basis and represents compensation for services performed on the Company's Board of Directors. Restricted stock awards to directors vest in equal installments over a three-year period from the grant date. Compensation cost for restricted stock is based on the award's grant date fair value, which is the closing market price of the Company's common stock on the grant date, multiplied by the number of shares awarded.

The Company's non-vested restricted stock at December 31, 2017, 2016 and 2015, and changes during the years ended December 31, 2017, 2016 and 2015 are presented below:

	Number of Shares	Weighted-Average Grant Date Fair Value Per Share
Outstanding at December 31, 2014	283,446	$ 19.36
Shares granted	399,742	11.66
Shares vested	(116,167)	18.50
Shares forfeited	(13,976)	22.00
Outstanding at December 31, 2015	553,045	13.91
Shares granted	920,444	4.93
Shares vested	(165,400)	14.30
Shares forfeited	(39,997)	11.80
Outstanding at December 31, 2016	1,268,092	$ 7.41
Shares granted	914,950	8.91
Shares vested	(416,157)	9.02
Shares forfeited	(42,048)	6.92
Outstanding at December 31, 2017	1,724,837	$ 7.83

For the year ended December 31, 2017, the Company granted 914,950 shares of restricted stock, at a weighted-average grant date fair value of $8.91 per share amounting to approximately $8.2 million in total aggregate fair value. At December 31, 2017, approximately 1,724,837 shares remained unvested and there was approximately $9.8 million of unrecognized compensation cost related to restricted stock which is expected to be recognized over a remaining weighted-average period of approximately 2.0 years. The total fair value of restricted stock vested during the year ended December 31, 2017 was approximately $3.8 million.

Performance Share Awards

Subsequent to the closing of the IPO, certain options and restricted stock began to vest to directors, consultants and key employees. The Company recorded approximately $1.4 million of stock-based compensation expense associated with 573,424 options with a weighted average exercise price of $2.38 and 8,625 shares of restricted stock that fully vested upon consummation of the IPO. In addition, 281,895 options commenced vesting based upon the consummation of the IPO. The Company has recorded approximately $1.8 million on the vesting of these options over their expected lives.

For awards with performance conditions, such as capital raises, an IPO, a change in control or a sale of the company, no expense is recognized, and no measurement date can occur, until the occurrence of the event is probable.

Awards Granted to Non-Employees

The Company grants stock options, restricted stock, and unrestricted stock to non-employee consultants. The Company periodically re-measures the fair value of stock-based awards issued to non-employees and records expense over the requisite service period. Total compensation cost that has been charged against operations related to stock-based awards granted to non-employee consultants was approximately $0.8 million, $0.5 million and $0.3 million for the years ended December 31, 2017, 2016 and 2015, respectively.

Employee Stock Purchase Plan

In September 2015, the Company adopted its 2015 Employee Stock Purchase Plan (the "2015 ESPP"). The 2015 ESPP is qualified as an employee stock purchase plan under Section 423 of the Internal Revenue Code of 1986, as amended (the "IRC"). Under the 2015 ESPP, the Company will grant rights to purchase shares of common stock under the 2015 ESPP at prices not less than 85% of the lesser of (i) the fair value of the shares on the date of grant of such rights or (ii) the fair value of the shares on the date such rights are exercised. Therefore, the 2015 ESPP is considered compensatory under FASB ASC 718 since, along with other factors, it includes a purchase discount of greater than 5%. For the twelve months ended December 31, 2017, the Company recorded approximately $68,496 of compensation expense, related to participation in the 2015 ESPP.

12. Income Taxes

For the year ended December 31, 2017, 2016, and 2015, the Company recognized $0, $25,296, and $0, respectively. The $25,296 of income tax benefit in 2016 was a result of the application of intraperiod tax allocation provisions of ASC 740, under which the Company is required to consider all items (including items recorded in other comprehensive income) in determining the amount of tax benefit that should be allocated to net loss. The non-cash income tax benefit was offset in full by income tax expense recorded in other comprehensive income. For years shown, components of the Company's income tax expense (benefit) were as follows:

	2017	2016	2015
Deferred:			
Federal	$ (8,370,870)	$ (16,917,231)	$ (17,654,150)
State and local	4,968,667	(3,063,684)	(3,735,379)
	(3,402,203)	(19,980,915)	(21,389,529)
Increase in valuation allowance	3,402,203	19,955,619	21,389,529
Total tax expense	$ —	$ (25,296)	$ —

A reconciliation of the statutory U.S. federal rate to the Company's effective tax rate is as follows:

	Year Ended December 31,		
	2017	2016	2015
Percent of pre-tax income:			
U.S. federal statutory income tax rate	(34.0)%	(34.0)%	(34.0)%
State taxes, net of federal benefit	(3.6)	(7.5)	(11.0)
Permanent items	5.6	7.0	7.9
R&D Credit	(14.8)	(17.2)	(21.3)
ASU 2016-09	(7.2)	—	—
Change in state deferred rate	10.8	(0.5)	1.0
Impact of tax reform	38.2	—	—
Change in valuation allowance	5.0	52.1	57.4
Effective income tax rate	—%	(0.1)%	—%

The tax effects of temporary differences that gave rise to significant portions of the deferred tax assets were as follows:

	December 31,	
	2017	2016
Current deferred tax assets:		
Accrued expenses	$ —	$ 1,478,437
Valuation allowance	—	(1,478,437)
Total current deferred tax assets	$ —	$ —
Noncurrent deferred tax assets:		
Accrued expenses	$ 784,671	$ —
Net operating loss carryforwards	34,220,690	28,412,080
Research and Development	31,591,504	21,536,090
Depreciable and Amortizable Assets	12,131,537	21,683,793
Nonqualified stock compensation	3,079,231	5,295,029
	81,807,633	76,926,992
Valuation allowance	(81,807,633)	(76,926,992)
Total noncurrent deferred tax assets	$ —	$ —

In assessing its ability to realize deferred tax assets, the Company considers whether it is more likely than not that some portion or all of the deferred tax assets will not be realized. The ultimate realization of deferred tax assets is dependent upon the generation of future taxable income during the periods in which the temporary differences representing net future deductible amounts become deductible.

Due to the Company's history of losses, the deferred tax assets are fully offset by a valuation allowance at December 31, 2017, 2016 and 2015.

The following table summarizes carryforwards of net operating losses and tax credits as of December 31, 2017:

	Amount	Expiration
Federal net operating losses	$ 122,768,866	2023-2037
State net operating losses	$ 127,278,196	2023-2037
Research and development credits	$ 31,591,504	2023-2037

The Internal Revenue Code of 1986, as amended (the "Code") provides for a limitation of the annual use of net operating losses and other tax attributes (such as research and development tax credit carryforwards) following certain ownership changes (as defined by the Code) that could limit the Company's ability to utilize these carryforwards. At this time, the Company has not completed a study to assess whether an ownership change under section 382 of the Code has occurred, or whether there have been multiple ownership changes since the Company's formation, due to the costs and complexities associated with such a study. The Company may have experienced various ownership changes, as defined by the Code, as a result of past financing transactions. Accordingly, the Company's ability to utilize the aforementioned carryforwards may be limited. Additionally, U.S. tax laws limit the time during which these carryforwards may be applied against future taxes. Therefore, the Company may not be able to take full advantage of these carryforwards for federal or state income tax purposes.

The Company did not have unrecognized tax benefits as of December 31, 2017 and does not expect this to change significantly over the next twelve months. As of December 31, 2017, the Company has not accrued interest or penalties related to uncertain tax positions. The Company's tax returns for the years ended December 31, 2009 through December 31, 2017 are still subject to examination by major tax jurisdictions.

The Company will recognize interest and penalties related to uncertain tax positions in income tax expense. As of December 31, 2017, 2016 and 2015, the Company had no accrued interest or penalties related to uncertain tax positions and no amounts have been recognized in the Company's consolidated statements of operations and comprehensive loss.

For all years through December 31, 2017, the Company generated research credits but has not conducted a study to document the qualified activities. This study may result in an adjustment to the Company's research and development credit carryforwards; however, until a study is completed and any adjustment is known, no amounts are being presented as an uncertain tax position for any years. A full valuation allowance has been provided against the Company's research and development credits and, if an adjustment is required, this adjustment would be offset by an adjustment to the deferred tax asset established for the research and development credit carryforwards and the valuation allowance.

13. Commitments and Contingencies

License Agreements

The Company has entered into research and development agreements with third-parties for the development of oncology products. These agreements require the Company to fund the development of such products and potentially make milestone payments and royalties on net sales in the future based on the Company's successful development of the products. The timing and the amount of milestone payments in the future are not certain.

Under the Company's license agreements, the Company could be required to pay up to a total of $217.2 million upon achieving certain milestones, such as the initiation of clinical trials or the granting of patents. From inception through December 31, 2017, the Company has paid or accrued $6.2 million in payments resulting from the execution of certain agreements, patent approvals, the initiation of sponsor research agreements, and compound development agreements. Milestone payments will also be due upon the issuance of certain patents, the initiation of certain clinical trials, the submission of regulatory applications and certain regulatory approvals, in addition to sales milestones and single digit royalties payable on commercial sales if any occur.

Scott and White Memorial Hospital

In June 2006, the Company entered into a research and license agreement, as amended in December 2008, March 2010 and July 2011 (collectively the "S&W Agreement"), with Scott and White Memorial Hospital and Scott, Sherwood and Brindley Foundation, and its affiliate Scott and White Clinic (collectively "S&W") for the rights to SL-401 and SL-501. SL-401 is a clinical stage targeted therapy directed to the interleukin-3 receptor, or IL-3R, and is being developed to treat patients with hematologic cancers. SL-501 is a next generation IL-3R-targeted therapy and is in preclinical development. The Company have made certain payments to Scott and White for such research services pursuant to the agreement, which through December 31, 2017, totals approximately $1.0 million in the aggregate. The Company is required to pay single-digit royalties on sales of these products, if any, and a percentage of upfront payments the Company may receive from a sublicensee. The S&W Agreement will expire in its entirety upon the later of (i) the 10th anniversary of the first commercial sale of a product and (ii) the expiration of the last issued patent claiming or covering a product. The Company may terminate the S&W Agreement at its sole discretion at any time after a specified number of days following written notice and either party may terminate for a material breach of the agreement that is not cured within a specified number of days.

University of Pittsburgh

In September 2009, the Company entered into an exclusive license agreement with the University of Pittsburgh ("UP") that covers patent rights claiming composition of a shortened variant of the IL-13Rα2 peptide (the "UP Agreement") utilized by the Company in its SL-701 composition. The Company paid UP an upfront license fee that was expensed to research and development cost for the year ended December 31, 2009. In addition to the upfront payment, the Company is required to pay annual fees, milestone payments (which are contingent upon achievement of pre-defined clinical, regulatory and commercial events), and, upon regulatory approval, single-digit royalty payments on net sales, and a percentage of non-royalty revenue from sublicensees, which decreases if the applicable sublicense agreement is entered into after a certain clinical milestone has been met. Through December 31, 2017, the Company have paid an aggregate of approximately $0.7 million in fees to the University under the agreement. The Company also must make certain payments to the University of up to approximately $4.2 million upon the achievement of specific regulatory and commercial milestone events. The UP Agreement will expire in its entirety upon the expiration of the last issued patent claiming or covering the product. The Company may terminate the UP Agreement at its sole discretion at any time after a specified number of

days following written notice and UP may terminate for a material breach of the agreement by the Company that is not cured within a specified number of days.

In March 2012, the Company entered into a non-exclusive license agreement with UP that covers patent rights claiming use of a shortened peptide of EphA2, which the Company may use for the diagnosis, treatment or prevention of diseases and tumors of the brain and which the Company is currently utilizing in its SL-701 composition. The Company paid UP an initial license fee and is required to pay UP annual license maintenance fees until the first commercial sale of a licensed product, a single-digit royalty on sales, and a minimum annual royalty following the first commercial sale of a licensed product. Through December 31, 2017, the Company have paid an aggregate of approximately $0.1 million in fees to the University under the agreement. The UP Agreement will expire in its entirety upon the expiration of the last issued patent claiming or covering the product. The Company may terminate the UP Agreement at its sole discretion at any time after a specified number of days following written notice and UP may terminate for a material breach of the agreement by the Company that is not cured within a specified number of days.

In March 2012, the Company entered into a non-exclusive license agreement with UP for the right to use certain information and data contained in the applications for investigational new drugs, or INDs, relating to clinical trials with an earlier version of SL-701 that were conducted by UP. The Company may use the information and data for the development, manufacture, regulatory approval and commercialization of pharmaceutical products. The Company paid UP an initial license fee and will be required to make a payment following a specified regulatory milestone, and a percentage of non-royalty revenue it may receive from any sublicensees. Through December 31, 2017, the Company have paid an aggregate of approximately $27,500 in fees to the University under the agreement. The UP Agreement will expire in its entirety in March 2032 unless earlier terminated by a party. The Company may terminate the UP Agreement at its sole discretion at any time prior to incorporating or referencing the data or UP INDs, after a specified number of days following written notice, and UP may terminate for a material breach of the agreement by the Company that is not cured within a specified number of days or if the IL-13Rα2 license agreement is terminated.

CanBas, Ltd

On December 26, 2014, the Company entered into a license agreement with CanBas, Ltd. (“CanBas”) for SL-801. SL-801 is a small molecule inhibitor of XPO1. Under the terms of the agreement, CanBas has granted the Company an exclusive, royalty-bearing, worldwide (excluding Japan, Korea, China and Taiwan) license, under certain patent rights, know-how and materials to research, develop, make, have made, formulate, use, sell, offer to sell and import SL-801, and any products containing or comprising such compound in finished dosage pharmaceutical form. The patent rights exclusively licensed to the Company under the agreement, which include issued patents in the U.S. and abroad, cover both composition of matter and use of SL-801. The Company has additional pending patent applications directed to SL-801 which would provide additional protection, should they issue, in certain non-U.S. territories.

The Company is responsible to pay one remaining annual technical advisory fee in December 2018 totaling 110 million Japanese Yen (JPY), if the clinical development continues over this time period. Additionally, the Company must pay CanBas tiered royalties based on aggregate net sales, by the Company or its sublicensees, of products containing the licensed compound until the latest date of a period of ten years following the first commercial sale of each product in each country; the date upon which there are no more valid claims or the expiration or termination of the last regulatory exclusivity period. The royalty rates start in the low single digits and are tiered up based on annual net sales. In the future, the Company may also be responsible, based on the achievement of specific clinical-development, regulatory and sales-based commercial milestones, for certain payments to CanBas of up to $86 million. The Company has sublicensing rights under this agreement, in which the Company would pay CanBas a standard percentage of the payments received by the Company from a sublicensee.

The Company must exercise commercially reasonable efforts to develop and commercialize a licensed product and to achieve certain regulatory milestones within certain periods, subject to extensions based on unforeseen technical, scientific, intellectual property or regulatory issues.

The agreement survives until the later of ten years following the first commercial sale of each product in each country; the date upon which there are no more valid claims; or the expiration or termination of the last regulatory exclusivity period, after which the Company’s license becomes fully paid, irrevocable, perpetual, non-exclusive and royalty-free. The Company may terminate the license for any or no reason upon 60 days advance written notice to CanBas. If either party breaches a material obligation under the agreement and such obligation is not cured within a specified period of time following written notice from the other party, then the non-breaching party may terminate the agreement upon an additional written notice.

UCB Biopharma Sprl

On December 31, 2017, the Company entered into a license agreement with UCB Biopharma Sprl ("UCB") for SL-901. SL-901 is a small molecule kinase inhibitor. Prior to in licensing, the agent had shown preclinical activity in several tumor models, and was evaluated in a small Phase 1 clinical trial in Europe. Neither a dose limiting toxicity ("DLT") nor a maximum tolerated dose ("MTD") was reached in the trial, and a partial response in one patient with advanced lung cancer was reported. We are currently evaluating plans to produce drug supply under good manufacturing practice ("GMP") and conduct necessary non-clinical studies to enable a new regulatory filing to continue clinical dose escalation.

UCB has granted the Company an exclusive, royalty-bearing, worldwide license, under certain patent rights, know-how and materials to research, develop, make, have made, formulate, use, sell, offer to sell and import SL-901, and any products containing or comprising such compound in finished dosage pharmaceutical form. In January 2018, the Company paid UCB an up-front consideration payment of $0.5 million. In the future, the Company may also be responsible, based on the achievement of specific clinical development, regulatory and sales-based commercial milestones, for certain payments to UCB of up to $111 million, largely consisting of approval and post-approval payments. The Company has sublicensing rights under this agreement, in which the Company would pay UCB a standard percentage of the payments received by the Company from a sublicensee.

Additionally, the Company may be obligated to pay UCB tiered royalties based on aggregate net sales, by the Company or its sublicensees, of products containing the licensed compound until the latest date of a period of ten years following the first commercial sale of each product in each country; the date upon which there are no more valid claims or the expiration or termination of the last regulatory exclusivity period. The royalty rates are in the low double digits and are tiered up based on annual net sales.The Company must exercise commercially reasonable efforts to develop, obtain regulatory approval and commercialize a licensed product in at least one indication in both the U.S. and EU. The licenses granted are contingent upon the Company achieving certain milestone events within specific timeframes.

The agreement survives until the date upon which the Company's obligation to pay royalties has expired in each specific country, after which the Company's license becomes fully paid, irrevocable, perpetual, non-exclusive and royalty-free. The Company may terminate the license for breach upon 45 days advance written notice to UCB. If either party breaches a material obligation under the agreement and such obligation is not cured within a specified period of time following written notice from the other party, then the non-breaching party may terminate the agreement upon an additional written notice.

Other

The Company has also licensed rights to certain technologies or intellectual property in the field of oncology. The Company is generally required to make upfront payments as well as other payments upon successful completion of preclinical, clinical, regulatory or sales milestones. In addition, these agreements generally require the Company to pay royalties on sales of the products arising from these agreements. These agreements generally permit the Company to terminate the agreement with no significant continuing obligation.

As part of the agreements discussed above, the Company has committed to make potential future milestone payments to third-parties as part of its licensing agreements. Payments generally become due and payable only upon the achievement of certain developmental, regulatory and/or commercial milestones. Because the achievement of these milestones is neither probable nor reasonably estimable, the Company has not recorded a liability on its balance sheet for any such contingencies.

Contractual Agreements

In February 2013, the Company entered into a bioprocessing services agreement with a vendor for approximately $2.9 million if all services are performed under the contract. As of December 31, 2014, the contract services were performed on the initial work order and had been paid by the Company. During 2014 through 2017, the Company entered into new work order agreements with this vendor totaling approximately $23.5 million, with services to be rendered on these agreements through 2018. From inception through December 31, 2017, the Company has received and paid for services relating to these agreements in the amount of $15.9 million.

The Company has agreements in place with CROs in connection with its clinical programs. The Company's total expenditures in the future would be approximately $5.3 million assuming the successful advancement of its programs.

Lease Agreement

In July 2013, the Company entered into a leasing agreement with respect to its corporate offices at 750 Lexington Avenue, New York, New York, for a monthly rent of $50,625. The term of this lease agreement was 36 months.

In February 2016, the Company entered into a new leasing agreement with respect to its corporate offices at 750 Lexington Avenue, New York, New York, for a monthly rent of $69,750 and a 42-month term. The term of this lease agreement commenced on July 1, 2016 and is set to expire on December 31, 2019. The aggregate minimum lease commitment over the 42-month term of the lease is approximately $2.7 million. The Company has provided the landlord with a security deposit equal to three months' rent, totaling $209,250, recorded in other assets.

The Company's future annual minimum lease payments for each of the following calendar years are as follows:

2018	$	837,000
2019		837,000
Total minimum payments	$	1,674,000

Rent expense charged to operations was $0.8 million, $0.7 million, and $0.6 million for the years ended December 31, 2017, 2016, and 2015, respectively. Rent expense is included in general and administrative expenses in the Company's Statements of Operations.

Contingencies

On March 15, 2018, the United States District Court for the Southern District of New York granted the defendants' motion to dismiss in its entirety a consolidated shareholder action against the Company, its directors, certain of its officers, and Jefferies LLC. All claims in the Amended Complaint are to be dismissed with prejudice unless Plaintiffs file for leave to amend their complaint within thirty days of March 15, 2018.

In February 2017, four putative class action lawsuits were filed against the Company and certain of its officers and directors in the Southern District of New York, alleging violations of the Exchange Act and Rule 10b-5 promulgated thereunder and violations of Section 11 and 15 of the Securities Act of 1933, or the Securities Act, arising from the Company's January 2017 follow-on public offering. Each of lawsuits was premised upon allegations that the defendants made false and misleading statements and/or omissions by failing to earlier disclose that a cancer patient in a Stemline clinical trial of SL-401 who experienced the side effect of CLS died on January 18, 2017. Additionally, the complaints alleged that, as a result of the foregoing, certain of the defendants' statements about Stemline's business, operations, and prospects were materially false and misleading and/or lacked a reasonable basis. In April 2017, the United States District Court for the Southern District of New York consolidated these four shareholder actions into a single action and appointed three purported individual investors in the Company as Lead Plaintiff to represent the proposed class. This class appointed Pomerantz LLP and the Rosen Law Firm as Co-Lead Counsel. On June 26, 2017, Lead Plaintiffs filed an amended complaint in the consolidated action, naming as defendants, the Company, its directors, certain of its officers, and Jefferies LLC, and alleging violations of the Exchange Act and Rule 10b-5 promulgated thereunder during the period from January 20, 2017 through February 1, 2017, as well as violations of the Securities Act arising from the Company's January 2017 follow-on public offering. On March 15, 2018, the United States District Court for the Southern District of New York granted the defendants' motion to dismiss in its entirety the consolidated action. All claims in the Amended Complaint are to be dismissed with prejudice unless Plaintiffs file for leave to amend their complaint within thirty days of March 15, 2018.

On May 2, 2017, a shareholder derivative litigation was filed in New York Supreme Court in New York County against all of the Company's directors and certain of the Company's officers in litigation captioned Hancock v. Bergstein. The Company is named only as a nominal defendant. The suit alleges that the officers and directors breached their fiduciary duties to the Company, unjustly enriched themselves, wasted corporate assets, abused their control, and grossly mismanaged the Company. The claims are based on allegations that the defendants engaged in improper conduct by failing to disclose in connection with its follow-on public offering of stock on January 20, 2017, that a cancer patient in a Stemline clinical trial died on January 18, 2017. It is alleged that the non-disclosure of that adverse event in the follow-on public offering has led us to incur losses, including defense and investigation costs, and allowed the defendants to reap substantial financial rewards in the form of bonuses and other compensation.

The Company intends to vigorously defend against these actions. However, there is no assurance that we will be successful in the Company's defense or that insurance will be available or adequate to fund any settlement or judgment or the litigation costs of the actions. The Company is unable to predict the outcome or reasonably estimate a range of possible loss at this time.

14. Related Party

On June 15, 2012, the Company entered into an assignment agreement with Dr. Bergstein, the Company's Chairman, President and Chief Executive Officer and owner of certain proprietary patent rights and related technology. Pursuant to the assignment agreement, as amended on November 7, 2012, effective immediately prior to the registration statement for the Company's initial public offering being declared effective by the Securities and Exchange Commission, Dr. Bergstein agrees to assign, sell, transfer and convey to the Company all of his right, title and interest in and to these patent rights and related technology in exchange for $2.0 million in cash or a combination of cash and shares of Company common stock, payable only if, within five years of the date of transfer, the Company either (i) has a change in control, as defined in the assignment agreement, or (ii) achieves a market capitalization of at least $200 million for a prescribed period. Under the terms of the assignment agreement, as amended, 50% of such payment shall be paid in cash and the remaining 50% may be paid in shares of Company common stock, or a combination of cash and common stock, as determined by the Company. If the Company elects to settle payment in shares, the Company will value the shares at the date of issuance. None of the assigned patent rights and related technology has alternative future uses, nor have they reached a stage of technological feasibility. The Company accounted for this transaction as an asset acquisition as it achieved a market capitalization of $200 million for the prescribed period because it did not acquire any processes or activities in addition to the assigned rights and technology. The Company has paid the $2.0 million in cash and recorded the entire purchase price to acquire in-process research and development expense for the year ended December 31, 2013. The assignment agreement does not contain any vesting or rescission/refund provisions.

15. Other Income

The components of other income for the years ended December 31, 2017, 2016 and 2015 are as follows:

	Year Ended December 31,		
Other Income:	**2017**	**2016**	**2015**
Other (expense)/income	(6,330)	11,438	1,609
Total other (expense)/income	$ (6,330)	$ 11,438	$ 1,609

Other income includes short term and long term capital gains from the sale of the Company's investment securities, net of any capital losses.

16. Subsequent Events

On January 26, 2018, the Company completed a fourth follow-on public offering, selling 3,700,000 shares at an offering price of $14 per share. Additionally, the underwriters exercised in full their over-allotment option to purchase an additional 555,000 shares at an offering price of $14 per share. Aggregate gross proceeds from the follow-on public offering, including the exercise of the over-allotment option, were $59.6 million, and net proceeds received after underwriting fees and offering expenses were approximately $55.5 million.

During January 2018, the Company's warrant holders elected to exercise warrants to purchase 99,529 shares of the Company's common stock whereby the Company received approximately $0.4 million in connection with this exercise. A portion of the transaction was processed via a cashless exercise. As of January 31, 2018, no warrants to purchase the Company's stock were outstanding.

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17. Selected Quarterly Financial Data (Unaudited)

	Quarters Ended			
	March 31	June 30	September 30	December 31
2017				
Net loss attributable to common stockholders	$ (14,566,010)	$ (15,456,936)	$ (16,056,147)	$ (21,749,301)
Basic and diluted net loss per common share	$ (0.67)	$ (0.66)	$ (0.68)	$ (0.93)
2016				
Net loss attributable to common stockholders	$ (9,048,544)	$ (9,322,143)	$ (9,923,222)	$ (10,009,096)
Basic and diluted net loss per common share	$ (0.51)	$ (0.52)	$ (0.56)	$ (0.56)

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SIGNATURES

Pursuant to the requirements of Section 13 or 15(d) of the Securities Exchange Act of 1934, as amended, the registrant has duly caused this report to be signed on its behalf by the undersigned, thereunto duly authorized.

Date: March 16, 2018

STEMLINE THERAPEUTICS, INC

By: /s/ Ivan Bergstein, M.D.
Ivan Bergstein, M.D.
Chairman, President and Chief Executive Officer

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POWER OF ATTORNEY

KNOW ALL MEN BY THESE PRESENTS, that each person whose signature appears below constitutes and appoints Ivan Bergstein, M.D. his true and lawful attorney-in-fact and agent, with full power of substitution and resubstitution, for him and his name, place and stead, in any and all capacities, to sign any or all amendments to this annual report on Form 10-K, and to file the same, with all exhibits thereto and other documents in connection therewith, with the SEC, granting unto said attorney-in-fact and agent, full power and authority to do and perform each and every act and thing requisite and necessary to be done in and about the premises, as fully to all intents and purposes as he might or could do in person, hereby ratifying and confirming all that said attorney-in-fact and agent or any of his substitutes, may lawfully do or cause to be done by virtue hereof.

Pursuant to the requirements of the Securities Exchange Act of 1934, as amended, this Form 10-K has been signed by the following persons on behalf of the Registrant on March 16, 2018, and in the capacities indicated:

Signatures	Title
/s/ Ivan Bergstein, M.D. Ivan Bergstein, M.D.	Chairman, President and Chief Executive Officer (Principal Executive Officer)
/s/ David G. Gionco David G. Gionco	Vice President of Finance and Chief Accounting Officer (Principal Financial and Accounting Officer)
/s/ Ron Bentsur Ron Bentsur	Director
/s/ Eric L. Dobmeier Eric L. Dobmeier	Director
/s/ Kenneth Zuerblis Kenneth Zuerblis	Director
/s/ Alan Forman Alan Forman	Director

Exhibit 21.1

STEMLINE THERAPEUTICS, INC.
List of Subsidiaries

Stemline Therapeutics, Inc. does not have any subsidiaries.

Exhibit 23.1

Consent of Independent Registered Public Accounting Firm

We consent to the incorporation by reference in the following Registration Statements:

(1) Registration Statement (Form S-8 No. 333-188115) pertaining to Stemline Therapeutics, Inc. 2012 Equity Incentive Plan and Stemline Therapeutics, Inc. Amended and Restated 2004 Employee, Director and Consultant Stock Plan,

(2) Registration Statement (Form S-8 No. 333-206303) pertaining to Stemline Therapeutics, Inc. 2015 Employee Stock Purchase Plan,

(3) Registration Statement (Form S-8 No. 333-211784) pertaining to Stemline Therapeutics, Inc. 2016 Equity Incentive Plan,

(4) Registration Statement (Form S-3 No. 333-219794) of Stemline Therapeutics, Inc. and,

(5) Registration Statement (Form S-8 No. 333-219796) pertaining to Stemline Therapeutics, Inc. 2016 Equity Incentive Plan;

of our report dated March 16, 2018, with respect to the financial statements of Stemline Therapeutics, Inc. included in this Annual Report (Form 10-K) of Stemline Therapeutics, Inc. for the year ended December 31, 2017.

/s/ Ernst & Young LLP

Stamford, Connecticut
March 16, 2018

Exhibit 31.1

CERTIFICATION OF PERIODIC REPORT
PURSUANT TO SECTION 302 OF THE SARBANES-OXLEY ACT OF 2002

I, Ivan Bergstein, M.D., certify that:

1. I have reviewed this annual report on Form 10-K of Stemline Therapeutics, Inc.;

2. Based on my knowledge, this report does not contain any untrue statement of a material fact or omit to state a material fact necessary to make the statements made, in light of the circumstances under which such statements were made, not misleading with respect to the period covered by this report;

3. Based on my knowledge, the financial statements, and other financial information included in this report, fairly present in all material respects the financial condition, results of operations and cash flows of the registrant as of, and for, the periods presented in this report;

4. The registrant's other certifying officer(s) and I are responsible for establishing and maintaining disclosure controls and procedures (as defined in Exchange Act Rules 13a-15(e) and 15d-15(e)) and internal control over financial reporting (as defined in Exchange Act Rules 13a-15(f) and 15d-15(f)) for the registrant and have:

    a) Designed such disclosure controls and procedures, or caused such disclosure controls and procedures to be designed under our supervision, to ensure that material information relating to the registrant, including its consolidated subsidiaries, is made known to us by others within those entities, particularly during the period in which this report is being prepared;

    b) Designed such internal control over financial reporting, or caused such internal control over financial reporting to be designed under our supervision, to provide reasonable assurance regarding the reliability of financial reporting and the preparation of financial statements for external purposes in accordance with generally accepted accounting principles;

    c) Evaluated the effectiveness of the registrant's disclosure controls and procedures and presented in this report our conclusions about the effectiveness of the disclosure controls and procedures, as of the end of the period covered by this report based on such evaluation; and

    d) Disclosed in this report any change in the registrant's internal control over financial reporting that occurred during the registrant's most recent fiscal quarter (the registrant's fourth fiscal quarter in the case of an annual report) that has materially affected, or is reasonably likely to materially affect, the registrant's internal control over financial reporting; and

5. The registrant's other certifying officer and I have disclosed, based on our most recent evaluation of internal control over financial reporting, to the registrant's auditors and the audit committee of the registrant's board of directors (or persons performing the equivalent functions):

    a) All significant deficiencies and material weaknesses in the design or operation of internal control over financial reporting which are reasonably likely to adversely affect the registrant's ability to record, process, summarize and report financial information; and

    b) Any fraud, whether or not material, that involves management or other employees who have a significant role in the registrant's internal control over financial reporting.

Date: March 16, 2018

/s/ Ivan Bergstein, M.D.
Ivan Bergstein, M.D.
Chief Executive Officer
Principal Executive Officer

Exhibit 31.2

CERTIFICATION OF PERIODIC REPORT PURSUANT TO SECTION 302 OF THE SARBANES-OXLEY ACT OF 2002

I, David G. Gionco, certify that:

1. I have reviewed this annual report on Form 10-K of Stemline Therapeutics, Inc.;

2. Based on my knowledge, this report does not contain any untrue statement of a material fact or omit to state a material fact necessary to make the statements made, in light of the circumstances under which such statements were made, not misleading with respect to the period covered by this report;

3. Based on my knowledge, the financial statements, and other financial information included in this report, fairly present in all material respects the financial condition, results of operations and cash flows of the registrant as of, and for, the periods presented in this report;

4. The registrant's other certifying officer(s) and I are responsible for establishing and maintaining disclosure controls and procedures (as defined in Exchange Act Rules 13a-15(e) and 15d-15(e)) and internal control over financial reporting (as defined in Exchange Act Rules 13a-15(f) and 15d-15(f)) for the registrant and have:

    a) Designed such disclosure controls and procedures, or caused such disclosure controls and procedures to be designed under our supervision, to ensure that material information relating to the registrant, including its consolidated subsidiaries, is made known to us by others within those entities, particularly during the period in which this report is being prepared;

    b) Designed such internal control over financial reporting, or caused such internal control over financial reporting to be designed under our supervision, to provide reasonable assurance regarding the reliability of financial reporting and the preparation of financial statements for external purposes in accordance with generally accepted accounting principles;

    c) Evaluated the effectiveness of the registrant's disclosure controls and procedures and presented in this report our conclusions about the effectiveness of the disclosure controls and procedures, as of the end of the period covered by this report based on such evaluation; and

    d) Disclosed in this report any change in the registrant's internal control over financial reporting that occurred during the registrant's most recent fiscal quarter (the registrant's fourth fiscal quarter in the case of an annual report) that has materially affected, or is reasonably likely to materially affect, the registrant's internal control over financial reporting; and

5. The registrant's other certifying officer and I have disclosed, based on our most recent evaluation of internal control over financial reporting, to the registrant's auditors and the audit committee of the registrant's board of directors (or persons performing the equivalent functions):

    a) All significant deficiencies and material weaknesses in the design or operation of internal control over financial reporting which are reasonably likely to adversely affect the registrant's ability to record, process, summarize and report financial information; and

    b) Any fraud, whether or not material, that involves management or other employees who have a significant role in the registrant's internal control over financial reporting.

Date: March 16, 2018

/s/ David G. Gionco
David G. Gionco
Chief Accounting Officer
Principal Financial and Accounting Officer

Exhibit 32.1

STATEMENT OF CHIEF EXECUTIVE OFFICER OF
STEMLINE THERAPEUTICS, INC.
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 annual report of Stemline Therapeutics, Inc. (the "Company") on Form 10-K for the period ended December 31, 2017 as filed with the Securities and Exchange Commission (the "Report"), I, Ivan Bergstein, M.D., Chief Executive Officer of the Company, certify, pursuant to 18 U.S.C. §1350, as adopted pursuant to §906 of the Sarbanes-Oxley Act of 2002, that, based on my knowledge:

1) The Report fully complies with the requirements of Section 13(a) or 15(d) of the Securities Exchange Act of 1934, as amended; 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: March 16, 2018

/s/ Ivan Bergstein, M.D.
Ivan Bergstein, M.D.
Chief Executive Officer
Principal Executive Officer

Exhibit 32.2

STATEMENT OF CHIEF ACCOUNTING OFFICER OF
STEMLINE THERAPEUTICS, INC.
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 annual report of Stemline Therapeutics, Inc. (the "Company") on Form 10-K for the period ended December 31, 2017 as filed with the Securities and Exchange Commission (the "Report"), I, David G. Gionco, Chief Accounting Officer of the Company, certify, pursuant to 18 U.S.C. §1350, as adopted pursuant to §906 of the Sarbanes-Oxley Act of 2002, that, based on my knowledge:

1) The Report fully complies with the requirements of Section 13(a) or 15(d) of the Securities Exchange Act of 1934, as amended; 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: March 16, 2018

/s/ David G. Gionco
David G. Gionco
Chief Accounting Officer
Principal Financial and Accounting Officer