STEMLINE THERAPEUTICS INC
Form 10-K
March 31, 2014
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UNITED STATES

SECURITIES AND EXCHANGE COMMISSION

Washington, D.C. 20549

FORM 10-K

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

For the fiscal year ended December 31, 2013

OR

o 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) (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. o Yes x 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. o Yes x 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. xYes o No

Indicate by check mark where 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). xYes o 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. o Yes x No

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

Large-accelerated filer o	Accelerated filer o
Non-accelerated filer x (Do not check if a smaller reporting company)	Smaller reporting company o

Indicate by check mark whether the registrant is a shell company (as defined in Rule 12b-2 of the Act). o Yes x 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 $253,653,000 as of June 28, 2013, based on the closing sale price of such stock as reported on the NASDAQ Capital Market.

There were 13,199,254 shares of the registrant s common stock outstanding as of March 28, 2014.

DOCUMENTS INCORPORATED BY REFERENCE

Portions of the registrant s Proxy Statement for the 2014 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.

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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 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 ongoing and planned discovery and development of drugs targeting cancer stem cells, the strength and breadth of our intellectual property, our ongoing and planned preclinical studies and clinical trials, the timing of and our ability to make regulatory filings and obtain and maintain regulatory approvals for our product candidates, the degree of clinical utility of our product candidates, particularly in specific patient populations, expectations regarding clinical trial data, our results of operations, financial condition, liquidity, prospects, growth and strategies, the length of time that we will be able to continue to fund our operating expenses and capital expenditures, our expected financing needs and sources of financing, the industry in which we operate and the trends that may affect the industry or us.

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 preclinical studies and clinical trials, including patient accrual;
- our ability to obtain and maintain regulatory approval of our product candidates for trial initiation or marketing, and the labeling under any approval we may obtain;
- our plans to develop and commercialize our product candidates;
- 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;
- regulatory developments in the United States and foreign countries;
- the rate and degree of market acceptance of any of our product candidates;
- our available cash;
- 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-701 and our other in-licensed product candidates;

- the ability of our product candidates to successfully perform in clinical trials;

- the successful development of our sales and marketing capabilities;

- our ability to manufacture and the performance of third-party manufacturers, clinical research organizations, or CROs, 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 factors, we cannot assure you that the forward-looking statements in this Form 10-K will prove to be accurate.

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.

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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 commercializing proprietary therapeutics that target both cancer stem cells, or CSCs, and tumor bulk. We are currently developing two clinical-stage product candidates, SL-401 and SL-701. SL-401 is a targeted therapy directed to the interleukin-3 receptor, or IL-3R, present on CSCs and tumor bulk. To date, SL-401 has demonstrated single-agent activity, including durable complete responses, or CRs, in investigator sponsored Phase 1/2 trials in several indications including blastic plasmacytoid dendritic cell neoplasm, or BPDCN, and relapsed or refractory acute myeloid leukemia, or AML. We plan to advance SL-401 into corporate sponsored trials for multiple hematologic cancer indications including BPDCN, where we intend to pursue a Phase 2 registration-directed path. We also plan to initiate trials in additional rare IL-3R+ malignancies including mastocytosis, hypereosinophilic syndrome, other myeloproliferative syndromes, and hairy cell leukemia. These studies could be expanded to serve as platform trials for potential registration. We also intend to pursue larger indications including trials in multiple myeloma, or MM, and AML in first CR as consolidation therapy, and a Phase 3 trial in third-line AML for potential registration. SL-701 is a subcutaneously-administered therapeutic cancer vaccine comprised of multiple synthetic peptides. To date, the vaccine has demonstrated single-agent activity, including durable CRs and partial responses, or PRs, in investigator sponsored Phase 1/2 trials in advanced adult and pediatric brain cancers. We plan to advance SL-701 into a corporate sponsored Phase 2 trial in adult patients with recurrent glioblastoma multiforme, or GBM, following initial treatment with surgery, radiation, and chemotherapy. We believe that the design of this study may enable SL-701 to obtain accelerated regulatory approval and/or serve as the foundation for a subsequent Phase 3 pivotal trial in this indication. We also plan to pursue a Phase 2 trial of SL-701 in pediatric patients with brainstem and non-brainstem high-grade glioma, which are also areas of unmet medical need. In addition, we have built a robust preclinical pipeline which includes next generation IL-3R-targeted compounds, SL-501 and SL-101, an innovative discovery platform, and an extensive intellectual property portfolio including some of the earliest patents in the CSC area. We believe this establishes us as a leader in this rapidly emerging area of oncology.

The field of CSCs is an emerging area of cancer biology that we believe is fundamentally altering the approach to oncology drug development. CSCs have been identified in virtually all major tumor types, including leukemia and cancers of the brain, breast, colon, prostate and pancreas. CSCs are the highly malignant seeds of a tumor that self-renew and generate more mature cells that comprise the bulk of the tumor, or the tumor bulk. As such, we believe that CSCs are responsible for tumor initiation, propagation, and metastasis. Moreover, many of the characteristics of CSCs, such as their slow growth, presence of multi-drug resistance proteins, anti-cell death mechanisms, and increased activity of cellular mechanisms that repair damaged DNA, may enable CSCs to resist therapeutic agents traditionally used to treat cancer. Further, we believe there is now a significant body of evidence indicating that while standard therapies may initially shrink tumors by targeting the tumor bulk, their failure to effectively eradicate CSCs contributes to treatment failure, tumor relapse and poor survival. Accordingly, we believe that targeting both CSCs and the tumor bulk may represent a major advance in the fight against cancer. This premise has formed the basis of our drug development strategy, as illustrated below.

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Our Company

We were incorporated under the laws of the State of Delaware in August 2003. Our principal executive offices are 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 managing biopharmaceutical companies and in oncology drug development, including:

- Ivan Bergstein, M.D. Chairman, Chief Executive Officer and President. Dr. Bergstein is Chief Executive Officer and Founder of Stemline Therapeutics. He led Stemline through multiple rounds of private financing and ultimately its successful IPO and subsequent follow-on offering, raising over $100 million as a public company. Dr. Bergstein s early and broad intellectual property founded and positioned Stemline with a competitive edge and deep domain expertise in the rapidly emerging CSC field. 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. Previously, he was a senior biopharmaceuticals analyst at a Wall Street-based firm that advised mutual funds and hedge funds on investments in public companies with late clinical stage assets. He completed an internal medicine residency and hematology-oncology fellowship at the New York Presbyterian Hospital Weill Medical College of Cornell University.

- Eric K. Rowinsky, M.D. Executive Vice President, Chief Medical Officer and Head of Research and Development. Dr. Rowinsky was previously the Chief Medical Officer for ImClone Systems, Inc. Dr. Rowinsky has more than 25 years of experience managing clinical trials and developing drugs in oncology, including leading the Food and Drug Administration, or FDA, approval of Erbitux® for head and neck and colorectal cancers and advancing eight other biological therapeutics through clinical development while at ImClone. He has also played integral roles in the development and registration of a wide range of cancer therapeutics, including paclitaxel, docetaxel, irinotecan, topotecan, erlotinib, gefitinib, panitumumab, lapatinib, and temsirolimus, among others. Dr. Rowinsky currently serves on the Board of Directors of Biogen Idec Inc., as well as several other public biopharmaceutical companies, and is an Adjunct Professor at New York University School of Medicine. He completed a medical oncology fellowship at The Johns Hopkins Hospital. Dr. Rowinsky was an Associate Professor of Oncology at Johns Hopkins and then Head of Clinical Research and Director of the Institute for Drug Development of the Cancer Therapy and Research Center in San Antonio, Texas.

- Kenneth Hoberman Chief Operating Officer. Mr. Hoberman was previously Vice President of Corporate and Business Development of Keryx Biopharmaceuticals, Inc., where he was instrumental in securing 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 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

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also held senior financial positions at Progenics Pharmaceuticals, Inc. and Odyssey Pharmaceuticals, Inc. (a subsidiary of Pliva, Inc., now Teva Pharmaceutical Industries Ltd.). 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 maintain and fortify a leadership position in the discovery, acquisition and development of novel oncology therapies that target CSCs and to build a fully integrated pharmaceutical company with commercial infrastructure to support the marketing of our CSC-targeted oncology drugs, if approved. The fundamental components of our business strategy to achieve this goal include the following:

- *Develop and commercialize SL-401 in multiple hematological cancers.* We plan to advance SL-401 into corporate sponsored trials for multiple hematologic cancer indications including blastic plasmacytoid dendritic cell neoplasm, or BPDCN, where we intend to pursue a Phase 2 registration-directed path. We also plan to initiate trials in additional rare IL-3R+ malignancies including mastocytosis, hypereosinophilic syndrome, other myeloproliferative syndromes, and hairy cell leukemia. These studies could be expanded to serve as platform trials for potential registration. We also intend to pursue larger indications including trials in myeloma myeloma, or MM, and acute myeloid, or AML, in first CR as consolidation therapy, and a Phase 3 trial in third-line AML for potential registration.

- *Develop and commercialize SL-701 in brain cancer.* We plan to advance SL-701 into a corporate sponsored Phase 2 trial in adult patients with recurrent glioblastoma multiforme, or GBM, following initial treatment with surgery, radiation, and chemotherapy. We believe that the design of this study may enable SL-701 to obtain accelerated regulatory approval and/or serve as the foundation for a subsequent Phase 3 pivotal trial in this indication. We also plan to pursue a Phase 2 trial of SL-701 in pediatric patients with brainstem and non-brainstem high-grade glioma, which are also areas of unmet medical need.

- *Continue to advance and build out our pipeline.* We also plan to advance and build out our pipeline of product candidates. In particular, we plan to advance SL-501 and SL-101, our next generation IL-3R-targeted compounds, into IND-enabling studies.

- *Leverage our proprietary drug discovery platform, StemScreen®, to identify new therapeutic candidates.* We intend to utilize our proprietary discovery platform, StemScreen®, to continue to identify new CSC-targeted drug candidates. We may conduct some of these efforts internally and/or leverage our platform to engage in strategic collaborations.

- *Develop commercialization capabilities in North America and Europe.* We believe that the infrastructure required to commercialize our oncology product candidates may make it cost-effective for us to internally develop a marketing effort and sales force. If SL-401, SL-701 or any of our other product candidates is approved by the FDA or other regulatory authorities, we intend to commercialize our product candidates in North America, and potentially in Europe through direct sales and distribution. However, we will remain opportunistic in seeking strategic partnerships in these and other markets when advantageous to us.

- *Continue to both leverage and fortify our intellectual property portfolio.* We believe that we have a very strong intellectual property position relating to the development and commercialization of our product candidates and technology and CSC-targeting in general. We plan to continue to leverage this portfolio to create value. In addition to fortifying our existing intellectual property position, we intend to file new patent applications, and we may in-license new intellectual property and take other steps to strengthen, leverage, and expand our intellectual property position.

SL-401 A targeted therapy directed to IL-3R on CSCs and tumor bulk

Overview

SL-401 is a clinically active targeted therapy directed to the interleukin-3 receptor, or IL-3R. IL-3R is overexpressed on CSCs and/or more mature cancer cells derived from CSCs (i.e., tumor bulk) of multiple hematologic cancers including acute myeloid leukemia, or AML, chronic myeloid leukemia, or CML, myelodysplastic syndrome, or MDS, certain lymphomas including Hodgkin s disease, multiple myeloma, or MM, and multiple rare hematologic malignancies such as blastic plasmacytoid dendritic cell neoplasm, or

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BPDCN, and others. In a completed investigator sponsored Phase 1/2 clinical trial in patients with advanced hematologic cancers, single agent SL-401 administered in a single cycle regimen demonstrated anti-tumor activity, including durable CRs, in relapsed or refractory patients. Specifically, a single cycle of single-agent SL-401 induced seven CRs: five CRs in BPDCN and two CRs in relapsed or refractory AML. Notably, SL-401 also improved the median overall survival, or OS, relative to historical data, of the 16 most heavily pretreated AML patients who had failed at least two previous therapies (i.e., third-line or greater) and who received therapeutically relevant doses of SL-401, with only a single cycle. Further, SL-401 has not demonstrated the protracted myelosuppression typically seen with traditional chemotherapy, which is a key differentiating feature relative to many other hematologic cancer therapies and which we believe is due to the lack of IL-3R expression on normal hematopoietic stem cells. Currently, there are limited effective treatment options for patients with relapsed or refractory hematologic cancers including BPDCN and AML. We believe that a major reason for the failures of traditional treatments to provide long-term benefit is that these traditional treatments target tumor bulk rather than both tumor bulk and CSCs, and are often toxic to the bone marrow. Accordingly, by pursuing hematologic cancer indications with SL-401, a therapeutic that uniquely targets both CSCs and tumor bulk and does not induce the protracted myelosuppression associated with standard therapy, we intend to provide benefit to patients who historically have been difficult to treat with traditional therapies.

We plan to advance SL-401 into corporate sponsored trials for multiple hematologic cancer indications including BPDCN and additional rare malignancies, as well as larger indications including AML and MM. In preparation for these studies, we have completed commercial-scale cGMP production of active pharmaceutical ingredient, or API. Upon completion of drug product formulation and fill-finish, which is currently underway, we plan to submit a corporate sponsored investigational new drug, or IND, application, and initiate trials in multiple indications including BPDCN, where we intend to pursue a Phase 2 registration-directed path. We also plan to initiate trials in additional rare IL-3R+ malignancies including mastocytosis, hypereosinophilic syndrome, other myeloproliferative syndromes, and hairy cell leukemia. These studies could be expanded to serve as platform trials for potential registration. We also intend to pursue larger indications including trials in patients with relapsed or refractory MM and AML in first CR as consolidation therapy, as well as a randomized Phase 3 trial in third-line AML for potential registration. Accordingly, we believe that SL-401 may represent a significant market opportunity.

In June 2013, SL-401 was awarded Orphan Drug designation from the FDA for the treatment of BPDCN. Previously, in February 2011, SL-401 was awarded Orphan Drug designation from the FDA for the treatment of AML.

Blastic plasmacytoid dendritic cell neoplasm (BPDCN)

BPDCN is a rare and 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 most commonly affects middle-aged and older patients and is approximately three times more common in men than women. This malignancy has features of both lymphomas, including cutaneous lymphomas, as well as leukemias, and typically presents with skin lesions, as well as extracutaneous disease that may include the bone marrow, blood, lymph nodes, and spleen. BPDCN growth in the bone marrow results in decreased blood cell counts, which can lead to serious infections, fatigue, bleeding, and death. Although BPDCN can be controlled for brief periods with various combination chemotherapy regimens, including high dose chemotherapy with allogeneic stem cell transplantation, overall prognosis remains poor. There are currently no approved therapies for BPDCN, and an optimal therapeutic regimen for BPDCN has not yet been established.

Other rare IL-3R cancers

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

Hairy cell leukemia. Hairy cell leukemia, or HCL, is an uncommon hematological malignancy characterized by a clonal accumulation of abnormal B lymphocytes. Approximately 2,000 new cases of HCL occur annually in the United States. The median age at diagnosis is approximately 62 years with male predominance. Although the 6-year overall survival rate has been estimated to be approximately 80% and there are FDA approved therapies for HCL, including cladribine (Litak® and Movectro®) and pentostatin (Nipant®), there is no permanent cure for the disease.

Mastocytosis. Systemic mastocytosis is a proliferative disorder characterized by an overabundance of mast cells in various organs and tissues. Mastocytosis can be systemic or localized to one or a few organs. The WHO classifies mastocytosis into the following categories: cutaneous, indolent, systemic (with associated hematologic non-mast cell lineage disease), aggressive systemic, mast cell leukemia, mast cell sarcoma, and extracutaneous astrocytoma. There are approximately 3,000 cases of mastocytosis diagnosed annually in the United States. Patients with indolent disease typically have a favorable prognosis, whereas aggressive cases of

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mastocytosis carry an overall survival of under 3.5 years. There are no currently approved drugs and no cure for mastocytosis. Treatment for aggressive variants includes various chemotherapy agents, imatinib (Gleevec®), corticosteroids, and antihistamines.

Eosinophilic disorders. Primary eosinophilic disorders include chronic eosinophilic leukemia, or CEL, idiopathic hypereosinophilic syndrome, or HES, lymphocyte-variant HES, and primary eosinophilia associated with an 8p11 chromosomal translocation. These rare disorders are characterized by a persistently elevated eosinophil count that may result in various symptoms depending on which organs are involved. Damage to the heart, lungs, peripheral nervous system, and other organs can occur. An acquired (non-familial) form of HES is particularly aggressive and debilitating. Acquired forms of HES are subclassified as secondary (reactive), idiopathic, and clonal HES, the latter often transitioning into CEL, which can result in myocardial fibrosis and congestive heart failure. Eosinophils are known to ubiquitously express the IL-3R. Current treatments for CEL include corticosteroids, mepolizumab, alemtuzumab (Campath®), and imatinib (Gleevec®), the latter of which is approved by the FDA for a very small proportion of HES patients who express the FIP1L1-PDGFRA fusion protein. However, some of these agents can cause severe toxicity and may not induce durable responses. Therefore, newer and more effective therapies are needed for certain patients, including those with symptomatic disease and/or extra-cutaneous organ involvement.

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. The yearly calculated incidence of PMF in the U.S. ranges from approximately 1,260 to 4,410 individuals per year. Median age at diagnosis is 65 years. About 20% of affected patients are less than 55 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®), which has recently received regulatory approval in the United States and elsewhere for the treatment for PMFs, 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, though peripheral neuropathy can be a troublesome side effect.

Chronic myelomonocytic leukemia. Chronic myelomonocytic leukemia, or 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 a myelodysplastic syndrome, or MDS, as well as a myeloproliferative disorder, or MPD. In the United States, the incidence of CMML is approximately less than 3,150 individuals per year and the disease affects approximately 9,450 individuals per year. 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 acute myeloid leukemia, or AML, in about 20%-30% 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®) have been used to treat CMML. High dose chemotherapy followed by bone marrow transplantation is also employed to treat CMML, and may provide long term benefit.

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 19,000 new AML cases occur annually in the United States, and approximately 27,000 new cases occur annually in Europe. The average age of an AML patient is 67 years. The National Cancer Institute estimated that the one-year survival rate for adult patients with AML was approximately 34%. The one-year survival rate for AML after first relapse is approximately 20%, and after second relapse is approximately 8%. The median OS for AML patients after failing second-line treatment, based on two large series, is 1.5 months. 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, while there are currently no approved treatments, 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, the high relapse rate and poor OS indicate that most patients harbor drug-resistant CSCs following chemotherapy. In third-line AML, there are currently no approved treatments, and these

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patients frequently have depressed bone marrow function and are often no longer optimal candidates for additional chemotherapy. As such, third-line AML constitutes an unmet medical need.

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 22,000 new cases of MM are reported annually in the United States and approximately 33,000 new MM cases are reported annually in Europe. The median age at diagnosis is approximately 62 years for men and 61 years for women. The median overall survival after conventional treatments is 3-4 years, but high-dose treatment followed by autologous stem cell transplantation can extend the median survival to 5-7 years. Despite FDA approved therapies for MM, including thalidomide (Thalomid®), lenalidomide (Revlimid®), bortezomib (Velcade®), dexamethasone (Decadron®), carfilzomib (Krypolis®), and pomalidomide (Pomalyst®), most patients invariably relapse from the disease.

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 15,000 to 25,000 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 55% for low-risk patients, to 7% to 35% 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. Annually, approximately 5,000 new cases are reported in the United States each year and approximately 4,000 to 9,000 new cases are reported each year in Europe. The five-year OS rate for CML patients is 57%. When CML advances to an accelerated or blastic phase, the median OS is less than 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 three TKIs: imatinib (Gleevec®), nilotinib (Tasigna®) and dasatinib (Sprycel®). 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 9,000 new HL cases occur annually in the United States and approximately 12,000-17,000 cases occur annually in Europe. 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 affective 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 a complete remission, or CR, prior to transplantation, or who relapse after second line therapy, have few effective therapeutic options. Recently, brentuximab vedotin (Adcentris®) received regulatory approval in the United States and elsewhere for the treatment of recurrent or refractory HL.

Design of SL-401 and mechanism of action

SL-401 is a biologic targeted therapy directed to the IL-3R. SL-401 consists of IL-3 recombinantly linked to a truncated diphtheria toxin payload. Mechanistically, the IL-3 domain of SL-401 directs the cytotoxic payload to IL-3R+ cells. SL-401 is then internalized by target cells, leading to intracellular release of the payload, inhibition of protein synthesis and cell death, or apoptosis. Accordingly, the targeting and mechanism by which SL-401 kills cells differs from therapeutics that are commonly used to treat hematologic malignancies. Traditional therapies, such as chemotherapy, largely target rapidly dividing cells, whether malignant or normal, by

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interfering with DNA replication and other processes. SL-401, in contrast, is a targeted therapy that specifically recognizes and binds to cells expressing IL-3R, a target which is overexpressed on leukemia cells relative to normal cells. Thus, SL-401 preferentially targets malignant, not normal cells, a feature expected to result in fewer toxicities relative to traditional therapies. Moreover, by inhibiting protein synthesis, we believe that SL-401 is able to kill not just rapidly dividing cells, but also slower-growing cells such as CSCs. In addition, the SL-401 payload does not appear to be subject to multi-drug resistance proteins highly expressed on CSCs and tumor bulk. Therefore, unlike traditional therapies which largely target and kill tumor bulk only, SL-401 is designed to target and kill both CSCs and tumor bulk.

IL-3R is normally expressed on certain maturing hematopoietic cells, including maturing myeloid cells, B cells, dendritic cells, mast cells, basophils and eosinophils, and appears to be involved in cell maturation, differentiation, and survival. Importantly, IL-3R is not expressed to a significant degree on normal hematopoietic stem cells. IL-3R is, however, overexpressed on multiple hematological malignancies including AML, BPDCN, MDS, CML, B cell acute lymphoid leukemia, Hodgkin s and certain aggressive non-Hodgkin s lymphomas, hairy cell leukemia, and rare malignancies and myeloproliferative disorders involving mast cell, basophilic and eosinophilic lineages including mastocytosis and hypereosinophilic syndrome. In addition to expression on tumor bulk, IL-3R is also expressed on the CSCs of multiple hematologic cancers including AML, CML, MDS, and T-cell acute lymphoid leukemia. Elevated IL-3R expression has been correlated with poor patient prognosis. For example, as described by Vergez in Haematologica in 2011, a higher percentage of IL-3R-expressing, or IL-3R+, CSCs within a patient s entire tumor correlates with poor outcome. In particular, AML patients with IL-3R+ CSCs that comprise greater than or equal to 1% of their entire leukemia were found to have a worse prognosis than patients with IL-3R+ CSCs that comprise less than 1% of their entire leukemia. We believe that these findings further validate that IL-3R is an important oncology target.

SL-401 preclinical activity and safety

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 IC_{50}, 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 very high potency against BPDCN cells from patients, with an IC_{50} in the femtomolar (10^{-15} molar) range. SL-401 has also demonstrated preclinical activity against a variety of additional hematologic cancers including certain rare IL-3R+ malignancies such as chronic eosinophilic leukemia, where it produced IC_{50} 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 potent *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. Interestingly, 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 dendritic cells (the cells of origin of BPDCN) that appear to provide a microenvironmental 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 the MM and BPDCN diseases via a common plasmacytoid dendritic cell, and IL-3R, target. SL-401 has also been shown to have a synergistic effect against MM when combined with existing therapies including lenalidomide (Revlimid®) and bortezomib (Velcade®).

Phase 1/2 clinical trial advanced hematologic cancers

Overview

SL-401 demonstrated single agent clinical efficacy, including durable CRs, in a multi-center investigator sponsored Phase 1/2 clinical trial of patients with advanced hematologic cancers, which we refer to as the 401-AHC Study. Specifically, an interim analysis of this trial presented at the annual meeting of the American Society of Hematology (ASH) in December 2013 show that a single cycle of single agent SL-401 induced seven CRs: five CRs in BPDCN and two CRs in relapsed or refractory AML. Although the study was designed so that all patients received only a single cycle of SL-401 treatment, the median OS, relative to historical data, was improved in the 16 most heavily pretreated AML patients who had failed at least two previous therapies (i.e., third-line or greater) and were treated with therapeutically relevant doses, with only a single cycle. Of note, we intend to administer multiple cycles of SL-401 in our future trials, which we believe may increase the efficacy with respect to both clinical response and survival. Further, SL-401 has not resulted in the protracted hematologic toxicity associated with traditional chemotherapy, which we believe is a key differentiating feature relative to other hematologic cancer therapies.

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This 401-AHC Study was undertaken in 84 patients with advanced hematologic cancers, including relapsed or refractory AML patients (n=59), AML patients who were poor risk and not candidates for chemotherapy (n=11), high risk MDS patients (n=7), or patients with blastic plasmacytoid dendritic cell neoplasm (BPDCN) (n=7, with n representing the number of patients). The median patient age was 65 years, with a range of seven to 84 years of age. Patients received a single cycle of single-agent SL-401 of doses ranging from 4.0 to 22.1 µg/kg/day, consisting of a 15-minute intravenous infusion on either an every-other-day schedule for up to six treatments, or daily for a five-day schedule. Participating sites in the 401-AHC Study were MD Anderson Cancer Center (Houston, TX), Duke University (Durham, NC), the Scott and White Cancer Research Institute/Texas A&M (Temple, TX), the University of Texas Southwestern (Dallas, TX), and the British Columbia Cancer Agency (Vancouver, Canada). Results from the 401-AHC Study, which are set forth below, were presented at the annual meetings of the American Society of Clinical Oncology (ASCO) in June 2013 and ASH in December 2013.

Well-tolerated at clinically active doses

SL-401 was well-tolerated at clinically active doses and had a generally acceptable side effect profile relative to other agents used for advanced hematologic cancers. This included largely transient transaminitis, hypoalbuminemia, edema, thrombocytopenia, fever and chills. This is largely similar to that reported with denileukin diftitox (Ontak®), a compound comprised of human interleukin-2 linked to a truncated diphtheria toxin payload, which is FDA approved and has been marketed for certain forms of cutaneous T-cell lymphoma for over a decade. Of note, many of the side effects of Ontak® have been reported to decrease with each successive cycle administered. Importantly, however, the anticancer activity of Ontak® appears to be maintained, and even augmented, with each successive cycle. In particular, patients who do not respond to an initial cycle have been shown capable of responding to later cycles, and patients who partially responded to an initial or early cycle have also been shown capable of converting to complete responders in subsequent cycles. Ontak® is approved on a daily-for-five-days schedule for eight cycles.

The maximum tolerated dose, or MTD, of SL-401 was 16.6 µg/kg/day for five consecutive days, with tolerable and active doses at 16.6 µg/kg/day as well as 12.5, 9.4, and 7.1 µg/kg/day.

Anti-tumor activity

In the 401-AHC Study, to date, one cycle of SL-401 has demonstrated robust single agent activity, including an 86% overall response rate, or ORR, including 5 CRs, in BPDCN. Additionally, SL-401 reduced leukemia blast counts in the bone marrow (i.e., reduced tumor bulk) or stabilized disease, in approximately half of all treated patients, the majority of whom were heavily pretreated, as summarized below. More specifically, tumor shrinkages or disease stabilizations were seen in 46% of patients with relapsed or refractory AML, 55% of AML who were poor risk and thus not candidates for chemotherapy, 43% of high-risk MDS patients and 86% of BPDCN patients. There were also multiple additional cases of tumor shrinkages in response to a single cycle of SL-401 treatment. Durable CRs were induced in two patients with relapsed or refractory AML. Five additional CRs and one PR occurred after a single cycle of SL-401 in seven patients with BPDCN.

Activity of a single-cycle of SL-401

in patients with advanced hematological cancers

	BPDCN (n=7)	AML (relapsed refractory) (n=59)	AML (≥ 3rd line) (n=35*)	AML (not chemo candidate) (n=11)	MDS (high risk) (n=7)
Tumor shrinkages/disease stabilization	86%	46%	43%	55%	43%
Tumor shrinkages	86%	25%	23%	27%	29%
	5CRs	2CRs	1CR		

AML, Acute myeloid leukemia; MDS, Myelodysplastic syndrome; BPDCN, Blastic plasmacytoid dendritic cell neoplasm; chemo, Chemotherapy; CR, Complete response

* Subpopulation of relapsed, refractory

A single cycle of SL-401 administered as a single agent induced major objective anti-tumor responses in 6 of 7 (86%) patients with BPDCN, including 5 CRs and 1 PR. Four of the five CRs had durations of at least 3 months, with 2 CR patients still in remission at 15+ and 5+ months. In addition, a single cycle of SL-401 as a single agent induced durable CRs in advanced AML patients. This included a CR of 25+ months duration in a fourth-line AML patient who had failed three previous treatment regimens, including two treatments with high-dose therapy followed by allogeneic stem cell transplantations, prior to entry into the 401-AHC Study. In addition, a patient with AML that was refractory to standard induction chemotherapy experienced a CR lasting 8 months following treatment with a single cycle of SL-401.

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Anti-CSC effect

SL-401 was shown to have potential dual activity not only against tumor bulk (as evidenced by tumor shrinkages and stabilizations) but also against CSCs. Bone marrow samples were collected from three patients both before (day 0) and after SL-401 treatment (day 15 and 30) and were tested for CSC activity in an *ex vivo* colony formation assay (an assay that measures the ability of CSCs to form colonies). As demonstrated by Konopleva in *Blood* in 2010, and as illustrated below, a considerable decrease in CSC activity was noted at 15 and 30 days after patients were treated with SL-401. At 30 days post-treatment, CSC activity decreased by an average of 79% of that measured at pretreatment, suggesting a clinical anti-CSC effect. We believe that these studies also provide preliminary evidence that the beneficial clinical effects noted in some patients in the 401-AHC Study may have been due, in part, to the anti-CSC activity of SL-401. In particular, reductions in leukemic CSC activity 30 days post-treatment of 79% and 84% were observed in two patients, both of whom notably outlived the historical median OS of heavily pretreated AML patients, with overall survival values of 7.2 months and 13.6 months, respectively. We intend to follow-up on these positive preliminarily provocative data in future clinical trials.

SL-401 demonstrates clinical anti-CSC effect

(adapted from Konopleva et al. Blood 2010; 116:21: Abstract #3298)

Survival signal

In the 401-AHC Study, the median overall survival, or OS was 5.6 months (95% CI: 2.5, 10.8 months) in the 16 most heavily pretreated AML patients treated with therapeutically relevant doses of single cycle SL-401, which is an improvement of several months over historical data reported by Giles et al. in *Cancer* in 2005.

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Overall survival signal in AML patients (≥3rd line) treated with only a *single cycle* of SL-401 (at therapeutically relevant doses*; n = 16 patients)

(Konopleva et al. American Society of Hematology 2012 Abstract #3625)

*Patients received the MTD (16.6 µg/kg/d) or one or two doses below the MTD (9.4 and 12.5 µg/kg/d)

Notably, these results derive from the 401-AHC Study wherein only a single cycle regimen of SL-401 was utilized. We believe that a multiple-cycle administration of SL-401 will further increase the clinical benefit of SL-401. Accordingly, to maximize the potential benefits of SL-401, we plan to administer multiple cycles of SL-401 in all of our planned clinical trials of SL-401.

Clinical and regulatory strategy for SL-401

We plan to advance SL-401 into corporate sponsored trials for multiple hematologic cancer indications including blastic plasmacytoid dendritic cell neoplasm, or BPDCN, and additional rare IL-3R+ malignancies, as well as larger indications including acute myeloid leukemia, or AML

and multiple myeloma, or MM. In preparation for these studies, we have completed commercial-scale cGMP production of API. Upon completion of drug product formulation and fill-finish, which is currently underway, we plan to submit a corporate-sponsored IND application, and initiate trials in multiple indications including BPDCN, where we intend to pursue a Phase 2 registration-directed path. We also plan to initiate trials in additional rare IL-3R+ malignancies including mastocytosis, hypereosinophilic syndrome, other myeloproliferative disorders, and hairy cell leukemia. If the results of these studies are notable, Stemline expects to meet with the appropriate regulatory agencies to define pathways leading to potential registration. We also intend to pursue larger indications including trials in patients with relapsed or refractory MM and AML in first CR as consolidation therapy, as well as a randomized Phase 3 trial in third-line AML for potential registration. We may also pursue investigator sponsored studies in additional indications which may include chronic myeloid leukemia, or CML, and certain lymphomas, including Hodgkin s lymphoma.

SL-701 a subcutaneously-administered cancer vaccine comprised of synthetic peptides

Overview

SL-701, a clinically active therapeutic cancer vaccine comprised of multiple synthetic peptides, is designed to direct the immune system to attack targets present on the CSCs and tumor bulk of brain cancer. High-grade gliomas, or HGGs, are the most aggressive

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brain cancers and have a poor prognosis. Treatment options are limited, particularly for adult patients with recurrent or refractory HGG, including glioblastoma multiforme, or GBM, and pediatric patients with HGG, including brainstem glioma, or BSG, and non-brainstem glioma, indications where SL-701 has shown activity. In two completed investigator sponsored Phase 1/2 clinical trials conducted at the University of Pittsburgh School of Medicine, the vaccine, now being developed by Stemline as SL-701, demonstrated single agent anti-tumor activity, including CRs, which is uncommon for a cancer vaccine, particularly in these indications. Tumor shrinkage or stabilization was noted in 59% (13/22) of HLA-A2+ (as defined below) adult patients with recurrent or refractory HGG (the 701-Adult-RHGG Study), and 87% (26/30) of HLA-A2+ pediatric glioma patients (the 701-Ped-G Study). To date, there have been eight major objective tumor responses (i.e., tumor regressions) in these two studies, consisting of two CRs and six PRs. SL-701 has also induced an additional tumor shrinkage, in the form of a minor response, or MR, a prolonged disease free survival following complete surgical resection (i.e. a clinical complete response, or CCR).

We plan to advance SL-701 into a corporate sponsored Phase 2 trial in adults, as well as a cooperative group led study in children. In preparation for these studies, we have completed commercial-scale cGMP production of API, as well as drug product formulation and fill-finish. Upon submission and acceptance by the FDA of a corporate-sponsored IND, we expect to initiate a Phase 2 trial of SL-701 in adult patients with recurrent GBM, following initial treatment with surgery, radiation, and chemotherapy. We believe that the design of this study may enable SL-701 to obtain accelerated regulatory approval and/or serve as the foundation for a subsequent Phase 3 pivotal trial in this indication. We also plan to pursue a Phase 2 trial of SL-701 in pediatric patients with brainstem and non-brainstem high-grade glioma, which are also areas of unmet medical need.

High-grade glioma (including adult glioblastoma and pediatric non-brainstem and brainstem glioma)

Gliomas are histologically heterogeneous tumors that are derived from glial cells in the brain. Gliomas are graded from 1 to 4, based on WHO classifications, with grade 4 glioma (i.e., glioblastoma, or GBM) and grade 3 glioma (i.e., anaplastic glioma, or AG) as the most aggressive gliomas and referred to as high-grade gliomas, or HGGs. GBM makes up the majority of HGG cases, with an annual incidence in adults of approximately 10,000 in the United States and 15,000 to 18,000 in Europe.

The standard of care for newly diagnosed adult GBM is resection, if operable, followed by a combination of radiation and temozolomide (i.e., the Stupp regimen). Although this combination treatment has improved patient outcomes, 85% to 90% of patients ultimately relapse, with a median OS from diagnosis of 15 months. Bevacizumab (Avastin®) received accelerated, but not full, approval for adults with recurrent or refractory adult GBM based, in part, on a response rate endpoint. However, most recurrent patients receiving bevacizumab (Avastin®) do not have durable clinical benefit, and the median OS for these second-line patients is approximately eight to nine months. Currently, no therapies have been approved for GBM patients who fail bevacizumab, which carries a median OS of three to four months.

Pediatric HGG, which includes non-brainstem HGG and brainstem glioma, or BSG, is a highly malignant disease with very poor outcomes. The annual incidence of pediatric HGG is approximately 1,600 to 2,000 in the United States and approximately 3,400 in Europe. No therapy has been shown to have a favorable outcome in this population and almost all patients relapse after receiving first-line treatment. Pediatric patients with newly diagnosed HGG are typically treated with surgery, chemotherapy and/or radiation and have an expected median OS from diagnosis of less than one year.

Design of SL-701 and mechanism of action

SL-701 is a therapeutic cancer vaccine comprised of several short synthetic peptides that correspond to epitopes of targets including IL-13R 2, EphA2, and survivin, present on the tumor bulk and/or CSCs of brain cancer. The synthetic peptides that correspond to IL-13R 2 and survivin are novel artificially constructed mutants designed to be immunogenic to amplify the vaccine s clinical anti-tumor immune response.

SL-701, like other cancer vaccines, is combined with additional elements designed to promote an immune response, such as a helper peptide and an adjuvant. A helper peptide helps activate cytotoxic T-cells, and is mixed with SL-701 prior to administration. An adjuvant similarly helps stimulate the immune system, and is administered to the patient concurrently with vaccine administration. Whereas the previous studies have used poly-ICLC as an adjuvant, we plan to use granulocyte-colony-stimulating factor, or GM-CSF, and imiquimod, a toll-like receptor 7, or TLR7, agonist, which we believe are commercially viable and state-of-the-art adjuvants.

Phase 1/2 clinical trial adult recurrent or refractory high-grade glioma

In an investigator sponsored Phase 1/2 clinical trial, the vaccine, now being developed by Stemline as SL-701, was evaluated in adult patients with recurrent or refractory HGG. This study, which we refer to as the 701-Adult-RHGG Study, enrolled 22 HLA-A2+ adult patients with recurrent or refractory HGG, 13 of which had refractory or recurrent GBM, and nine of which had anaplastic glioma, or AG. 50% of patients were second relapse or greater and two of the refractory or recurrent GBM patients had received prior treatment

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with bevacizumab (Avastin®). The vaccine was loaded *ex vivo* onto dendritic cells that had been removed from the patient, which were then re-injected intra/peri-nodally back into the patient with a separate concurrent injection of an adjuvant. This delivery method contrasts with that used in the 701-Ped-G Study and 701-Adult-LGG Study, in which the vaccine was administered to patients and demonstrated activity as a direct subcutaneous injection. The 701-Adult-RHGG Study was a single-arm trial whose objectives were to determine the general safety, dosage and efficacy of the vaccine.

Well-tolerated at clinically active doses

The vaccine was well-tolerated at clinically active doses in the 701-Adult-RHGG Study. Injection site reactions were the most common adverse events and generally resolved within 24 hours. These side effects do not overlap with those of radiation, chemotherapy agents, and anti-angiogenic agents like bevacizumab (Avastin®), which are mainstay therapies used to treat adult HGG. We believe this implies that the development of SL-701-based combination regimens may be feasible.

Clinical activity

In the 701-Adult-RHGG Study, the vaccine demonstrated single agent clinical activity. Forty-six percent (6/13) of refractory or recurrent GBM and 78% (7/9) of recurrent AG patients sustained an anti-tumor response or disease stabilization. This included two durable CRs, one of which occurred in a 62-year-old male GBM patient who was refractory to prior surgical resection, radiation therapy and temozolomide. Following vaccine treatment, this patient s gadolinium enhanced tumor mass disappeared, and the patient was determined to have sustained a durable CR that exceeded 23 months. Notably, in this patient there was also a significant increase in target-specific T-cells by week 29 as determined by a tetramer assay, consistent with a positive immune response to the vaccine. A recurrent AG patient with anaplastic oligoastrocytoma sustained a CR that exceeded nine months. In addition to the two durable CRs, there were also three PRs. One PR was sustained by a patient with recurrent GBM (second salvage, i.e., third-line) and lasted seven months. Notably, a post-vaccine brain biopsy from this PR patient demonstrated the presence of macrophages and CD8+ T lymphocytes, which are cells of the immune system, within the tumor. We believe this indicates that the vaccine induced the immune system, and cytotoxic T-cells in particular, to migrate to the area of the brain tumor and induce tumor shrinkage by targeting specific antigen-bearing CSCs and tumor bulk. This activity is consistent with the proposed mechanism of action of the vaccine wherein it induces the immune system, and cytotoxic T cell in particular, to cross the blood-brain barrier and attack the antigen expressing tumor. A second PR was sustained by a patient with recurrent GBM whose PR exceeded 11 months in duration. The third PR was seen in a recurrent AG patient.

Eighty-one percent (13/16) of evaluable patients had at least one positive immunological assay. We believe this indicates that the vaccine stimulated the immune system in a highly specific fashion.

Survival signal

The vaccine improved the median, six-month, and 12-month OS of adult patients with refractory or recurrent GBM as well as recurrent AG, compared with historical data. In refractory or recurrent GBM patients treated with vaccine, median OS was 13 months, six-month OS was 80%, and 12-month OS was 55%, as illustrated in the figure below. These rates represent improvements over the historical median OS of five to seven months, the historical six-month OS of 38% to 55%, and the historical 12-month OS of 14% to 25%. Recurrent AG patients treated with vaccine

also experienced an improvement in OS compared with historical results.

Kaplan-Meier survival curve
of recurrent or refractory adult HGG patients treated with vaccine
(Okada et al., Journal of Clinical Oncology 2011; 29:330-336)

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Phase 1/2 clinical trial pediatric glioma

In a completed investigator sponsored Phase 1/2 trial, the vaccine was evaluated in pediatric patients with glioma. This study, which we refer to as the 701-Ped-G-Study, was undertaken in 30 HLA-A2+ pediatric patients with glioma. Twenty of these patients had newly diagnosed brainstem glioma, or BSG, four had newly diagnosed non-brainstem HGG, three had recurrent non-brainstem HGG and three had multiply recurrent low-grade glioma, or LGG. Patients received a direct subcutaneous injection of the vaccine (without dendritic cells) in the right or left upper arms associated with intact draining auxiliary lymph nodes once every three weeks with a separate concurrent injection of an adjuvant. This 701-Ped-G Study was a single-arm trial whose objectives were to determine the general safety, dosage and efficacy of the vaccine.

Well-tolerated at clinically active doses

The vaccine was well-tolerated at clinically active doses in this 701-Ped-G Study. Adverse effects included local injection site reactions and low grade fever in almost all patients, which were generally mild and controlled with analgesics.

Clinical activity

In this 701-Ped-G Study, the vaccine demonstrated single agent clinical activity. Eighty seven percent (26/30) of evaluable patients sustained durable tumor reductions or disease stabilizations, including three patients who experienced durable PRs. One of these PR patients is a child with newly diagnosed BSG whose PR demonstrated greater than 50% tumor shrinkage and was 15 months in duration. The second PR occurred in a child with newly diagnosed non-brainstem HGG and was 14 months in duration. The third PR occurred in a child with multiply recurrent LGG and was nine months in duration. Also, a MR was induced in a pediatric patient with non-brainstem HGG. An additional child with newly diagnosed non-brainstem HGG had prolonged disease-free status of 20 months following surgery. In addition, there were four stable disease patients who survived at least 13 months.

In five cases, tumor pseudoprogression was seen. Tumor pseudoprogression is believed to represent a positive sign, or surrogate marker, of anti-tumor activity. Tumor pseudoprogression is manifested by edema and contrast enhancement on MRI and can transiently mimic tumor progression prior to regression and thus must be carefully monitored. Pseudoprogression has been noted with the introduction of effective treatments for brain tumors, such as stereotactic radiotherapy, which have led to tumor responses. Notably, the PR patient whose response lasted 15 months is believed to have experienced tumor pseudoprogression prior to the PR**.**

Positive immunological assays (both ELISPOT and tetramer assays) were demonstrated in six of seven evaluable children, including the newly diagnosed BSG pediatric patient who sustained a durable PR that lasted 15 months. We believe that these data indicate that vaccine treatment stimulated the immune system in a highly specific fashion.

Low-grade glioma trial in adult patients

An investigator sponsored study of the vaccine was also conducted at the University of Pittsburgh School of Medicine in adult patients with LGG, which we refer to as the 701-Adult-LGG Study. Twenty-three HLA-A2+ patients have been enrolled, including twelve with newly diagnosed high-risk LGG without prior radiotherapy, one with newly diagnosed high-risk LGG with prior radiotherapy and ten with recurrent LGG. Patients were treated with the vaccine via direct subcutaneous injection every three weeks. The vaccine was well-tolerated and demonstrated immune responses in high-risk adult patients with LGG. Side effects were minimal with one grade 3 fever. Sustained and specific immune responses, as assessed by ELISPOT assays, were observed in the majority of evaluable patients. A positive correlation between immune response and progression-free survival, or PFS, was noted. Although a thorough evaluation of PFS requires a longer observation period, among 17 patients who completed eight courses, 10 had stable disease.

Clinical and regulatory strategy for SL-701

We plan to advance SL-701 into a corporate sponsored Phase 2 trial in adults, as well as a cooperative group led study in children. In preparation for these studies, we have completed commercial-scale cGMP production of API, as well as drug product formulation and fill-finish for use in our clinical trials. Upon submission and acceptance by the FDA of a corporate-sponsored IND, we expect to initiate a Phase 2 trial of SL-701 in adult patients with recurrent GBM, following initial treatment with surgery, radiation, and chemotherapy. We believe that the design of this study may enable SL-701 to obtain accelerated regulatory approval and/or serve as the foundation for a subsequent Phase 3 pivotal trial in this indication. We also plan to pursue a Phase 2 trial of SL-701 in pediatric patients with brainstem and non-brainstem HGG, which are also areas of unmet medical need.

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StemScreen®-1

StemScreen®-1 is a validated, proprietary drug discovery platform designed to identify CSC-targeted compounds based on the isolation of CSCs and evaluation of CSC gene expression profiles. CSCs are isolated from primary tumor tissue or cell lines, and then subjected to gene expression analysis using a variety of technologies, including microarray. A control tissue, such as normal bone marrow is analyzed as a comparator against the gene expression profile of the isolated CSCs. These data are then interfaced with an information base of compounds and their mechanisms of action (i.e. which gene products and pathways they impact). Compound classes are then identified as likely to impact CSC-specific pathways discovered by the gene expression analyses. Select compounds within these classes are then tested in our anti-CSC functional *in vitro* and *in vivo* assays. Compounds that demonstrate anti-CSC activity are then considered for further development, which may include lead optimization. We have utilized StemScreen®-1 to discover a number of our preclinical drug candidates.

StemScreen®-2

StemScreen®-2 is a proprietary high throughput drug discovery platform we are developing to discover novel anti-CSC compounds. Traditional oncology drug discovery screens have largely relied upon readouts that measure activity against tumor bulk, and have not been specifically designed to identify compounds with activity against CSCs. StemScreen®-2 is based on a key discovery that immortal cancer cell lines harbor not only tumor bulk but also CSCs. This discovery enables compounds to be screened, in a high throughput manner, for activity against CSCs in their natural state.

StemScreen®-2 utilizes an assay that uses live cells to track and follow CSCs in their natural state during high throughput screening and permits the rapid testing of many compounds on a small scale for enhanced efficiency. In particular, StemScreen®-2 utilizes a CSC-specific promoter linked to a reporter as a method for identifying and following CSCs in their native environment of surrounding tumor bulk, as illustrated below. In this way, StemScreen®-2 enables the identification of compound hits, in a high throughput manner, with anti-CSC activity.

Notably, prior to the development of StemScreen®-2, screens for anti-CSC compounds had been limited due to 1) reliance on finite sources of primary tissue specimens rather than immortal cancer cell lines, and 2) purification of CSCs away from the rest of the tumor, each thereby limiting screens to small libraries in relatively low throughput systems. Moreover, displacement of CSCs from their tumor microenvironment is not ideal because it can lead to unwanted changes in the CSC phenotype. Additionally, other CSC-focused screens have recently been developed that require artificial manipulation to create the CSC phenotype from non-CSCs in the context of an immortal cell line. Thus, we believe that StemScreen®-2, unlike other CSC-focused screening systems, is distinct because it is both high throughput and accurately represents the CSC phenotype in its native, unaltered state.

An initial screen of a moderately sized chemical compound library led to the identification of several hits, comprising 2.4% of the library which demonstrated activity against CSCs with greater than 50% growth inhibition. Several of these compounds were then further validated using secondary functional assays to confirm anti-CSC activity. We are currently optimizing and miniaturizing StemScreen®-2 for larger scale screening as well as expand its applicability for use in a broad range of tumor types. We have partnered with a major academic research institution with significant experience in high throughput and high-content screening for assay optimization and plans to undergo a large chemical library screening are underway.

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Preclinical pipeline

Stemline has discovered and developed a pipeline of small molecules and monoclonal antibody-based, or mAb-based, compounds directed to targets on CSCs and tumor bulk. This pipeline was built through a variety of methods, including target and lead discovery via StemScreen®, as well as through in-licensing of certain key intellectual property (see Table below).

Target	Compound	Class	In Vitro Efficacy	In Vivo Efficacy
IL-3R	SL-501	Targeted therapy	+	+
IL-3R-alpha (CD123)	SL-101	mAb-based	+	
Glypican-3, Tie-1		mAb-based		
Undisclosed	SL-601	mAb-based		
Frz, Smo, Ptc		mAb-based		
CD133		mAb-based		
Notch	SL-301	Small molecule	+	+
Undisclosed	SL-201	Small molecule	+	

IL-3R-targeted platform. We have leveraged our demonstration of clinical proof of concept for the IL-3R target with SL-401 as well as our know-how and intellectual property around IL-3R, to invest in and build out a larger IL-3R-targeted platform which currently includes the two additional product candidates: SL-501 and SL-101, our second and third generation IL-3R-targeted product candidates, respectively. Each of these product candidates has distinguishing characteristics. SL-501 is a rationally designed variant of SL-401 that binds to IL-3R with high affinity and has shown elevated potency against hematologic cancer cells both *in vitro* and in *in vivo* xenograft experiments. Because of the high potency of SL-501, less drug may be required to treat patients. SL-101 is a mAb-conjugate that binds to the alpha chain of IL-3R (CD123). IL-3R is comprised of both an alpha and beta chain. SL-101 has demonstrated potent *in vitro* cytotoxic activity against several hematologic cancer cell lines. We plan to advance SL-501 and SL-101 into IND-enabling studies.

In addition, we have identified small molecules, SL-301 and SL-201, directed to Notch and an undisclosed target, as well as a mAb program, SL-601, directed to an undisclosed target, which are all in early development. We have also in-licensed intellectual property directed to mAb-based therapeutics to validated oncology targets including Glypican-3, Tie-1, CD133, Frizzled, Smoothened and Patched. Some of these antibody targets are also being pursued by other biopharmaceutical companies. We may develop, or partner with third parties to develop, one or more of these mAbs. As with our StemScreen® discovery program, we may conduct some of these efforts by using third party contractors or by building/acquiring internal laboratories facilities.

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Patents and Proprietary Rights

We strive to protect our product candidates and exclusivity rights, as well as both maintain and fortify our position in the CSC field. We believe our intellectual property portfolio consists of early and broad filings in the area. We have focused on patents and patent applications covering, where possible, our products and methods of use of our products in disease treatment. We have also focused on patents and patent applications covering, wherever possible, broad facets of CSC-directed therapeutics, diagnostics, including companion diagnostics, and drug discovery. We have sought and continue to seek the strongest possible intellectual property protection available to us in order to prevent others from directly competing with us, as well as to exclude competition around our products, their methods of use in disease treatment, as well as, more generally, CSC-directed therapeutics, diagnostics including companion diagnostics, and drug discovery.

Our intellectual property portfolio contains 18 issued patents and 35 pending applications in the U.S. and worldwide of both in-licensed and Stemline-originated inventions. This portfolio includes patents and proprietary rights around (i) Stemline s drug candidates and (ii) CSC-focused intellectual property, which includes early and broad filings in the CSC field covering CSC therapeutics, diagnostics, including companion diagnostics, and drug discovery.

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 8,470,307 and 7,763,242 covering methods of treating AML and MDS that both expire in 2027. There are also 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 to SL-701 component, IL-13R 2 mutant, a non-exclusive worldwide license to SL-701 component, EphA2, and have filed a PCT patent application to 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-13R 2 peptide expiring in 2025, issued U.S. method of use patents (U.S. Patents 7,297,337 and 8,114,407) directed to the use of EphA2 peptides used in SL-701 expiring in 2024 and 2025, and a pending PCT patent application directed to the use of an immunogenic mutant survivin peptide that, 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 2024, 2025, or 2033.

We also in-licensed or own exclusive 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 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:

- A therapeutic 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, and related pending applications that cover methods to treat cancer through use of small molecule or oligonucleotide-based compounds that target CSCs. Patent protection for these patent families extends from 2017 or 2019, as applicable;

- A diagnostic patent (U.S. Patent 6,004,528), and related pending applications, that covers the diagnosis of cancer through detection of CSCs. Patent protection extends from 2017 or 2019, as applicable;

- Four issued patents that cover methods to treat cancer through use of monoclonal antibodies and other antibody-based compounds directed to six specific key targets: Frizzled, Glypican-3, Tie-1, CD133, Smoothened, and Patched. These U.S. Patents are: 7,361,336; 7,427,400; 7,504,103; and 7,608,259. Patent protection extends from 2017 or 2019, as applicable;

- Two pending patent applications filed in 2006 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;

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- 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-3R, including U.S. Patent 7,651,678; U.S. Patent 6,733,743; U.S. Patent 8,163,279; and other pending applications. Patent protection, to the extent it has or may issue, would be expected to extend to 2021; and

- Pending patent applications 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.

Intellectual Property Strategy

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. 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

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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.

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 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 $0.8 million in the aggregate. Additionally, upon our request, the agreement requires Scott and White to either assign to us its IND for SL-401 or grant us the exclusive right to reference its IND in

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the event we file our own IND for SL-401. 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.

University of Pittsburgh

Exclusive License Agreement to IL-13R 2 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-13R 2, an active ingredient of SL-701, our brain cancer vaccine 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 vaccines (including SL-701, which has been developed by the University under a separate vaccine 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 $0.4 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 $4.1 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-13R 2 peptide, as well as other vaccines 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-13R 2 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 $25,000 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

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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-13R 2 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 $15,000 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-13R 2 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. 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,

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specialty pharmaceutical and biotechnology companies, academic institutions and 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., Dainippon Sumitomo Pharma Co. Ltd., Bionomics Limited and Stem CentRx, Inc. 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, 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, including Ambit Biosciences Corporation, Celator Pharmaceuticals, Inc., Celgene Corporation, Clavis Pharma ASA, Cyclacel Pharmaceuticals, Inc., Eisai Co. Ltd., Genzyme Corporation (now a Sanofi company), CSL Limited and Sunesis Pharmaceuticals, Inc., Janssen Pharmaceutical Companies of Johnson and Johnson, 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 Celldex Therapeutics, Inc., ImmunoCellular Therapeutics, Ltd., Northwest Biotherapeutics, Inc., Agenus Inc., Merck & Co. Inc., Novartis AG, Roche Holding AG and others.

Many of our competitors may 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. In addition, 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 Cyclacel Pharmaceuticals, Inc., Sunesis Pharmaceuticals Inc., Genzyme Corporation (now a Sanofi company), Clavis Pharma ASA, Ambit Biosciences Corporation, Celgene Corporation, Eisai Co. Ltd., Macrogenics Inc., Janssen Pharmaceutical Companies of Johnson and Johnson, and Celator Pharmaceuticals, Inc., among others.

Unlike many of these drug candidates, SL-401 has been developed to target both tumor bulk and CSCs and, to date, has been shown to spare the bone marrow of toxicity. While SL-401 is currently being developed as a single agent, its favorable safety profile suggests the potential to safely combine it with other agents.

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Competition for SL-701

There are a limited number of drugs used for the treatment of brain cancer, including Temodar® (Merck & Co., Inc.), nitrosureas 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 Roche Holding AG, Novartis AG, Merck & Co., Inc., Celldex Therapeutics, Inc., ImmunoCellular Therapeutics, Ltd., Northwest Biotherapeutics, Inc., Agenus Inc., and others.

Unlike many of these drug candidates, SL-701 has been developed to target both tumor bulk and CSCs. While SL-701 is currently being developed as a single agent, its favorable safety profile suggests the potential to safely combine it with other agents.

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 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 Investigational New Drug Application, or an IND, which must become effective 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 GCPs, to establish the safety and efficacy of the proposed drug or biologic for its intended use;

- Submission to the FDA of a New Drug Application, or an NDA, for a new drug product, or a Biologics License Application, 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 cGMP, 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.

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

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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 and with applicable cGMP requirements. Further, each clinical trial must be reviewed and approved by an independent 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 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.

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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.

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 ten months in which to complete its initial review of a standard NDA or BLA and respond to the applicant, and 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, 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. During the approval process, the FDA also will determine whether a risk evaluation and mitigation strategy, or REMS, is necessary to assure the safe use of the drug or biologic. 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, 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 practice, or cGCP. If the FDA determines the application, 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. 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.

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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 the United States.

Expedited Development and Review Programs

The FDA 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.

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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, record-keeping requirements, reporting of unanticipated changes in 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, and promotional activities involving the internet. Failure to comply with FDA requirements can have negative consequences, including for cause inspections; warning letters from the FDA, including demands for immediate discontinuation of noncomplying materials; 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. 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 results 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.

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. 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. 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. 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, can also delay the submission or the approval of certain applications of companies seeking to reference another company s NDA. If the new

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drug is a new chemical entity subject to an NDA, the FDCA provides a five-year period of non-patent marketing exclusivity within the United States to the first applicant to obtain approval of an NDA for a new chemical entity. A drug is a new chemical entity if the FDA has not previously approved any other new drug containing the same active moiety, which is the molecule or ion responsible for the action of the drug substance. During the exclusivity period, the FDA may not accept for review an abbreviated new drug application, or ANDA, or a 505(b)(2) NDA submitted by another company for another version of such drug where the applicant does not own or have a legal right of reference to all the data required for approval.

However, such an application may be submitted after four years if it contains a certification of patent invalidity or non-infringement to one of the patents listed with the FDA by the innovator NDA holder. The FDCA also provides three years of marketing exclusivity for an NDA, or supplement to an existing NDA if new clinical investigations, other than bioavailability studies, that were conducted or sponsored by the applicant are deemed by the FDA to be essential to the approval of the application, for example new indications, dosages or strengths of an existing drug. This three-year exclusivity covers only the conditions associated with the new clinical investigations and does not prohibit the FDA from approving ANDAs for drugs containing the original active agent. Five-year and three-year exclusivity will not delay the submission or approval of a full NDA. However, an applicant submitting a full NDA would be required to conduct or obtain a right of reference to all of the preclinical studies and adequate and well-controlled clinical trials necessary to demonstrate safety and effectiveness.

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 voluntary 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. Under the FY2014 budget proposal President Obama submitted to Congress in 2013, the Administration requested that reference product exclusivity would decrease from 12 to seven years beginning in 2013. 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 February 2012, the FDA issued three draft guidance documents on biosimilar product development. The FDA is soliciting comments on the draft guidance documents which are described by the FDA as follows: (1) Scientific Considerations in Demonstrating Biosimilarity to a Reference Product, which is intended to assist companies in demonstrating that a proposed therapeutic protein product is biosimilar to a reference product for the purpose of submitting an application, called a 351(k) application, to the FDA. This draft guidance describes a risk-based totality-of-the-evidence approach that the FDA intends to use to evaluate the data and information submitted in support of a determination of biosimilarity of the proposed product to the reference product; (2) Quality Considerations in Demonstrating Biosimilarity to a Reference Protein Product, which provides an overview of

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analytical factors to consider when assessing biosimilarity between a proposed therapeutic protein product and a reference product for the purpose of submitting a 351(k) application; and (3) Biosimilars: Questions and Answers Regarding Implementation of the Biologics Price Competition and Innovation Act of 2009, which provides answers to common questions from people interested in developing biosimilar products. We cannot predict when or whether these draft guidance documents will be finalized or what changes the agency may make in its approach to implementation of the BPCIA.

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.

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 (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 Antikickback Statute, the federal False Claims Act, the privacy and security provisions of the Health Insurance Portability and Accountability Act, or HIPAA, 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 Antikickback 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.

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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 conducted 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 European Medicines Agency, or 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 GCP 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.

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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 are currently qualifying FDA-audited third-party CMOs to produce sufficient quantities of SL-401 and SL-701 drug substance and drug product of suitable quality for our contemplated corporate sponsored clinical trials and potential commercialization. Our manufacturing programs are being managed 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 planned corporate sponsored clinical trials. The drug product formulation and manufacturing process has been finalized and is currently being transferred to a third-party CMO with expertise in product manufacture for clinical and commercial supply.

SL-701 Manufacturing and Supply

SL-701 is a multi-peptide vaccine 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 is adequate to supply our planned corporate sponsored clinical studies. We have also now 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 now produced cGMP drug product of sufficient quality and quantity to supply our planned corporate sponsored clinical trials and at a scale that we believe will be suitable for commercialization.

Sales and Marketing

We believe that the infrastructure required to commercialize oncology products is relatively limited, which makes it cost-effective for us to internally develop a marketing and sales force. If SL-401 and SL-701 are approved by the FDA and other regulatory authorities, we plan to 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 SL-701 are being 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

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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.

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 $16.2 million in 2013, $3.4 million in 2012, and $1.6 million in 2011. 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 28, 2014, we had 17 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 part of your investment.

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

We are heavily dependent on the success of our two lead product candidates, SL-401 and SL-701, and we cannot provide any assurance that any of our product candidates will be commercialized.

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

may never be able to develop or commercialize a marketable drug.

Before we generate any revenues from product sales, 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 FDA, qualify a third-party contract manufacturer, receive regulatory approval in one or more jurisdictions, satisfy the FDA that our contract manufacturer is capable of manufacturing the product in compliance with cGMP, build a commercial organization, make substantial investments and undertake significant marketing efforts ourselves or in partnership with others. 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 biologics license application, or 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 of our product candidates will be successful in clinical trials or receive regulatory approval for trial initiation or marketing. Further, our product candidates may not receive regulatory approval even if they are successful in clinical trials. 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 size of the markets in the territories for which we gain regulatory approval and have commercial rights. 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 plan to seek regulatory approval to commercialize our product candidates in the United States, the European Union and potentially in additional foreign countries. While the scope of regulatory review and approval is similar in other countries, to obtain separate regulatory review and approval in many other countries we must comply with numerous and varying regulatory requirements of such countries regarding safety and efficacy, clinical trials and commercial sales, pricing and distribution of our product candidates, and we cannot predict success in these jurisdictions.

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

Clinical testing is expensive and, depending on the stage of development, 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 planned clinical trials that will cause us or regulatory authorities to delay, suspend or terminate those clinical trials.

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

- delays or failure reaching agreement on acceptable terms with prospective contract manufacturing organizations, or CMOs, contract research organizations, or CROs, and clinical trial sites, the terms of which can be subject to extensive negotiation and may vary significantly among different CROs and trial sites;

- failure of our third-party contractors, such as CROs and CMOs, or our investigators to comply with regulatory requirements or otherwise meet their contractual obligations in a timely manner;

- delays or failure in obtaining the necessary approvals from regulators or institutional review boards, or IRBs, in order to commence a clinical trial at a prospective trial site or to market our product candidates;

- our inability to manufacture, or obtain from third parties, adequate supply of drug substance or drug product sufficient to complete our preclinical studies and clinical trials;

- 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 requiring alterations to any of our study designs, 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 protocol 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 targets, such that we are not first to market with our product candidate;

- governmental or regulatory delays and changes in regulatory 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 IRBs of the institutions in which 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, inspection of the clinical trial operations or trial site by the FDA or other regulatory authorities resulting in the imposition of a clinical hold, unforeseen safety issues or adverse side effects, 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.

We have not initiated or completed a Company-sponsored clinical trial. Consequently, we may not have the necessary expertise or capabilities, including adequate staffing, to successfully manage the initiation, execution and completion of any of our clinical trials, including our planned Company-sponsored clinical trials of SL-401 and SL-701, to lead to our obtaining marketing approval for our product candidates in a timely manner, or at all.

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If we are able to conduct our intended pivotal clinical trial of a product candidate, the results of such trial may not be adequate to support marketing approval. Because our product candidates are intended for use in life-threatening diseases, in most cases we ultimately intend to seek marketing approval for each product candidate based on the results of a single pivotal clinical trial. As a result, these trials may receive enhanced scrutiny from the FDA. For any such pivotal trial, if the FDA disagrees with our choice of primary endpoint or the results for the primary endpoint are not robust or significant relative to control, are subject to confounding factors, or are not adequately supported by other study endpoints, including possibly overall survival, or OS, or overall response rate, or ORR, the FDA may refuse to approve a BLA or NDA based on such intended pivotal trial. The FDA may require the completion of additional clinical trials as a condition for approving our product candidates.

If we experience delays in the completion of, or 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. 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.

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 clinical trials of product candidates may not be predictive of the results of subsequent later-stage clinical trials. Product candidates in later-stage 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 future clinical trial results may not be successful for these or other reasons.

This drug candidate development risk is heightened by any changes in planned clinical trials compared to completed clinical trials. As product candidates are advanced through preclinical studies to early and later-stage clinical trials 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 planned clinical trials may be adversely affected by the following anticipated changes:

- As we optimize and scale-up production of SL-401 and SL-701, there will be manufacturing, formulation and other process and analytical changes that are part of the optimization and scale-up typically necessary for producing drug substance and drug product of a quality and quantity sufficient for later-stage clinical development and commercialization. Delays in any of these steps may delay initiation and completion of clinical trials. We will 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. We may determine that certain doses and regimens are optimal for initial near-term therapy whereas the same, or other, doses and regimens are optimal for longer-term maintenance therapy.

- We plan to treat patients with certain indications that have not yet been treated with SL-401. These may include certain rare malignancies such as mastocytosis, hypereosinophilic syndrome, myelofibrosis, chronic myelomonocytic leukemia, hairy cell leukemia, and others, as well as multiple myeloma, or MM, and early stages of acute myeloid leukemia, or AML. In these instances, we may choose to treat patients at several different doses and multi-cycle dosing regimens to determine to optimal doses and regimens for both near-term and long-term disease control in each indication.

- We plan to develop SL-701 as an injection administered under the skin, or subcutaneously, in future trials. The 701-Ped-G Study and 701-Adult-LGG Study used this method of delivery. The 701-Adult-RHGG Study used a different method of delivery, in which dendritic cells, which are a type of immune cell, were removed from the patient, exposed to vaccine peptides, and then re-injected into or near a lymph node of the patient (intra/peri-nodally). Thus, our plan continues the subcutaneous injection method used in two of the previous studies and represents a change from one of the previous studies.

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- We plan to manufacture and formulate SL-701 as a mixture of IL-13R 2 mutant peptide, EphA2 peptide, survivin mutant peptide, and a helper peptide. In the 701-Ped-G and 701-Adult-RHGG Studies, the vaccine, which is comprised of IL-13R 2 and EphA2, was mixed with additional peptides, including YKL-40 and GP-100 peptides in the Adult-RHGG Study, and survivin wildtype peptide in the 701-Ped-G Study. We have decided to advance SL-701 into future trials with an additional peptide an immunogenic mutant form of survivin. Whereas wildtype survivin had been used previously in the 701-Ped-G Study in combination with IL-13R 2 and EphA2, we believe that an immunogenic mutant form of survivin may provide benefit over wildtype when combined with IL-13R 2 and EphA2.

- We plan to change the adjuvant used in the administration of the vaccine from poly-ICLC to granulocyte-colony-stimulating factor, or GM-CSF, and imiquimod which we believe are commercially viable and state-of-the-art adjuvants.

- In some of our future trials, we may combine SL-401 or SL-701 with other therapies such as chemotherapy, radiation, targeted therapy, or anti-angiogenic therapy. We have not yet clinically tested these combinations. While there do not appear to be overlapping toxicities with these combinations, there is always the risk of unforeseen toxicities. Accordingly, we plan to conduct early analyses of safety in such trials and make any appropriate adjustments, if necessary.

Any of the aforementioned, or other, changes could make the timing, including initiation, patient accrual, or results of our planned clinical trials or other future clinical trials, less predictable and could cause our product candidates to perform differently, including causing toxicities, which could delay completion of our clinical trials, delay approval of our product candidates, and/or jeopardize our ability to 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 initiate or continue clinical trials for our product candidates if we are unable to locate and enroll a sufficient number of eligible patients to participate in these trials 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 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). In particular, SL-401 is being developed initially in BPDCN and other rare diseases as well as AML; and SL-701 is being developed in adult and pediatric brain cancer. Some of these represent orphan indications for which there are very limited independently reported data on annual incidences. If the incidences 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 planned clinical trials.

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 abandon one or more clinical trials altogether.

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. In addition, approval policies, regulations, or the type and amount of preclinical 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 studies to generate data related to toxicity and other data required to support the submission of a BLA or an NDA to the FDA. 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.

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Our product 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 or implementation of our 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 from preclinical studies or clinical trials;

- the data collected from clinical trials of our product candidates or the adequacy of our right of reference to it 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;

- 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 SL-401 and SL-701, or any of our other product candidates that we may advance into 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, may grant approval contingent on the performance of costly post-marketing clinical trials, or may approve a product candidate with a label that does not include the labeling claims necessary or desirable for the successful commercialization of that product candidate. In addition, we may not be able to ultimately achieve the price we intend to charge 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. 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 that target CSCs 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.

We believe that SL-401 and SL-701 target both tumor bulk and CSCs. However, it is conceivable that SL-401, SL-701 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 clinical outcome. In addition, it is conceivable that our platform technology may ultimately fail to identify any commercially viable drugs to treat human patients with cancer.

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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 potential approval of SL-401 and SL-701, another key element of our strategy is to identify and test additional compounds that target CSCs in a variety of different types of cancer, including SL-501 and SL-101. A portion of the research that we are conducting involves new and unproven drug discovery methods, including our proprietary StemScreen® platform technology, as well as the 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 side effects or other characteristics that indicate it is unlikely to be 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, or contain requirements for potentially costly post-marketing testing, including Phase 4 clinical trials, and surveillance to monitor the safety and efficacy of the product candidate. In addition, if the FDA 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 requirements. These requirements include submissions of safety and other post-marketing information and reports, as well as continued compliance with cGMP for commercial manufacturing and compliance with cGMP and good clinical practices, or GCP, for any clinical trials that we conduct post-approval. Later discovery of previously unknown problems with a product, including adverse events of unanticipated severity or frequency, 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 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; and

- injunctions, fines or the imposition of other civil penalties or criminal penalties.

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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, 2013 of approximately $44.9 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 and cash equivalents 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 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 our lead product candidates, SL-401 and SL-701, as well as our preclinical 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 planned and anticipated 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.

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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, obtaining 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 and medical 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 (including patient accrual) or other research and development activities for one or more 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.

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.

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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 debt or equity financing more difficult to secure, more costly, and/or more dilutive. 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, leverage and expand our intellectual property portfolio;
- build and maintain robust sales, distribution and marketing capabilities, either on our own or in collaboration with strategic partners;
- gain market acceptance for our products;
- develop and maintain any strategic relationships we elect to enter into;

- satisfy our obligations under our in-license 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 are targeting. 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 second competitor.

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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 target CSCs, including Verastem, Inc., OncoMed Pharmaceuticals, Inc., Dainippon Sumitomo Pharmaceuticals, Bionomics Limited and Stem CentRx, Inc. 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, 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, including Ambit Biosciences Corporation, Celator Pharmaceuticals, Inc., Celgene Corporation, Cyclacel Pharmaceuticals, Inc., Eisai Co. Ltd., Genzyme Corporation (now a Sanofi company), Janssen Pharmaceutical Companies of Johnson and Johnson, 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 Celldex Therapeutics, Inc., ImmunoCellular Therapeutics, Ltd., Northwest Biotherapeutics, Inc., Agenus, Inc., Merck & Co. 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. We may not be able to compete successfully unless we successfully:

- design and develop products that are superior to other products in the market;
- 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 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, particularly Ivan Bergstein, M.D., our Chairman, Chief Executive Officer and President, and Eric K. Rowinsky, M.D., our Executive Vice President, Chief Medical Officer and Head of Research and Development, Ken Hoberman, our Chief Operating Officer, David Gionco, our Vice President of Finance and Chief Accounting Officer, as well as other employees, consultants and scientific and medical collaborators. As of March 28, 2014, we had 17 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 planned clinical trials or the commercialization 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 be employed by employers other than us and may have commitments under consulting or advisory contracts with other entities that may limit their availability to us.

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If our employees 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 employee fraud or other misconduct. Misconduct by employees 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 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 manage additional relationships with various strategic partners, 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, 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 injury or is found to be otherwise unsuitable during product testing, 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. Claims could also be asserted under state consumer protection acts. 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;
- 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

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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 to applicable anti-kickback, fraud and abuse and other healthcare laws and regulations, which could expose us to 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 Health Care Reform Law requires manufacturers of drugs, devices, biologics and medical supplies to report to the Department of Health and Human Services information related to physician payments and other transfers of value and physician 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.

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.

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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 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 produce hazardous waste products. We generally contract with third parties for the disposal of these materials and wastes. We cannot eliminate the risk of contamination or injury from these materials. In the event of 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 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, in the future, we are unable to establish our own sales, marketing and distribution capabilities 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, and we, including our executive officers, do not have any significant sales, marketing or distribution experience. We will be opportunistic in seeking to either build our own commercial infrastructure to commercialize SL-401, SL-701 and any future product candidates if and when they are approved, or enter into 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 any confirmation that SL-401 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 and retain adequate numbers of effective sales and marketing personnel;

- the inability of sales personnel to obtain access to physicians or persuade adequate numbers of physicians to prescribe any future products;

- the lack of complementary 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; and

- our inability to build our own commercial infrastructure to manufacture, market and sell our product candidates; and

- our inability to build and staff, or enter a partnership to support, a commercial distribution capability.

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 likely will have little 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.

If we do not establish sales, marketing and distribution capabilities successfully, either on our own or in collaboration with third parties, we will not be successful in commercializing our product candidates.

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

Even if SL-401, 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;
- acceptance by physicians, major operators of 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;

- the prevalence and severity of adverse 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.

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. In the United States, recently passed legislation may significantly change the approval requirements in ways that could involve additional costs and cause delays in obtaining approvals. 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.

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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 Affordable Care Act or 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. Although the Supreme Court has upheld the ACA in the main challenge to the constitutionality of the statute and the 2012 elections maintained divided government at the federal level, Congressional efforts to repeal the ACA continue. In addition, there may be Congressional efforts to expand the Medicaid drug rebate program to the Medicare Part D program (or to provide authority for the government to negotiate drug prices under the Medicare Part D program). This adds to the uncertainty of the legislative changes enacted as part of the ACA, and we cannot predict the impact that the ACA or any other legislative or regulatory proposals will have on our business. Regardless of whether or not the ACA is repealed, 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 Health Care Reform Law, 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. As a result, its 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 or SL-701, were to be approved as biological products under a BLA, such approved products 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 as proposed by President Obama or that may be proposed by his successors, 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.

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Risks Related to Our Dependence on Third Parties

Third parties have conducted all clinical trials of SL-401 and SL-701 so far, and our ability to influence the design and conduct of such trials was limited. Our plans to assume control over the future clinical and regulatory development of such product candidates will entail additional expenses and require us to rely on additional 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.

To date, we have not sponsored any clinical trials relating to SL-401 or SL-701. Instead, faculty members at academic institutions have conducted and sponsored all clinical trials relating to SL-401 and SL-701 under their own INDs. Because the completed SL-401 and SL-701 clinical trials 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, whether controlled by us or third parties, for any one or more reasons, including elements of the design or execution of the previous trials or safety concerns or other trial results.

While we plan to assume control of the overall clinical and regulatory development of SL-401 and SL-701 going forward, we have so far been dependent on contractual arrangements with each investigator and their respective academic institutions, and will continue to be until we assume control. Such arrangements provide us certain information rights with respect to the completed 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 compared to the first-hand knowledge we might have gained had the completed trials been Company-sponsored trials, then our ability to design and conduct our planned Company-sponsored clinical trials may be adversely affected. Additionally, the FDA may disagree with the adequacy of our interpretation 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 before we may initiate our planned trials and/or may not accept such additional data as adequate to initiate our planned trials.

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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.

To date, we have relied on academic institutions, CROs, hospitals, clinics and other third-party collaborators who are outside our control to monitor, support, conduct and/or oversee preclinical and clinical studies of our product candidates. As a result, we had 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. If we successfully assume control of the further clinical and regulatory development of SL-401 and SL-701, we will likely need to engage additional third parties. Because we currently lack and may lack in the future sufficient internal staff to monitor such third parties and to interact with the FDA, we will also be required to build out our internal staff and/or engage consultants for such purposes. 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.

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We intend to rely on third-party manufacturers to produce our clinical and preclinical product candidate supplies and we intend to rely on third-party manufacturers to produce commercial supplies of any approved product candidates. 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 product candidates or continue to sell any products we commercialize.

We do not currently own or operate any manufacturing facilities, and we lack sufficient internal staff to produce clinical and preclinical product candidate supplies ourselves. As a result, we work with third-party contract manufacturers to produce sufficient quantities of SL-401 and SL-701 for future clinical trials, preclinical testing and commercialization. If we are unable to arrange for or 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 or SL-701 or may be delayed in doing so.

We also expect to rely upon third parties to produce drug product required for the clinical trials and commercialization of our other product candidates, including SL-101 and SL-501. 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 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 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 manufacturer to optimize the manufacturing processes for SL-401 and SL-701 drug substance and drug product so that these product candidates may be routinely produced in adequate quantities of adequate quality, and at an acceptable cost, to support our planned clinical trials and ultimate commercialization. Our manufacturer may not be able to adequately demonstrate that an optimized product candidate is comparable to a previously manufactured product candidate which could cause significant delays and increased

costs to our programs. Our manufacturer 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.

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We rely on a single third-party to manufacture and supply each of our lead product candidates. Any problems experienced by a vendor could result in a delay or interruption in the supply of our product candidate to us until this vendor cures the problem or until we locate and qualify an alternative source of 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. For each of our lead product candidates we currently rely on a single third-party to purchase from third-party suppliers the materials necessary to produce our product candidates for our clinical studies. Any prolonged disruption in the operations of our third-party manufacturer 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 and any commercialization costs. In addition, 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 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. 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 validation and commercialization of our product candidates could negatively affect our business.

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

Our 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 a collaboration under the terms provided is not in our best interest, and we may terminate the 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.

We cannot be certain that patents will be issued or granted with respect to applications that are currently pending, or that issued or granted patents will not later be found to be invalid and/or unenforceable. The patent position of biotechnology and pharmaceutical companies is generally uncertain because it involves complex legal and factual considerations. The standards applied by the U.S. Patent and Trademark Office and foreign patent offices in granting patents are not always applied uniformly or predictably. For example, there is no uniform worldwide policy regarding patentable subject matter or the scope of claims allowable in biotechnology and pharmaceutical patents. Consequently, patents may not issue from our pending patent applications. As such, we do not know the degree of future protection that we will have on our proprietary product candidates and technology.

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 for SL-401 due to earlier published prior art. We have however obtained U.S. patents

for certain methods of using SL-401 to treat AML and 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 would enable a competitor to market SL-401 for such unpatented indication(s), which could lead to price erosion for sales of SL-401 for our patented indications through off-label use. Although we have an issued U.S. patent directed to the composition of matter for the mutant immunogenic IL-13R 2 peptide used in SL-701, which has been altered to make it more stimulatory to the immune system and thus designed to increase a patient s immune response to SL-701, we do not have any foreign composition of matter patent protection. We do not expect that we will be able to obtain such protection outside the U.S. in the future although we do have foreign pending patent applications that seek to 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 used in SL-701, we do not have any composition of matter patent protection. We do not expect that we will be able to obtain such protection in the future. While we have filed a PCT pending patent applications directed to methods of use of a survivin mutant peptide to be used in SL-701, we do not have any composition of matter patent protection. We do not expect that we will be able to obtain such protection in the future. While we have patent applications pending in the United States and Canada directed to our StemScreen® technology, we currently have no issued patents covering StemScreen®.

Although we have various patent applications pending in the United States and/or abroad that we anticipate may result in additional protection for both SL-401, 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 meaningful protection for SL-401, SL-701 or StemScreen®. Our inability to obtain adequate patent protection for our product candidates or platform technology could adversely affect our business.

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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 U.S. Patent and Trademark Office, or made a misleading statement, during prosecution. The outcome following legal assertions of invalidity and unenforceability during patent litigation is unpredictable. 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.

Claims that StemScreen®, our product candidates or the sale or use of our products 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 potential platform technology, StemScreen®, do not infringe third-party patents. 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. For example, we are aware of a third-party European patent 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, which could result in us having to cease commercialization of our products and subject us to money damages in such territories.

It is also possible that we 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 plaintiff. 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.

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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.

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-701, some of our other product candidates and our platform technology are protected by intellectual property licensed from academic institutions. If the licensors terminate the licenses or fail to prosecute patent applications or maintain or enforce the underlying patents, our competitive position, market share, and business prospects will be harmed.

We are a party to several license agreements relating to certain patents and/or patent applications owned by other institutions, 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 three licenses, including an exclusive license and two non-exclusive licenses, from the University of Pittsburgh for 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 also hold licenses from academic institutions relating to intellectual property underlying our SL-501 and SL-101 product candidates and our StemScreen platform technology. We expect to enter into additional license agreements as part of the development of our business. 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. 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

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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. 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, our competitors may independently develop equivalent knowledge, methods and know-how.

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.

- 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.

- The patents 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 complexity and legal complexity. Therefore,

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obtaining and enforcing biopharma patents is costly, time-consuming and inherently uncertain. In addition, Congress has passed patent reform legislation which provides new limitations on attaining, maintaining and enforcing intellectual property. Further, the Supreme Court has ruled on several patent cases in recent years, either narrowing the scope of patent protection available in certain circumstances or weakening 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 U.S. Patent and Trademark Office, the laws and regulations governing patents could change in unpredictable ways that would weaken 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 trading 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 $10.00 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 CROs and CMOs, 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 SL-401, SL-701 and other in-licensed 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;
- 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

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- 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 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 56.5% 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.

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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 will be required to devote substantial time to new 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. These rules and regulations are creating 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 for up to five years. 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 anticipate paying any cash 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.

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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

We are not a party to, and our property is not the subject of, any material pending legal proceedings.

Item 4. Mine Safety Disclosures

Not applicable.

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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 closing sales prices for our common stock as reported on the NASDAQ Capital Market for the periods indicated.

Fiscal Year Ended December 31, 2013	High	Low
First Quarter (beginning January 31, 2013)	$ 13.69	$ 10.47
Second Quarter	25.48	11.19
Third Quarter	45.30	23.10
Fourth Quarter	47.13	18.77

Holders

The number of record holders of our common stock as of March 25, 2014 was 24. 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, 2013.

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	1,326,486	$ 4,89	1,217,699
Restricted stock	229,250	N/A	
Equity compensation plans not approved by security holders			
Total	1,555,736		1,217,699

Common Stock Performance Graph

The following graph compares the cumulative total stockholder return on our common stock for the period from January 31, 2013 through December 31, 2013, 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 31, 2013, in our common stock (at the closing market price) and in each of the indices listed above, and assumes the reinvestment of dividends.

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COMPARISON OF 5 YEARS CUMULATIVE TOTAL RETURN*

Among Stemline Therapeutics, Inc., the NASDAQ Composite Index

and the NASDAQ Biotechnology Index

* $100 invested on 1/28/2013 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,					Period from August 8, 2003 (inception) to December 31,
	2013	2012	2011	2010	2009	2013
Statement of operations data:						
Grant Revenue	$ 71,000	$	$	$	$	$ 71,000
Operating expenses:						
Research and development	$ 16,178,744	$ 3,376,962	$ 1,629,026	$ 1,329,509	$ 1,054,446	$ 27,624,412
General and administrative	7,871,719	3,090,611	1,088,028	930,331	1,026,675	17,356,515
Total operating expenses	24,050,463	6,467,573	2,717,054	2,259,840	2,081,121	44,980,927
Loss from operations	(23,979,463)	(6,467,573)	(2,717,054)	(2,259,840)	(2,081,121)	(44,909,927)
Other income:	280,687	301,684	46,673	484,905	102,257	1,216,205
Other expense		(35)	(9,670)			(9,705)
Interest expense	(516,871)	(118,765)	(98,643)	(69,493)		(813,821)
Interest income	19,136	9,907	24,068	43,045	201,088	979,719
Net loss	$ (24,196,511)	$ (6,274,782)	$ (2,754,626)	$ (1,801,383)	$ (1,777,776)	$ (43,537,529)
Less: accretion of preferred stock dividends				(239,720)	(1,100,107)	(2,591,165)
Add: discount on redemption of preferred stock				12,171,765		12,171,765
Net (loss) / income attributable to common stockholders	$ (24,196,511)	$ (6,274,782)	$ (2,754,626)	$ 10,130,662	$ (2,877,883)	$ (33,956,929)
Net (loss) / income attributable to common stockholders per common share:						
Basic	$ (2.35)	$ (1.82)	$ (0.80)	$ 3.07	$ (1.02)	
Diluted	$ (2.35)	$ (1.82)	$ (0.80)	$ 2.81	$ (1.02)	
Weighted average number of common shares:						
Basic	10,317,351	3,441,995	3,441,995	3,298,793	2,824,647	
Diluted	10,317,351	3,441,995	3,441,995	3,607,030	2,824,647	

	As of December 31,				
	2013	2012	2011	2010	2009
Balance sheet data:					
Cash and cash equivalents	$ 44,200,420	$ 2,025,338	$ 5,829,886	$ 7,226,366	$ 9,236,395
Total assets	$ 85,281,196	$ 5,029,611	$ 6,453,096	$ 7,502,912	$ 9,329,341
Long-term liabilities	$ 643,000	$ 2,037,296	$ 1,665,346	$ 1,017,033	$
(Deficit)/earnings accumulated during development stage	$ (31,365,766)	$ (7,169,255)	$ (894,473)	$ 1,860,153	$ (8,510,229)
Total stockholders (deficit)/equity	$ 79,624,388	$ (2,508,420)	$ 3,205,340	$ 5,851,561	$ (6,162,215)

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

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 commercializing proprietary therapeutics that target both cancer stem cells, or CSCs, and tumor bulk. We are currently developing two clinical-stage product candidates, SL-401 and SL-701. SL-401 is a targeted therapy directed to the interleukin-3 receptor, or IL-3R, present on CSCs and tumor bulk. To date, SL-401 has demonstrated single-agent activity, including durable complete responses, or CRs, in investigator sponsored Phase 1/2 trials in several indications including blastic plasmacytoid dendritic cell neoplasm, or BPDCN, and relapsed or refractory acute myeloid leukemia, or AML. We plan to advance SL-401 into corporate sponsored trials for multiple hematologic cancer indications including BPDCN, where we intend to pursue a Phase 2 registration-directed path. We also plan to initiate in additional rare IL-3R+ malignancies including mastocytosis, hypereosinophilic syndrome, other myeloproliferative syndromes, and hairy cell leukemia studies. These studies could be expanded to serve as platform trials for potential registration. We also intend to pursue larger indications including trials in multiple myeloma, or MM, and AML in first relapse as consolidation therapy, and a Phase 3 trial in third-line AML for potential registration. SL-701 is a subcutaneously administered therapeutic cancer vaccine comprised of multiple synthetic peptides. To date, the vaccine has demonstrated single-agent activity, including durable CRs and partial responses, or PRs, in investigator sponsored Phase 1/2 trials in advanced adult and pediatric brain cancers. We plan to advance SL-701 into a corporate sponsored Phase 2 trial in adult patients with recurrent glioblastoma multiforme, or GBM, following initial treatment with surgery, radiation, and chemotherapy. We believe that the design of this study may enable SL-701 to obtain accelerated regulatory approval and/or serve as the foundation for a subsequent Phase 3 pivotal trial in this indication. We also plan to pursue a Phase 2 trial of SL-701 in pediatric patients with brainstem and non-brainstem high-grade glioma, which are also areas of unmet medical need. In addition, we have built a robust preclinical pipeline which includes next generation IL-3R-targeted compounds, SL-501 and SL-101, an innovative discovery platform, and an extensive intellectual property portfolio including some of the earliest patents in the CSC area. We believe this establishes us as a leader in this rapidly emerging area of oncology.

We are a development-stage company. We have devoted substantially all of our resources to developing our product candidates and our platform technology, building our intellectual property portfolio, business planning, raising capital, and providing 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 and issuances of convertible debt to our investors. From inception through December 31, 2013, we have received net proceeds of $101.6 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.

We have never been profitable and, from inception through December 31, 2013, our net losses from operations are $43.5 million. Our net loss from operations was $24.2 million for the year ended December 31, 2013, $6.2 million for the year ended December 31, 2012, $2.8 million for

the year ended December 31, 2011, and $1.8 million for the year ended December 31, 2010. 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. Furthermore, we expect to incur additional costs associated with operating as a public company. Accordingly, we will 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.

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 $1.0 million research grant received from the Leukemia and Lymphoma Society, or LLS, where we recognized revenue of $0.1 million during 2013. In the future, we may generate revenue from product sales. In addition, to the extent we enter into licensing or collaboration arrangements, we may have additional sources of revenue.

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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 product 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, 2013, 2012 and 2011:

	Year Ended December 31,		
	2013	**2012**	**2011**
Clinical (SL-401 and SL-701)	$ 16,085,742	$ 3,292,724	$ 1,566,141
Preclinical	93,002	84,238	62,885
Total	$ 16,178,744	$ 3,376,962	$ 1,629,026

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, including salaries, benefits, travel and stock-based compensation expense, and consultant costs;
- external research and development expenses incurred under arrangements with third parties, such as contract research organizations, or CROs, contract manufacturing organizations, or CMOs, academic institutions, and consultants;
- license fees and milestone payments related to in-licensed products and technology; and

- facilities, depreciation and other allocated expenses, which include direct and allocated expenses for rent and maintenance of facilities, depreciation of leasehold improvements, equipment and supplies.

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 increase significantly in future periods as we seek to complete development of our most advanced product candidates, SL-401 and SL-701, and continue to develop our other product candidates and our platform technology. We anticipate the majority of our research and development expense will be devoted to the development of SL-401 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, rate of progress and costs of our planned, as well as any additional, clinical trials and other research and development activities;

- future clinical trial results;

- the potential benefits of our product candidates over other therapies;

- 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;

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- the costs, timing and outcome of regulatory approvals; and

- the costs of preparing, filing, prosecuting, defending and enforcing patent claims and other intellectual property rights.

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 or if we experience significant delays in enrollment in any clinical trials, we could be required to expend significant additional financial resources and time on the completion of clinical development.

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, and business development departments. Other general and administrative expenses include facility costs, insurance expenses and professional fees for legal, consulting and accounting services.

We anticipate that our general and administrative expenses will be higher in future periods to support increases in our research and development activities, which will result in increased payroll, expanded infrastructure, increased legal, compliance, accounting and investor and public relations expenses associated with being a public company, among other factors.

Interest Income

Interest income consists of interest earned on our cash, cash equivalents and long-term investments. Given the current interest rate environment and that our primary investments are in U.S. Treasury and Agency securities and related money market funds, we expect interest income to be minimal in future quarters.

Interest Expense

Interest expense consists primarily of cash and non-cash interest costs related to our previously outstanding debt. In addition, we capitalize costs incurred in connection with the issuance of debt. We amortize these costs over the life of our debt agreements as interest expense in our statement of operations. Further, in conjunction with the conversion of our convertible notes we recorded the beneficial conversion charge as an interest expense. As a result of the successful completion of our IPO in January 2013, our 2.45% Convertible Notes were converted into stock by April 2013 and all obligations under these Convertible Notes have been satisfied as of December 31, 2013.

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,

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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. There have been no significant adjustments to date. 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 the realizability of our deferred tax assets, which primarily consist of net operating losses, or NOL, carry-forwards. In assessing the realizability of 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, 2013, 2012 and 2011, 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, 2013, we had U.S. federal net operating loss carryforwards of $50.6 million (of which $14.3 million will result in a benefit to additional paid in capital upon realization as they relate to excess benefits from stock option exercises) and research and development credits of $0.6 million which expire in 2023 through 2033.

We adopted 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.

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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, we are required to include an estimate of the number of awards that will be forfeited in calculating compensation costs. Any changes to the estimated forfeiture rates are accounted for prospectively.

For stock options 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 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 our common stock and the non-cash 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.

Revenue Recognition

We have not yet generated any revenue from product sales. Our sole source of revenue is grant revenue related to a $1.0 million research grant received from the Leukemia and Lymphoma Society in October 2013. 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, 2013 and 2012

Research and development expense. Research and development expense was $16.2 million for the year ended December 31, 2013, compared with $3.4 million for the year ended December 31, 2012, and increase of $12.8 million. The higher costs were primarily attributable to the ramp up in development activities for our lead compound SL-401. The increase in costs associated with SL-401 includes higher salaries, benefits and non-cash compensation costs of $6.0 million and manufacturing development expenses of $2.4 million. Additionally, the higher expense was partially due to $2.0 million of in-process research and development associated with an assignment agreement with our chief executive officer. As we begin to initiate clinical trials for SL-401 and SL-701 during 2014, we expect that our research and development expenses will ramp up and increase compared to prior periods. We expect this increase in costs will continue for the foreseeable future.

General and administrative expense. General and administrative expenses were $7.9 million for the year ended December 31, 2013, compared with $3.0 million for the year ended December 31, 2012, an increase of $4.9 million. The higher expenses were driven by increased salary,

benefit and non-cash compensation costs of $2.6 million to support the increased governance responsibility of a public company coupled with one-time IPO bonuses. Additionally, we incurred higher expenses for consultants, insurance and rent during 2013.

Interest expense. Interest expense was $0.5 million for the year ended December 31, 2013, compared with $0.1 million for the year ended December 31, 2012. The increase in expense of $0.4 million is due to the amortization of the debt discount of the 2.45% convertible notes and due to the charge to earnings for the beneficial conversion of the 2.45% convertible notes. The 2.45% convertible notes were converted to common stock in April 2014.

Other income. Other income was $0.3 million for both of the years ended December 31, 2013 and December 31, 2012. This income was primarily related to the research and development credit refund received from the City of New York in both years.

Comparison of Years Ended December 31, 2012 and 2011

Research and development expense. Research and development expense was $3.4 million for the year ended December 31, 2012, compared with $1.6 million for the year ended December 31, 2011, an increase of $1.8 million. This increase was primarily attributable to increased costs pertaining to the continued development of our lead compound SL-401, including $1.6 million of consulting fees and $1.5 million of salary and related costs including stock-based compensation.

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General and administrative expense. General and administrative expense was $3.0 million for the year ended December 31, 2012, compared with $1.1 million for the year ended December 31, 2011. This increase was primarily attributable to $2.0 million of corporate legal fees and professional fees.

Interest expense. Interest expense was $118,765 for the year ended December 31, 2012, compared with $98,643 for the year ended December 31, 2011, resulting in a $20,122 increase.

Interest income. Interest income was $9,907 for the year ended December 31, 2012, compared with $24,068 for the year ended December 31, 2011. The $14,161 decrease in interest income for 2012 as compared to 2011 reflected lower cash balances in 2012.

Other income. Other income was $301,684 for the year ended December 31, 2012, compared with $46,673 for the year ended December 31, 2011. The $255,011 increase in other income for 2012 as compared to 2011 was primarily due to the tax filing in the third quarter of 2012 for a $203,806 Biotechnology Tax Credit from the City of New York for calendar year 2011 and the $68,815 mark to market of the put option liability. There are no continuing performance or refund obligations related to these grants.

Liquidity and Capital Resources

Sources of Liquidity

We have generated minimal revenues to date and have funded our operations primarily through public and private sales of common stock and private sales of convertible preferred stock and issuances of convertible debt to our investors. From inception through December 31, 2013, we have received net proceeds of $101.6 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. We may continue to incur substantial operating losses even if we begin to generate revenue from our drug candidate.

As of December 31, 2013, our cash, cash equivalents and long-term investments totaled $84.4 million. We primarily invest our cash, cash equivalents and long-term investments in U.S. Treasury and Agency securities and related U.S. Treasury money market funds with the balance in commercial operating accounts. We believe that our existing cash, cash equivalents and long-term investments will be sufficient to fund our operations and our capital expenditures 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,		
	2013	2012	2011
Net cash used in operating activities	$ (16,118,487)	$ (4,126,548)	$ (1,936,480)
Net cash used in investing activities	(40,708,687)		
Net cash provided by financing activities	99,002,256	322,000	540,000
Net increase (decrease) in cash and cash equivalents	$ 42,175,082	$ (3,804,548)	$ (1,396,480)

Operating activities. The use of cash in all periods resulted primarily from our net losses adjusted for non-cash stock-based compensation expense, non-cash interest expense, the beneficial conversion of convertible interest, depreciation and charges associated with the mark to market of the put option liability and changes in the components of working capital. The net cash used in operating activities increased in 2013, 2012 and 2011 mainly due to the ramp up and acceleration of the continued clinical and manufacturing development of our lead product candidates, SL-401 and SL-701. As we begin to initiate clinical trials for SL-401 and SL-701 during 2014, we expect that our research and development expenses will ramp up and increase compared to prior periods. We expect these higher costs will continue over the next two to three years, which will increase our use of cash to fund operations. The cash used for the years ended December 31, 2013, 2012 and 2011 was also impacted by higher research and development expenses as we increased our research and development headcount coupled with an increase in general and administrative expenses to support our IPO and becoming a public company.

Investing activities. The net cash used in investing activities during 2013 resulted primarily from the purchase of long-term U.S. Treasury Agency securities.

Financing activities. The net cash provided by financing activities for the year ended December 31, 2013 were the net proceeds from our initial public offering in January 2013 and secondary public offering in May 2013. The net cash provided by financing activities for the year ended December 31, 2012 and, 2011 were due to the issuance of $0.4 million and $0.5 million of convertible notes, respectively.

Funding Requirements

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

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- continue the ongoing clinical trials, and initiate the planned clinical trials, of our lead product candidates, SL-401 and SL-701;

- continue the research and development of our other product candidates and our platform technology;

- seek to identify additional product candidates;

- acquire or in-license other products and technologies;

- seek marketing approvals for our product candidates that successfully complete clinical trials;

- establish, either on our own or with strategic partners, a sales, marketing and distribution infrastructure to commercialize any products for which we may obtain marketing approval;

- 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 clinical trials of our lead product candidates;

- the scope, progress, results and costs of compound discovery, preclinical development, laboratory testing and clinical trials for our other product candidates;

- 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, 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;

- our ability to obtain government funding and operational support for our planned clinical trial of SL-701 in pediatric patients;

- 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 your rights as 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

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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, 2013:

	Total	Less than 1 year	1-3 years	3-5 years	More than 5 years
Operating lease obligations (1)	$ 1,518,750	$ 607,500	$ 911,250	$	$
Investigator initiated clinical trials (2)	972,931	243,233	729,698		
Clinical trial CRO obligations (3)	12,507,757	5,156,452	7,351,305		
Bioprocessing Contract (4)	1,066,164	1,066,164			
License agreements (5)	695,400	81,300	273,900	137,600	202,600
Total	$ 16,761,002	$ 7,154,649	$ 9,266,153	$ 137,600	$ 202,600

(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 $50,625. The term of this lease agreement is 36 months.

(2) Reflects our investigator initiated clinical trial agreement with a leading research hospital and other participating health institutions relating to the performance of our feasibility/pilot study to evaluate the effects of SL-701, for a period through 2017.

(3) We have agreements in place with two contract research organizations (CRO s) to facilitate research, clinical and data management services in connection with our two clinical-stage product candidates, SL-401 and SL-701.

(4) 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. Services under this contract are expected to be performed during 2013 and 2014.

(5) 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.

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 sixth year. However, these payments would continue each subsequent year until the specified milestones are achieved.

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Off-Balance Sheet Arrangements

We have not entered into any transactions with unconsolidated entities whereby we have financial guarantees, subordinated retained interests, derivative instruments or other contingent arrangements that expose us to material continuing risks, contingent liabilities, or any other obligations under a variable interest in an unconsolidated entity that provides us with financing, liquidity, market risk or credit risk support, or engages in leasing, hedging, or research and development services on our behalf.

Tax Loss Carryforwards

As of December 31, 2013, we had federal net operating loss carryforwards of $50.6 million, which may be available to reduce future taxable income. We also had federal tax credits of $0.6 million, which may be used to offset future tax liabilities. The net operating loss and tax credit carryforwards will expire at various dates through 2033. 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 limitation in the event of certain cumulative changes in the ownership interest of significant stockholders over a three-year period in excess of 50%, as defined under Sections 382 and 383 of the Internal Revenue Code, as well as similar state provisions. This could limit the amount of tax attributes that can be utilized annually to offset future taxable income or tax liabilities. 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, 2013, 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. In the future, if we determine that a portion or all of the tax benefits associated with our tax carryforwards will be realized, net income would increase in the period of determination.

Recently Adopted Accounting Standards

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

Jumpstart Our Business Startups Act of 2012

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 are choosing to opt out of a provision that would allow us to take advantage of an extended transition period to comply with new or revised accounting standards applicable to public companies 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 and long-term investments of $84.4 million as of December 31, 2013, $2.0 million as of December 31, 2012 and $5.8 million as of December 31, 2011, consisting of cash, U. S. Treasury and Agency securities and Treasury-related money market funds. 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 in short-term securities. 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, 2013, 2012 and 2011, all of our liabilities were denominated in our functional currency.

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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. As of December 31, 2013, management carried out, under the supervision and with the participation of our Chief Executive Officer and Chief Accounting Officer, an evaluation of the effectiveness of the design and operation of our disclosure controls and procedures (as defined in Rules 13a-15(e) and 15d-15(e) under the Exchange Act). Our disclosure controls and procedures are designed to provide reasonable assurance that information we are required to disclose in the reports that we file or submit under the Exchange Act is recorded, processed, summarized and reported within the time periods specified in applicable rules and forms. Based upon that evaluation, our Chief Executive Officer and Chief Accounting Officer concluded that, as of December 31, 2013, our disclosure controls and procedures were effective.

Management s Annual Report on Internal Control over Financial Reporting. Our management is responsible for establishing and maintaining adequate internal control over financial reporting (as defined in Rule 13a-15(f) or Rule 15d-15(f) under the Exchange Act). Our management assessed the effectiveness of our internal control over financial reporting as of December 31, 2013. In making this assessment, our management used the criteria set forth by the Committee of Sponsoring Organizations of the Treadway Commission, known as COSO, in the 1992 Internal Control-Integrated Framework. Our management has concluded that, as of December 31, 2013, our internal control over financial reporting was effective based on these criteria.

This annual report does not include an attestation report of our independent registered public accounting firm regarding internal control over financial reporting. Management s report was not subject to attestation by our independent registered public accounting firm pursuant to rules of the Securities Exchange Commission that permit us to provide only management s report on internal control in this report.

Changes in Internal Control Over Financial Reporting. There were no changes in our internal control over financial reporting during the quarter ended December 31, 2013, that have materially affected, or are reasonably likely to materially affect, our internal control over financial reporting.

Limitations on the Effectiveness of Controls. Our management, including our Chief Executive Officer and Chief Accounting Officer, does not expect that our disclosure controls and procedures or our internal control over financial reporting will prevent all errors and all fraud. A control system, no matter how well conceived and operated, can provide only reasonable, not absolute, assurance that the objectives of the control system are met. Further, the design of a control system must reflect the fact that there are resource constraints, and the benefits of controls must be considered relative to their costs. Because of the inherent limitations in all control systems, no evaluation of controls can provide absolute assurance that all control issues and instances of fraud, if any, within our company have been detected.

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 2014 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 2014 Annual Meeting of Stockholders.

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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 2014 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 2014 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 2014 Annual Meeting of Stockholders.

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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.

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 filed on February 6, 2013 (File No.001-35619) and incorporated herein by reference.
3.2	Amended and Restated Bylaws of Stemline Therapeutics, Inc., filed as Exhibit 3.2 to Form 8-K filed on February 6, 2013 (File No. 001-35619) and incorporated herein by reference.
3.3	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. 001-35691) and incorporated herein by reference.
4.1	Specimen certificate evidencing shares of common stock, filed as Exhibit 4.1 to Form S-1/A filed 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 filed 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 filed on June 20, 2012 (File No. 333-180515) and incorporated herein by reference.
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 filed 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

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	University Technical Services Limited, commenced September 16, 2004, filed as Exhibit 10.3 to Form S-1/A filed 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 filed 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 filed 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 filed on April 2, 2012 (File No. 333-180515) and incorporated herein by reference.
10.7*	Employment Agreement, dated March 27, 2012, between the Company and John T. Cavan, filed as Exhibit 10.7 to Form S-1/A filed on June 20, 2012 (File No. 333-180515) and incorporated herein by reference.
10.8*	Employment Agreement, dated June 15, 2012, between the Registrant and Ivan Bergstein, M.D., filed as Exhibit 10.8 to Form S-1/A filed on June 20, 2012 (File No. 333-180515) and incorporated herein by reference.
10.9*	Form of Indemnification Agreement between the Registrant and each director, filed as Exhibit 10.9 to Form S-1/A filed on June 20, 2012 (File No. 333-180515) and incorporated herein by reference.
10.10*	Amended and Restated 2004 Employee, Director and Consultant Stock Plan, filed as Exhibit 10.10 to Form S-1 filed on April 2, 2012 (File No. 333-180515) and incorporated herein by reference.
10.11*	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 filed on April 2, 2012 (File No. 333-180515) and incorporated herein by reference.
10.12*	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 filed on April 2, 2012 (File No. 333-180515) and incorporated herein by reference.
10.13*	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.
10.14*	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.
10.15*	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.
10.16*	2011 Employee Cash Bonus Plan, filed as Exhibit 10.16 to Form S-1 filed on April 2, 2012 (File No. 333-180515) and incorporated herein by reference.
10.17	Senior Unsecured Convertible Note issued by the Company in favor of NB Athyrium LLC, dated March 16, 2010, filed as Exhibit 10.17 to Form S-1 filed on April 2, 2012 (File No. 333-180515) and incorporated herein by reference.
10.18	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.
10.19*	Amended and Restated 2011 Employee Cash Bonus Plan, filed as Exhibit 10.19 to Form S-1/A filed on May 21, 2012 (File No. 333-180515) and incorporated herein by reference.
10.20	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 filed on June 20, 2012 (File No. 333-180515) and incorporated herein by reference.

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10.21*	Offer Letter between the Company and Eric L. Dobmeier, dated April 25, 2012, filed as Exhibit 10.21 to Form S-1/A filed on June 20, 2012 (File No. 333-180515) and incorporated herein by reference.
10.22*	Offer Letter between the Company and J. Kevin Buchi, dated March 2, 2012, filed as Exhibit 10.22 to Form S-1/A filed on June 20, 2012 (File No. 333-180515) and incorporated herein by reference.
10.23*	Offer Letter between the Company and Kenneth Zuerblis, dated March 8, 2012, filed as Exhibit 10.23 to Form S-1/A filed on June 20, 2012 (File No. 333-180515) and incorporated herein by reference.
10.24	Amendment, dated July 26, 2012, to Senior Unsecured Convertible Note issued by the Company in favor of NB Athyrium LLC, filed as Exhibit 10.24 to Form S-1/A on July 27, 2012 (File No.333-180515) and incorporated herein by reference.
10.25*	Letter Agreement between the Company and John T. Cavan, dated July 26, 2012, filed as Exhibit 10.25 to Form S-1/A on July 27, 2012 (File No.333-180515) and incorporated herein by reference.
10.26	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.
10.27	Amendment No. 2 dated November 14, 2012, to Senior Unsecured Convertible Note issued by the Company in favor of NB Athyrium LLC, filed as Exhibit 10.27 to Form S-1/A on November 14, 2012 (File No. 333-180515) and incorporated herein by reference.
10.28*	Offer Letter between the Company and Stephen P. Hall, dated October 1, 2012, filed as Exhibit 10.28 to Form S-1/A on January 8, 2013 (File No. 333-180515) and incorporated herein by reference.
10.29*	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.
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, 2013, 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.

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STEMLINE THERAPEUTICS, INC.

(A DEVELOPMENT STAGE COMPANY)

Index to Financial Statements

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Report of Independent Registered Public Accounting Firm

The Board of Directors and Stockholders

Stemline Therapeutics, Inc.

We have audited the accompanying balance sheets of Stemline Therapeutics, Inc. (a development stage company) as of December 31, 2013 and 2012, and the related statements of operations, comprehensive loss, preferred stock and stockholders equity (deficit) and cash flows for each of the three years in the period ended December 31, 2013 and the period from August 8, 2003 (Inception) to December 31, 2013. These financial statements are the responsibility of the Company s management. Our responsibility is to express an opinion on these financial statements based on our audits.

We conducted our audits in accordance with the standards of the Public Company Accounting Oversight Board (United States). Those standards require that we plan and perform the audit to obtain reasonable assurance about whether the financial statements are free of material misstatement. We were not engaged to perform an audit of the Company s internal control over financial reporting. Our audits included consideration of internal control over financial reporting as a basis for designing audit procedures that are appropriate in the circumstances, 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. An audit also includes examining, on a test basis, evidence supporting the amounts and disclosures in the financial statements, assessing the accounting principles used and significant estimates made by management, and evaluating the overall financial statement presentation. We believe that our audits provide a reasonable basis for our opinion.

In our opinion, the financial statements referred to above present fairly, in all material respects, the financial position of Stemline Therapeutics, Inc. at December 31, 2013 and 2012, and the results of its operations and its cash flows for each of the three years in the period ended December 31, 2013 and the period from August 8, 2003 (Inception) to December 31, 2013, in conformity with U.S. generally accepted accounting principles.

/s/ Ernst & Young LLP

MetroPark, New Jersey
March 31, 2014

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STEMLINE THERAPEUTICS, INC.

(A DEVELOPMENT STAGE COMPANY)

Balance Sheets

	December 31, 2013	December 31, 2012
Assets		
Current assets:		
Cash and cash equivalents	**$ 44,200,420**	$ 2,025,338
Related party receivable	**199,615**	
Prepaid expenses and other current assets	**292,916**	299,089
Total current assets	**44,692,951**	2,324,427
Furniture and fixtures, net	**383,333**	
Long-term investments	**40,204,912**	
Deferred financing fees		2,705,184
Total assets	**$ 85,281,196**	$ 5,029,611
Liabilities and stockholders equity (deficit)		
Current liabilities:		
Accounts payable and accrued expenses	**$ 5,013,808**	$ 5,500,735
Total current liabilities	**5,013,808**	5,500,735
Deferred grant revenue	**643,000**	
Convertible notes		2,006,881
Put option liability		30,415
Total liabilities	**5,656,808**	7,538,031
Stockholders equity:		
Preferred stock $0.0001 par value, 5,000,000 shares authorized, none issued and outstanding at December 31, 2013 and 2012		
Common stock $0.0001 par value, 33,750,000 shares authorized at December 31, 2013 and 22,500,000 shares authorized at December 31, 2012, 13,095,726 shares issued and outstanding at December 31, 2013 and 3,476,501 shares issued and outstanding at December 31, 2012	**1,310**	347
Additional paid-in capital	**111,032,619**	4,660,488
Accumulated other comprehensive loss	**(43,775)**	
Accumulated deficit during the development stage	**(31,365,766)**	(7,169,255)
Total stockholders equity (deficit)	**79,624,388**	(2,508,420)
Total liabilities and stockholders equity (deficit)	**$ 85,281,196**	$ 5,029,611

See accompanying notes.

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STEMLINE THERAPEUTICS, INC.

(A DEVELOPMENT STAGE COMPANY)

Statements of Operations

	Year Ended December 31,			Period From August 8, 2003 (Inception) to December 31,
	2013	**2012**	**2011**	**2013**
Revenues:				
Grant revenue	**$ 71,000**	$	$	$ 71,000
Operating expenses:				
Research and development	**16,178,744**	3,376,962	1,629,026	27,624,412
General and administrative	**7,871,719**	3,090,611	1,088,028	17,356,515
Total operating expenses	**24,050,463**	6,467,573	2,717,054	44,980,927
Loss from operations	**(23,979,463)**	(6,467,573)	(2,717,054)	(44,909,927)
Other income	**280,687**	301,684	46,673	1,216,205
Other expense		(35)	(9,670)	(9,705)
Interest expense	**(516,871)**	(118,765)	(98,643)	(813,821)
Interest income	**19,136**	9,907	24,068	979,719
Net loss from operations	**(24,196,511)**	(6,274,782)	(2,754,626)	(43,537,529)
Less accretion of preferred stock dividends				(2,591,165)
Add discount on redemption of preferred stock				12,171,765
Net loss attributable to common stockholders	**$ (24,196,511)**	$ (6,274,782)	$ (2,754,626)	$ (33,956,929)
Net loss attributable to common stockholders per common share:				
Basic and Diluted	**$ (2.35)**	$ (1.82)	$ (0.80)	
Weighted-average shares outstanding:				
Basic and Diluted	**10,317,351**	3,441,995	3,441,995	

See accompanying notes.

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Stemline Therapeutics, Inc.

(A Development Stage Company)

Statements of Comprehensive Loss

	Year Ended December 31			Period from August 8, 2003 (Inception) to December 31,
	2013	**2012**	**2011**	**2013**
Net loss	**$ (24,196,511)**	$ (6,274,782)	$ (2,754,626)	$ (33,956,929)
Other comprehensive loss:				
Unrealized loss on investments	**(43,775)**			(43,775)
Other comprehensive loss	**(43,775)**			(43,775)
Comprehensive loss	**$ (24,240,286)**	$ (6,274,782)	$ (2,754,626)	$ (34,000,704)

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Stemline Therapeutics, Inc.

(A Development Stage Company)

Statements of Preferred Stock and Stockholders Equity (Deficit)

Period from August 8, 2003 (Inception) to December 31, 2013

	Preferred Stock		Common Stock		Subscription	Additional Paid-in	Accumulated Other Comprehensive	Earnings (Deficit) Accumulated During the Development	Total Stockholders
	Shares	Capital	Shares	Capital	Receivable	Capital	Loss	Stage	Equity (Deficit)
Balance, August 8, 2003 (Inception)									
Issuance of common stock to founder			1,807,050	$ 181	$	$ 11,756		$	$ 11,937
Nonemployee stock based compensation						16,670			16,670
Issuance of common stock			451,758	45		499,955			500,000
Subscription receivable					(25,000)				(25,000)
Net loss								(166,538)	(166,538)
Balance, December 31, 2003			2,258,808	226	(25,000)	528,381		(166,538)	337,069
Issuance of common stock			489,349	49		1,999,951			2,000,000
Nonemployee stock based compensation						551,826			551,826
Payment of subscription receivable					25,000				25,000
Net loss								(950,448)	(950,448)
Balance, December 31, 2004			2,748,157	275		3,080,158		(1,116,986)	1,963,447
Nonemployee stock based compensation						286,931			286,931
Net loss								(807,622)	(807,622)
Balance, December 31, 2005			2,748,157	275		3,367,089		(1,924,608)	1,442,756
Issuance of common stock			75,471	7		739,703			739,710
Stock-based compensation						403,132			403,132
Net loss								(1,415,982)	(1,415,982)
Balance, December 31, 2006			2,823,628	282		4,509,924		(3,340,590)	1,169,616
Issuance of preferred stock	455,518	12,500,000							
Issuance of common stock			1,019			10,043			10,043
						(35,706)			(35,706)

Stock-based compensation							
Accretion of preferred stock dividend		230,137			(230,137)		(230,137)
Net loss						(1,571,755)	(1,571,755)
Balance, December 31, 2007	455,518	12,730,137	2,824,647	282	4,254,124	(4,912,345)	(657,939)
Stock-based compensation					143,738		143,738
Accretion of preferred stock dividend		1,021,201			(1,021,201)		(1,021,201)
Net loss						(1,820,108)	(1,820,108)
Balance, December 31, 2008	455,518	13,751,338	2,824,647	282	3,376,661	(6,732,453)	(3,355,510)
Stock-based compensation					71,177		71,177
Accretion of preferred stock dividend		1,100,107			(1,100,107)		(1,100,107)
Net loss						(1,777,776)	(1,777,776)
Balance, December 31, 2009	455,518	14,851,445	2,824,647	282	2,347,731	(8,510,229)	(6,162,216)
Issuance of common stock			617,348	62	1,802,518		1,802,580
Stock-based compensation					80,535		80,535
Accretion of preferred stock dividend		239,720			(239,720)		(239,720)
Redemption of preferred stock	(455,518)	(15,091,165)				12,171,765	12,171,765
Net loss						(1,801,383)	(1,801,383)
Balance, December 31, 2010			3,441,995	344	3,991,064	1,860,153	5,851,561
Stock-based compensation					108,405		108,405
Net loss						(2,754,626)	(2,754,626)
Balance, December 31, 2011			3,441,995	344	4,099,469	(894,473)	3,205,340
Stock-based compensation					561,022		561,022
Restricted stock grants			34,506	3	(3)		
Net loss						(6,274,782)	(6,274,782)
Balance, December 31, 2012			3,476,501	347	4,660,488	(7,169,255)	(2,508,420)
Issuance of common stock for cash in connection with initial and secondary public offerings, net of issuance costs			**8,573,911**	**857**	**97,707,649**		**97,708,506**
Restricted stock grants			**304,528**	**30**	**(30)**		
Forfeiture of restricted stock grants			**(43,982)**	**(4)**	**4**		
Stock-based compensation					**4,847,086**		**4,847,086**
Issuance of common stock in connection with the exercise of stock options			**550,801**	**56**	**1,293,694**		**1,293,750**
Issuance of common stock in connection			**233,967**	**24**	**2,101,080**		**2,101,104**

with the conversion of convertible notes								
Beneficial conversion related to interest expense					**422,648**			**422,648**
Net loss							**(24,196,511)**	**(24,196,511)**
Other comprehensive loss						**(43,775)**		**(43,775)**
Balance, December 31, 2013	**$**	**13,095,726**	**$**	**1,310**	**$** **111,032,619**	**$** **(43,775)**	**$** **(31,365,766)**	**$** **79,624,388**

See accompanying notes.

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STEMLINE THERAPEUTICS, INC.

(A DEVELOPMENT STAGE COMPANY)

Statements of Cash Flows

	Year Ended December 31,			Period From August 8, 2003 (Inception) to December 31,
	2013	**2012**	**2011**	**2013**
Cash flows from operating activities				
Net loss	**$ (24,196,511)**	$ (6,274,782)	$ (2,754,626)	$ (43,537,529)
Adjustments to reconcile net loss to net cash used in operating activities:				
Depreciation	**76,667**			76,667
Stock-based compensation expense	**4,847,086**	561,022	108,405	7,046,753
Non-cash interest expense	**94,223**	118,765	98,643	391,173
Mark-to-market of put option liability	**(30,415)**	(68,815)	9,670	(111,420)
Beneficial conversion of convertible interest	**422,648**			422,648
Changes in operating assets and liabilities:				
Prepaid expenses and other current assets	**6,173**	(75,879)	53,336	(292,916)
Related party receivable	**(199,615)**			(199,615)
Accounts payable and accrued expenses	**2,218,257**	1,613,141	548,092	5,013,808
Deferred grant revenue	**643,000**			643,000
Net cash used in operating activities	**(16,118,487)**	(4,126,548)	(1,936,480)	(30,547,431)
Cash flows from investing activities				
Purchase of furniture and fixtures	**(460,000)**			(460,000)
Purchase of marketable securities	**(40,248,687)**			(60,793,774)
Redemption of marketable securities				20,545,087
Net cash used in investing activities	**(40,708,687)**			(40,708,687)
Cash flows from financing activities				
Proceeds from issuance of preferred stock, net				12,500,000
Redemption of preferred stock				(750,000)
Proceeds from issuance of common stock, net	**97,708,506**			101,550,788
Proceeds from exercise of stock options	**1,293,750**			1,293,750
Proceeds from issuance of convertible notes		322,000	540,000	862,000
Net cash provided by financing activities	**99,002,256**	322,000	540,000	115,456,538
Net increase (decrease) in cash and cash equivalents	**42,175,082**	(3,804,548)	(1,396,480)	44,200,420
Cash and cash equivalents at beginning of period	**2,025,338**	5,829,886	7,226,366	
Cash and cash equivalents at end of period	**$ 44,200,420**	$ 2,025,338	$ 5,829,886	$ 44,200,420
Supplemental disclosure of non-cash transactions				
Discount on redemption of preferred stock	$	$	$	$ 12,921,765
Issuance of common stock on redemption of preferred stock	$	$	$	$ 1,200,000
Accretion of preferred stock dividend	$	$	$	$ 1,339,827

See accompanying notes.

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STEMLINE THERAPEUTICS, INC.

(A DEVELOPMENT STAGE COMPANY)

NOTES TO FINANCIAL STATEMENTS

December 31, 2013

1. Organization and Basis of Presentation

Organization

Stemline Therapeutics, Inc. (the Company) is a clinical-stage biopharmaceutical company focused on discovering, acquiring, developing and commercializing proprietary therapeutics that target both cancer stem cells (CSCs) and tumor bulk. The Company s activities to date have primarily consisted of advancing its two clinical stage programs, expanding and strengthening its intellectual property portfolio, developing its proprietary drug discovery platform, identifying and acquiring additional product and technology rights and raising capital. Accordingly, the Company is considered to be in the development stage as defined in Financial Accounting Standards Board Accounting Standards Codification (ASC) Topic 915, Development Stage Entities. The Company was incorporated in Delaware on August 8, 2003 (Inception) and has its principal office in New York, New York.

Stemline Therapeutics, Inc. has incurred losses from operations since inception of $43.5 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 and SL-701, as well as its preclinical product candidates and drug discovery and acquisition efforts. These expenditures will 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. Furthermore, it expects to incur additional costs associated with operating as a public company. 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 planned Phase 2b clinical trial of SL-401 in BPDCN and its planned randomized Phase 2b clinical trial of SL-401 for the treatment of patients with AML as well as its planned Phase 2b clinical trials of SL-701 for the treatment of patients with brain cancer. As a result, the Company expects to continue to incur significant and increasing operating losses for the foreseeable future.

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.

Secondary Public Offering

On May 16, 2013, the Company completed a follow-on public offering (the Secondary 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 Secondary 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.

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

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 or SL-701, or for one or more indications for which it is developing SL-401 and SL-701, or delay its establishment of sales and marketing capabilities or other activities that may be necessary to commercialize SL-401 or SL-701, if the Company obtains marketing approval.

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1. Organization and Basis of Presentation (continued)

Common Stock Splits and Amendments to Certificate of Incorporation

In March 2012, the Company amended its certificate of incorporation to increase the number of authorized shares of common stock from 3,000,000 shares to 3,515,000 shares.

On July 16, 2012, the Company filed an amendment to its Certificate of Incorporation whereby it (i) increased the number of authorized shares of common stock from 3,515,000 to 45,000,000 shares and increased the number of authorized shares of preferred stock from 100,000 to 10,000,000 shares and (ii) effectuated a 3.6141-for-1 forward stock split of its common stock.

On November 8, 2012, the Company filed an amendment to its Certificate of Incorporation whereby it (i) decreased the number of authorized shares of common stock from 45,000,000 to 22,500,000 shares and decreased the number of authorized shares of preferred stock from 10,000,000 to 5,000,000 shares and (ii) effectuated a 1-for 2.0 reverse stock split of its common stock. The accompanying audited financial statements and notes to the audited financial statements give retroactive effect to the July 16, 2012 and the November 8, 2012 stock splits for all periods presented.

At the 2013 meeting of stockholders held on June 19, 2013, stockholders voted in favor of an amendment to the Company s Restated Certificate of Incorporation to increase the Company s authorized share capital by 11,250,000 shares of common stock. As of December 31, 2013, the Company was authorized to issue 33,750,000 shares of common stock.

2. Summary of Significant Accounting Policies

Use of Estimates

The preparation of financial statements in conformity with generally accepted accounting principles in the US (US 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 consolidated financial statements and accompanying notes. Actual results could differ from those estimates.

Reclassifications

Certain prior period amounts have been reclassified to conform to the current year presentation.

Cash and Cash Equivalents

Cash and cash equivalents consist of highly liquid instruments purchased with an original maturity of three months or less. At December 31, 2013 and 2012, cash equivalents consist of deposits in financial institutions. 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 income (expense), net. Realized gains and losses, if any, are determined using the specific identification method and are included in other income (expense), net. 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 long-term investments are carried at fair value (see Note 6). Financial instruments including other assets, accounts payable and accrued liabilities are carried at cost, which approximated fair value given their short-term nature.

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2. Summary of Significant Accounting Policies (continued)

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. The Company established its available for sale portfolio in 2013. For the year ended December 31, 2013 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 is three 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 2013. For the year ended December 31, 2013 the Company did not realize any impairment losses.

Deferred Financing Fees

Deferred financing fees include legal, accounting, printing, and other fees directly attributable to the Company s offering of its equity securities. These fees are deferred and capitalized on the balance sheet. Costs attributable to equity offerings are charged against proceeds of the offering once the offering is completed.

Grant Revenue Recognition

The Company has not yet generated any revenue from product sales. The Company s sole source of revenue is grant revenue related to a $1.0 million research grant received from the Leukemia and Lymphoma Society in October 2013. This research grant was awarded to the Company to support funding some of the costs for the upcoming 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 $0.1 million of revenue related to the Leukemia and Lymphoma Society grant for the year ended December 31, 2013, which reflects three months of revenue recognized 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 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 performed by third parties; 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.

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2. Summary of Significant Accounting Policies (continued)

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 their realizability. 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.

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 is required to include an estimate of the number of awards that will be forfeited in calculating compensation costs. Any changes to the estimated forfeiture rates are accounted for prospectively.

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 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.

Segment information

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

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2. Summary of Significant Accounting Policies (continued)

Recent Accounting Pronouncements

In July 2013, the FASB issued ASU 2013-11, *Income Taxes (Topic 740): Presentation of an Unrecognized Tax Benefit When a Net Operating Loss Carryforward, a Similar Tax Loss, or a Tax Credit Carryforward Exists.* Under this new guidance, companies must present this unrecognized tax benefit in the consolidated financial statements as a reduction to deferred tax assets created by net operating losses or other tax credits from prior periods that occur in the same taxing jurisdiction. If the unrecognized tax benefit exceeds such credits it should be presented in the consolidated financial statements as a liability. This update is effective for annual and interim reporting periods for fiscal years beginning after December 15, 2013. The adoption of this standard is disclosure related only and will not have any impact on the Company s operating results and financial position.

In February 2013, the FASB issued ASU 2013-03, *Financial Instruments (Topic 825)*. The amendments in the Update clarifies the scope and applicability of a disclosure exemption that resulted from the issuance of Accounting Standards Update No. 2011-04, Fair Value Measurement (Topic 820): Amendments to Achieve Common Fair Value Measurement and Disclosure Requirements in U.S. GAAP and IFRSs . The amendment clarifies that the requirement to disclose the level of the fair value hierarchy within which the fair value measurements are categorized in their entirety (Level 1, 2, or 3) does not apply to nonpublic entities for items that are not measured at fair value in the statement of financial position, but for which fair value is disclosed. ASU 2013-03 is effective upon its issuance. The adoption of this standard did not have a material impact on the Company s consolidated financial statements.

In February 2013, the FASB issued ASU No. 2013-02, *Comprehensive Income (Topic 220): Reporting of Amounts Reclassified Out of Accumulated Other Comprehensive Income* (ASU 2013-02). This ASU requires companies to present either in a single note or parenthetically on the face of the financial statements, the effect of significant amounts reclassified from each component of accumulated other comprehensive income based on its source and the income statement line items affected by the reclassification. This guidance is effective for annual reporting periods beginning after December 15, 2012. The adoption of this standard did not have a material impact on the Company s consolidated financial statements.

In June 2011, the FASB issued ASU No. 2011-05, *Comprehensive Income (Topic 220): Presentation of Comprehensive Income* (ASU 2011-05). This ASU amends Accounting Standards Codification (ASC) Topic 220, *Comprehensive Income* , to require an entity to present the total of comprehensive income, the components of net income and the components of other comprehensive income either in a single continuous statement of comprehensive income or in two separate but consecutive statements. ASU 2011-05 eliminates the option to present the components of other comprehensive income as part of the statement of equity. ASU 2011-05 became effective for the Company in 2012 and was applied retrospectively. The Company adopted this pronouncement and elected to present a separate statement of comprehensive income. The adoption of ASU 2011-05 did not have any impact on the Company s operating results and financial position.

3. Net Income (Loss) Per Common Share

The Company accounts for and discloses net income (loss) per share using the treasury stock method. Net income (loss) per common share, or basic income (loss) per share, is computed by dividing net income (loss) by the weighted-average number of common shares outstanding. Net

income (loss) per common share assuming dilutions, or diluted income (loss) per share, is computed by reflecting the potential dilution from the exercise of in-the-money stock options, non-vested restricted stock and non-vested restricted stock units.

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

	Year Ended December 31,		
	2013	**2012**	**2011**
Basic and Diluted loss per common share calculation:			
Net income loss attributable to common shareholders basic and diluted	**$ (24,196,511)**	$ (6,274,782)	$ (2,754,626)
Basic and diluted weighted-average common shares	**10,317,351**	3,441,995	3,441,995
Basic and diluted net loss per share	**$ (2.35)**	$ (1.82)	$ (0.80)

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3. Net Income (Loss) Per Common Share (continued)

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, outstanding warrants are issued and the conversion of convertible notes. For the years ended 2013, 2012, and 2011, the Company reported a loss from operations and therefore, all potentially dilutive stock options, restricted stock, outstanding warrants and convertible notes 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, outstanding warrants and convertible notes 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 affect would have been anti-dilutive were as follows:

	Year Ended December 31		
	2013	**2012**	**2011**
Unvested restricted stock	**229,250**	34,506	
Options outstanding	**1,228,486**	1,819,839	1,233,074
Warrants	**99,529**		
Convertible notes		233,967	233,967
Total	**1,557,265**	2,088,312	1,467,041

4. Investments

The following table summarizes the Company s cash equivalents and investments:

	December 31, 2013			
	Amortized Cost	**Gross Unrealized Gains**	**Gross Unrealized Losses**	**Estimated Fair Value**
Cash equivalents:				
Money market funds	$ 41,441,975	$	$	$ 41,441,975
Long-term investments:				
Fixed-income treasury portfolio:				
Federal home loan bank	13,789,246		(14,752)	13,774,494
Federal farm credit bank	11,476,874		(13,701)	11,463,173
Freddie Mac	10,020,626		(8,340)	10,012,286
Fannie Mae	4,961,941		(6,982)	4,954,959
Total Long-term investments	40,248,687		(43,775)	40,204,912
Total	$ 81,690,662	$	$ (43,775)	$ 81,646,887

The company did not hold any investments or cash equivalents at December 31, 2012.

At December 31, 2013, remaining contractual maturities of available-for-sale investments classified as current on the balance sheet were 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 unrealized gains or losses on investments reclassified from accumulated other comprehensive income to other income (expense) in the Statement of Operations during the year ended December 31, 2013.

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5. Furniture and Fixtures

Furniture and fixtures consist of the following at December 31, 2013 and 2012:

	December 31, 2013	December 31, 2012
Office furniture and fixtures	**$ 460,000**	$
Less accumulated depreciation	**(76,667)**	
Furniture and fixtures, net	**$ 383,333**	$

Depreciation expense was $76,667 for the year ended December 31, 2013.

6. 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.

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6. Fair Value Measurements (continued)

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, 2013 and 2012:

	December 31, 2013			
	Quoted Prices in Active Markets for Identical Assets (Level 1)	Significant Other Observable Inputs (Level 2)	Significant Unobservabl Inputs (Level 3)	Balance at December 31, 2013
Assets:				
Cash and cash equivalents	$ 44,200,420	$	$	$ 44,200,420
Long-term investments	40,204,912			40,204,912
Total assets at fair value	$ 84,405,332	$	$	$ 84,405,332

	December 31, 2012			
	Quoted Prices in Active Markets for Identical Assets (Level 1)	Significant Other Observable Inputs (Level 2)	Significant Unobservabl Inputs (Level 3)	Balance at December 31, 2012
Assets:				
Cash and cash equivalents	$ 2,025,338	$	$	$ 2,025,338
Total assets at fair value	$ 2,025,338	$	$	$ 2,025,338
Liabilities:				
Put option	$	$	$ (30,415)	$ (30,415)
Total liabilities at fair value	$	$	$ (30,415)	$ (30,415)

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

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6. Fair Value Measurements (continued)

Level 3 Disclosures

The changes in fair value of the Company s Level 3 put option liability during the years ended December 31, 2013, December 31, 2012 and December 31, 2011 were as follows:

		Level 3
Balance at December 31, 2010	$	89,560
Fair value adjustment to put option liability included in other expense		9,670
Balance at December 31, 2011		99,230
Fair value adjustment to put option liability included in other income		(68,815)
Balance as of December 31, 2012		30,415
Fair value adjustment to put option liability included in other income		(30,415)
Balance as of December 31, 2013	$	

For the year ended December 31, 2013, the changes in the fair value of the put option liability resulted from the expiration of the put option in conjunction with the conversion of half of the principal of the 2.45% convertible note, along with accrued interest, into common stock as a result of the IPO. The balance of the note was converted into common stock in April 2013. No other changes in valuation techniques or inputs occurred during the year ended December 31, 2013.

For the year ended December 31, 2012, the changes in the fair value of the put option liability resulted from an adjustment to the remaining period to the expected outcome and taking into consideration the July 26, 2012 and November 14, 2012 amendments to the 2.45% convertible note described under Convertible Notes below. No other changes in valuation techniques or inputs occurred during the year ended December 31, 2012.

The fair value of the put option liability was determined utilizing a probability weighted discounted financial model based on management s assessment of the likelihood of achievement of certain outcomes based on significant inputs not observable in the market, which causes it to be classified as a Level 3 measurement within the fair value hierarchy. The valuation of the put option liability uses assumptions and estimates the Company believes would be made by a market participant in making the same valuation.

7. Other Accrued Liabilities

Other accrued liabilities consist of the following:

	December 31,

		2013		2012
Accrued research and development costs	$	**1,966,360**	**$**	972,218
Accrued compensation		**2,043,704**		100,000
Short-term portion of deferred revenue		**286,000**		
Accrued legal		**372,267**		3,289,806
Other accrued liabilities		**345,477**		1,138,711
Total	$	**5,013,808**	**$**	5,500,735

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8. Convertible Notes

The following table summarizes the Company s convertible notes at December 31, 2013 and 2012:

	Principal	December 31, 2013 Accrued Interest and bond discount	Total
2.45% Convertible Notes	$	$	$
1.27% Convertible Notes			
Total	$	$	$

	Principal	December 31, 2012 Accrued Interest and bond discount	Total
2.45% Convertible Notes	$ 1,250,000	$ (116,036)	$ 1,133,964
1.27% Convertible Notes	862,000	10,917	872,917
Total	$ 2,112,000	$ (105,119)	$ 2,006,881

As a result of the successful completion of the IPO in January 2013, $625,000 in principal amount, plus accrued interest thereon, of the 2.45% Convertible Note converted into 66,913 shares of the Company s common stock at the initial public offering price of $10.00 per share. In addition, in April 2013, the Company and NB Athyrium LLC, the holder of the 2.45% Convertible Note, entered into an amendment to the 2.45% Convertible Notes pursuant to which NB Athyrium agreed to convert the remaining $625,000 in principal amount, plus accrued interest thereon, into 67,198 shares of the Company s common stock at the initial public offering price of $10.00 per share. As of December 31, 2013, all of the obligations under the 2.45% Convertible Note had been satisfied.

As a result of the successful completion of the IPO in January 2013, the 1.27% Convertible Notes and related accrued interest were converted into 99,856 shares of the Company s common stock at a conversion price equal to 87.5% of the initial public offering of $10.00 per share.

The Company originally issued the 2.45% Convertible Note on March 16, 2010, in the amount of $1.25 million in connection with the redemption of the Series A preferred stock. The 2.45% Convertible Note was initially recorded at fair value of $0.90 million and due on March 16, 2015 bearing interest at the rate of 2.45% per annum.

Pursuant to the terms of the 2.45% Convertible Notes, upon the occurrence of a non-qualified financing event, as defined in the agreement, the 2.45% Convertible Note and any accrued interest are convertible at the option of the holder into cash or shares (the Put Option) of the same securities issued in the non-qualified financing event at the same price per share used in the non-qualified financing event, or the holder may elect to continue to retain the note. The Put Option was originally recorded at approximately $111,000, its fair value on the date of issuance and marked to fair value at each reporting period. The Fair value of the Put Option was zero and $30,415 at December 31, 2013 and 2012,

respectively. The change in the fair value of the put option liability resulted from the expiration of the put option in conjunction with the conversion of half of the principal of the 2.45% convertible note, along with accrued interest, into common stock as a result of the IPO in January 2013. The balance of the note was converted into common stock in April 2013. For the years ended December 31, 2013, 2012 and 2011, changes in the fair value of the Put Option of $(30,415), $(68,815), and $9,760 respectively, were recorded in Other Income and Other Expense in the Statement of Operations.

The 2.45% and the 1.27% Convertible Notes contained a beneficial conversion option such that immediately upon the occurrence of one of the financings discussed above. As a result of the successful completion of the IPO in January 2013 and the Secondary Offering in May 2013, the Company recorded a beneficial conversion charge to interest expense with a corresponding credit to additional paid-in capital in the amount of $422,648, which reflects the difference between the conversion price and the fair value of the new shares multiplied by the number of shares.

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8. Convertible Notes (continued)

For the years ended December 31, 2013, 2012 and 2011, the Company recorded interest expense of $76,355, 77,224 and $99,000, respectively, related to the amortization of the debt discount on the Convertible Notes.

9. Capital Structure

Common Stock

At the 2013 annual meeting of stockholders held on June 19, 2013, the stockholders voted in favor of an amendment to the Company s Restated Certificate of Incorporation to increase the Company s authorized share capital by 11,250,000 shares of common stock. As of December 31, 2013, the Company was authorized to issue 33,750,000 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.

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 four 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.

10. Revenue

In October 2013, the Company entered into an award contract (the 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 Leukemia, lymphoma, Hodgkin s disease and myeloma to develop therapies to cure or mitigate these Disease s. 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 the above Diseases and their complications. Pursuant to the 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 pre-clinical and clinical product development

activities. The Company received $1.0 million in October 2013, upon execution of the Agreement and could receive the additional $2.5 million based on the completion of certain milestone events. The Company has recognized approximately $0.1 million of revenue related to the Leukemia and Lymphoma Society grant for the year ended December 31, 2013, which reflects three months of revenue recognized on a straight line basis, based on the Company s best estimates of work performed and qualifying costs incurred. The agreement terminates when there are no longer any payment obligations.

11. Stock-Based Compensation

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 1,819,839 options to purchase common stock and 34,506 restricted stock awards executed prior to the effective date of such termination remain in full force and effect pursuant to their terms and the terms of the 2004 Plan. The 2012 Plan initially 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.

As of December 31, 2013, there were 1,217,699 shares of common stock available for future grants under the 2012 Plan.

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11. Stock-Based Compensation (continued)

Total compensation cost that has been charged against operations related to the above plans was approximately $4.8 million, $0.6 million and $0.1 million for the years ended December 31, 2013, 2012 and 2011, respectively. The exercise of stock options and the vesting of restricted stock during the year ended December 31, 2013 generated an income tax deduction of approximately $16.1 million. The Company does not recognize a tax benefit with respect to an excess stock compensation deduction until the deduction actually reduces the Company s income tax liability. At such time, the Company utilizes the net operating losses generated by excess stock-based compensation to reduce its income tax payable and the tax benefit is recorded as an increase in additional paid-in-capital. No income tax benefit was recognized in the statements of operations for share-based compensation arrangements for the years ended December 31, 2013, 2012 and 2011.

The following table summarizes stock-based compensation related to the above plans by expense category for the years ended December 31, 2013, 2012 and 2011:

	Year Ended December 31,		
	2013	**2012**	**2011**
Research and development	$ **3,301,996**	$ 412,536	$ 79,955
General and administrative	**1,545,090**	148,486	28,450
Total	$ **4,847,086**	$ 561,022	$ 108,405

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 and 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, 2013, 2012 and 2011, are as follows:

	Year Ended December 31,		
	2013	**2012**	**2011**
Risk-free interest rate	**2.10%**	0.95%	2.66%
Expected volatility	**78.23%**	78.95%	72.86%
Dividend yield			
Expected life	**6.26 years**	6.25 years	6.26 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 year ended December 31, 2013 the Company issued approximately 550,801 shares of the Company s common stock upon the exercise of outstanding stock options and received proceeds of approximately $1.3 million. There were no exercises of stock options for the years ended December 31, 2012 and 2011. For the years ended December 31, 2013, 2012 and 2011 the Company realized no tax benefit from the exercise of stock options. As of December 31, 2013, there was approximately $2.8 of unrecognized compensation cost, adjusted for estimated forfeitures, related to unamortized stock option compensation which is expected to be recognized over a remaining weighted-average period of approximately 2.4 years. Total unrecognized compensation cost will be adjusted for future changes in estimated forfeitures.

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11. Stock-Based Compensation (continued)

The Company s stock options outstanding at December 31, 2013, 2012 and 2011 and changes during the years ended December 31, 2013, 2012 and 2011 are presented below:

	Options	Weighted-Average Exercise Price	Weighted-Average Remaining Contractual Life	Aggregate Intrinsic Value
Outstanding at December 31, 2010	1,009,007	$ 2.42		
Options granted	224,067	2.92		
Options exercised				
Options forfeited				
Outstanding at December 31, 2011	1,233,074	$ 2.51		
Options granted	621,828	3.30		
Options exercised				
Options forfeited	(35,063)	3.30		
Outstanding at December 31, 2012	1,819,839	$ 2.76		
Options granted	**159,500**	**19.08**		
Options exercised	**(550,801)**	**2.37**		
Options forfeited	**(102,052)**	**2.71**		
Outstanding at December 31, 2013	**1,326,486**	**$ 4.89**	**7.08**	**$ 19,937,678**
Options exercisable at December 31, 2013	**751,752**	**$ 3.44**	**6.12**	**$ 12,149,061**

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, 2013. 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 $14.8 million for the year ended December 31, 2013. There were no exercises of stock options for the years ended December 31, 2012 and 2011.

Restricted Stock

The Company grants restricted stock to its employees and Directors. Restricted stock is recorded as deferred compensation and charged against income on a straight-line basis over the vesting period, which ranges from one to four years in duration. Restricted stock to Directors are granted on a yearly basis and represent 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, 2013, 2012 and 2011, and changes during the years ended December 31, 2013 and 2012 are presented below:

	Number of Shares		Weighted-Average Grant Date Fair Value Per Share
Outstanding at December 31, 2011		$	
Shares granted	34,506		5.97
Shares vested			
Shares forfeited			
Outstanding at December 31, 2012	34,506	$	5.97
Shares granted	**304,528**		**18.18**
Shares vested	**(65,802)**		**11.53**
Shares forfeited	**(43,982)**		**23.35**
Outstanding at December 31, 2013	**229,250**	**$**	**17.34**

For the year ended December 31, 2013, the Company granted 304,528 shares of restricted stock, at a weighted-average grant date fair value of $18.18 per share amounting to approximately $5.5 million in total aggregate fair value. At December 31, 2013, approximately 229,250 shares remained unvested and there was approximately $3.1 million of unrecognized compensation cost related to restricted

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11. Stock-Based Compensation (continued)

stock which is expected to be recognized over a remaining weighted-average period of approximately 2.9 years. The total fair value of restricted stock vested during the year ended December 31, 2013 was approximately $1.3 million. There were no vestings of restricted stock for the years ended December 31, 2012 and 2011.

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 an IPO. In addition, 281,895 options commenced vesting based upon the consummation of the IPO and the Company will record $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. As of December 31, 2012, it was not probable that one of these performance conditions would be met, and as such, there is no accounting for these shares as of December 31, 2012.

Awards Granted to Non-Employees

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 options granted to non-employees was approximately $0.8 million, $0.1 million and $24,000 for the years ended December 31, 2013, 2012 and 2011, respectively.

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12. Income Taxes

The benefit for income taxes consists of the following for the years ended December 31:

	2013	2012	2011
Deferred:			
Federal	$ (8,861,971)	$ (1,775,748)	$ (786,282)
State and local	(2,813,740)	(1,062,155)	(470,377)
	(11,675,711)	(2,837,903)	(1,256,659)
Increase in valuation allowance	11,675,711	2,837,903	1,256,659
Total tax expense	$	$	$

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

	Year Ended December 31,		
	2013	2012	2011
Percent of pre-tax income:			
U.S. federal statutory income tax rate	(34.0)%	(34.0)%	(34.0)%
State taxes, net of federal benefit	(11.6)	(11.3)	(12.2)
Permanent items	(2.6)	(0.3)	0.6
Change in valuation allowance	48.2	45.6	45.6
Effective income tax rate	%	%	%

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

	December 31,	
	2013	2012
Current deferred tax assets:		
Accrued expenses	$ 1,259,692	**$ 712,590**
Valuation allowance	(1,259,692)	**(712,590)**
Total current deferred tax assets	$	**$**
Noncurrent deferred tax assets:		
Net operating loss carryforwards	$ 16,374,002	**$ 6,682,819**
Research credits	644,203	**473,380**
Convertible debt interest expense		**81,087**
Nonqualified stock compensation	2,188,701	**824,898**
	19,206,906	**8,062,184**
Valuation allowance	(19,206,906)	**(8,062,184)**
Total noncurrent deferred tax assets	$	**$**

In assessing the realizability of 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, 2013, 2012 and 2011.

The following table summarizes carryforwards of net operating losses and tax credits as of December 31, 2013:

		Amount	Expiration	
Federal net operating losses	$	50,590,000(A)	2023	2033
State net operating losses	$	50,648,000(A)	2023	2033
Research and development credits	$	644,203	2023	2033

(A) Of which $14.3 million will be a benefit to additional paid in capital upon realization as they relate to excess benefits from stock option exercises.

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12. Income Taxes (continued)

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, 2013 and does not expect this to change significantly over the next twelve months. As of December 31, 2013, the Company has not accrued interest or penalties related to uncertain tax positions. The Company s tax returns for the years ended December 31, 2008 through December 31, 2013 are still subject to examination by major tax jurisdictions.

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 $29.0 million upon achieving certain milestones, such as the initiation of clinical trials or the granting of patents. From inception through December 31, 2013, the Company has paid or accrued $2.2 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

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) to fund the activities of S&W to conduct research involving SL-401, a clinical-stage compound that the Company has exclusively licensed. This compound is being developed to treat patients with AML, BPDCN, and other hematologic cancers.

The Company is required to pay customary single digit royalties on sales, if any, of new products approved utilizing the licensed compounds, and a percentage of up-front payments the Company receives 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, or (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.

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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 an analog peptide of IL-13R 2, an active ingredient of SL-701, a vaccine that is being developed to treat patients with advanced brain cancer (the UP Agreement). 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 will be required to pay annual fees, milestones (which are contingent upon achievement of pre-defined clinical, regulatory and commercial events), single-digit royalties on net sales, if any, of new products approved utilizing the licensed compounds, 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. 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 a peptide of EphA2, another active ingredient of SL-701, which the Company may use in or packaged with proprietary vaccines, including SL-701, for the diagnosis, treatment or prevention of diseases and tumors of the brain. The Company paid UP an initial license fee and will be required to pay UP annual license maintenance fees until the first commercial sale of a licensed product, a customary single digit royalty on sales, and a minimum annual royalty following the first commercial sale of a licensed product. 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 also entered into a non-exclusive license agreement with UP for the right to use certain information and data contained in the INDs for the clinical trials 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, and UP has granted the Company a right of reference to such INDs for its planned SL-701 clinical trial of pediatric patients with glioma. The Company paid UP an initial license fee, part of which is deferred until March 2013, and will be required to make a payment following a specified regulatory milestone, and a percentage of non-royalty revenue received from any sublicensees. 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-13R 2 license agreement is terminated.

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.

Compensation Arrangements

Subsequent to the closing of the IPO, certain bonuses and salary increases in the amount of $1.0 million were paid upon approval of the board of directors and the satisfaction of certain contingencies, with an additional $0.4 million subject to the same contingencies and payable one year after the IPO.

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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, 2013, approximately $2.1 million of the contract had been paid or accrued. The remaining services under this contract are expected to be performed during 2014.

In October 2013, the Company entered into a clinical trial agreement with a leading research hospital and other participating institutions, relating to the performance of our feasibility/pilot study to evaluate the effects of SL-701. As of December 31, 2013 approximately $0.1 million in costs relating to this contract had been incurred and paid or accrued. Services under this contract are expected to be performed through 2017. The Company s total obligation under the contract is expected to be approximately $1.0 million.

The Company has agreements in place with two contract research organizations (CRO s) to facilitate research and clinical and data management services in connection with our two clinical-stage product candidates, SL-401 and SL-701. The Company s total obligation under these contracts is expected to be approximately $12.5 million through 2016.

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 is 36 months.

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

2014	$	607,500
2015		607,500
2016		303,750
	$	1,518,750

Rent expense charged to operations was approximately $0.3 million for the year ended December 31, 2013. Rent expense is included in general and administrative expenses in the Company s Statement of Operations.

In June 2008, the Company entered into an office sharing agreement relating to its corporate headquarters in New York, New York. Expense incurred under the office sharing agreement was $60,000 for each of the years ended December 31, 2010 and 2011. The Company subsequently terminated the office sharing agreement as of December 2011. In February 2012, the Company entered into a leasing agreement with respect to its current corporate offices at 750 Lexington Avenue, New York, New York, for a monthly rent of $2,041. The term of this lease agreement was six months. The Company is currently leasing the same office space on a month-to-month basis.

14. Related Party Items

Receivable from Related Party

In November 2013, the Company recorded a $0.2 million receivable from an executive of the Company related to New York State tax withholdings resulting from an exercise of stock options. This item is reflected on the Company s December 31, 2013 balance sheet as a related party receivable.

Assignment Agreement with the Company s Chief Executive Officer

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 recorded the entire purchase price to acquire in-process research and development expense. The assignment agreement does not contain any vesting or rescission/refund provisions.

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15. Other Income and Expense

The components of other income and other expense for the years ended December 31, 2013, 2012 and 2011are as follows:

Other Income:	**2013**	**2012**	**2011**
Mark-to-market valuation adjustment to Put Option	**$ 30,415**	$ 68,615	$
New York City Biotechnology R&D tax credit	**249,477**	203,806	46,673
Other	**795**	29,263	
Total other income	**$ 280,687**	$ 301,684	$ 46,673

Other expense:	**2013**	**2012**	**2011**
Mark-to-market valuation adjustment to Put Option	**$**	$	$ 9,670
Other		35	
Total other income	**$**	$ 35	$ 9,670

Other income includes funds from the City of New York for the 2013, 2012 and 2011 Biotechnology Tax Credit program. The income from these programs is a reimbursement of expenses directly related to specific qualifying research programs in accordance with the guidelines of the respective tax credit programs and there are no performance or refund obligations. These expenses were incurred in prior periods and therefore the income was recorded when the funds were received. Other income and other expense also includes the mark-to-market of the put option liability associated with the issuance of convertible notes. See Notes 6 and 8 for further discussion of the Put Option.

16. Selected Quarterly Financial Data (Unaudited)

	Quarters Ended			
	March 31	**June 30**	**September 30**	**December 31**
2013				
Net loss attributable to common stockholders	$ (5,505,646)	$ (5,450,638)	$ (5,573,524)	$ (7,666,703)
Basic and diluted net loss per common share	$ (0.90)	$ (0.55)	$ (0.45)	$ (0.60)
2012				
Net loss attributable to common stockholders	$ (1,170,384)	$ (1,844,839)	$ (1,965,463)	$ (1,294,096)
Basic and diluted net loss per common share	$ (0.34)	$ (0.54)	$ (0.57)	$ (0.38)

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Index to Exhibits

Exhibit No.	Description
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, 2013, 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, and (v) the Notes to Consolidated Financial Statements.

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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 31, 2014

STEMLINE THERAPEUTICS, INC.

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

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 31, 2014, 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/ J. Kevin Buchi	

J. Kevin Buchi	Director
/s/ Eric L. Dobmeier Eric L. Dobmeier	Director
/s/ Kenneth Zuerblis Kenneth Zuerblis	Director