STEMLINE THERAPEUTICS INC

FORM 10-K
(Annual Report)

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	NEW YORK, NY 10022
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UNITED STATES
SECURITIES AND EXCHANGE COMMISSION

Washington, D.C. 20549

FORM 10-K

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

For the fiscal year ended December 31, 2012 .

OR

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

For the transition period from to .

Commission File Number: 001-35619

STEMLINE THERAPEUTICS, INC.

(Exact name of registrant as specified in its charter)

Delaware	**45-0522567**
(State or other jurisdiction of incorporation or organization)	(I.R.S. Employer Identification No.)

750 Lexington Avenue
Sixth Floor
New York, New York 10022
(Address of principal executive offices) (Zip Code)

(646)-502-2310
(Registrant's telephone number, including area code)

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

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

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

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

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

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

Indicate by check mark 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). ☒ Yes ☐ No

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

Indicate by check mark whether the registrant is a large accelerated filer, an accelerated filer, a non-accelerated filer, 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 ☐	Accelerated filer ☐
Non-accelerated filer ☒ (Do not check if a smaller reporting company)	Smaller reporting company ☐

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

The aggregate market value of voting common stock held by non-affiliates of the registrant (assuming, for purposes of this calculation, without conceding, that all executive officers and directors are "affiliates") was $53,375,306 as of March 27, 2013, based on the closing sale price of such stock as reported on the NASDAQ Capital Market. The registrant has provided this information as of March 27, 2013 because its common stock was not publicly traded as of the last business day of its most recently completed second fiscal quarter.

There were 7,458,561 shares of the registrant's common stock outstanding as of March 27, 2013.

DOCUMENTS INCORPORATED BY REFERENCE

Portions of the registrant's Proxy Statement for the 2013 Annual Meeting of Stockholders are incorporated by reference in Part III of this Annual Report on Form 10-K.

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

SPECIAL CAUTIONARY NOTICE REGARDING FORWARD-LOOKING STATEMENTS

This annual report on Form 10-K ("Form 10-K") includes statements that are, or may be deemed, "forward-looking statements." In some cases, these forward-looking statements can be identified by the use of forward-looking terminology, including the terms "believes," "estimates," "anticipates," "expects," "plans," "intends," "may," "could," "might," "will," "should," "approximately" or, in each case, their negative or other variations thereon or comparable terminology, although not all forward-looking statements contain these words. They appear in a number of places throughout this Form 10-K and include statements regarding our intentions, beliefs, projections, outlook, analyses or current expectations concerning, among other things, our 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 products, 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;
- our ability to obtain and maintain regulatory approval of our product candidates, 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;
- the successful development of our sales and marketing capabilities;
- the performance of third-party manufacturers; 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. Furthermore, if our forward-looking statements prove to be inaccurate, the inaccuracy may be material. In light of the significant uncertainties in these forward-looking statements, you should not regard these statements as a representation or warranty by us or any other person that we will achieve our objectives and plans in any specified timeframe, or at all.

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

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

Part I

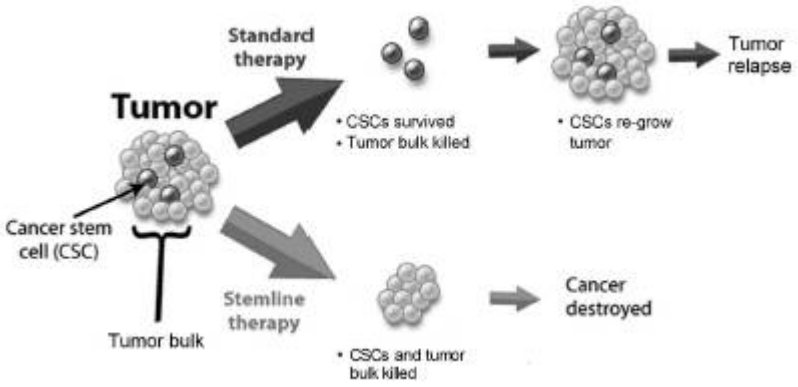
Unless the context requires otherwise, references in this report to "Stemline," "Company," "we," "us" and "our" refer to Stemline Therapeutics, Inc.

Item 1. Business

Overview

We are a clinical-stage biopharmaceutical company focused on discovering, acquiring, developing and 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 biologic targeted therapy directed to CSCs and tumor bulk, and is currently being developed for orphan indications: blastic plasmacytoid dendritic cell neoplasm, or BPDCN, a rare hematologic cancer, and third-line acute myeloid leukemia, or AML. SL-701 is a subcutaneously-delivered therapeutic cancer vaccine comprised of synthetic peptides, and is currently being developed for use in advanced pediatric and adult brain cancer. In completed Phase 1/2 clinical trials, both SL-401 and SL-701 have demonstrated single agent activity, including durable complete responses, or CRs, and longer overall survival, or OS, in heavily pretreated patients compared with that achieved in the past with traditional therapies. We plan to complete a pivotal Phase 2b single-arm trial of SL-401 in patients with BPDCN, with overall response rate as the primary endpoint. We also plan to advance SL-401 into a registration-directed randomized Phase 2b clinical trial to treat adult relapsed or refractory AML patients who failed two previous treatments (i.e., third-line AML) with CR rate and OS as co-primary endpoints. We plan to advance SL-701 into a Phase 2b clinical trial to treat pediatric patients with malignant brainstem and non-brainstem glioma. In addition, we plan to advance SL-701 into a Phase 2b clinical trial in adult recurrent or refractory glioblastoma multiforme, or GBM. We have an extensive intellectual property portfolio, a deep preclinical pipeline, and an innovative discovery platform which we believe establishes us as a leader in the CSC field.

The field of CSCs is an emerging area of cancer biology with the potential to fundamentally alter 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. 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, while standard therapies may initially shrink tumors by targeting the tumor bulk, which excludes CSCs, we believe there is a body of evidence indicating that treatment failure, tumor relapse and poor survival are largely the result of the failure of conventional cancer treatments to eradicate CSCs. Accordingly, we believe that targeting CSCs, in addition to 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.



Since our inception, we have leveraged our knowledge of CSCs to anticipate and establish a leadership position in this new field of oncology. During this time, we have developed or strategically in-licensed key intellectual property, built and validated a drug discovery platform and developed clinically active drug candidates. We believe that our early and comprehensive effort to develop a new generation of oncology therapeutics that target CSCs as well as the tumor bulk provides us with a competitive advantage.

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, Sixth Floor, New York, New York 10022 and our telephone number is (646) 502-2310.

Our website address is www.stemline.com. We will make available free of charge through our Internet 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 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 on official business days during the hours of 10:00 a.m. to 3:00 p.m. Please call the SEC at 1-800-SEC-0330 for information on the Public Reference Room. 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, founded Stemline and has advanced the Company from concept to late-stage clinical development. He was previously Medical Director of Access Oncology Inc., a private clinical stage oncology-focused biotechnology company, which was subsequently acquired. Prior to that, Dr. Bergstein was a biopharmaceuticals industry research analyst. He previously completed a residency and fellowship in internal medicine and hematology-oncology at the New York Presbyterian Hospital — Weill Medical College of Cornell University.

- Eric K. Rowinsky, M.D. — 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 FDA approval of Erbitux ® for head and neck and colorectal cancers. Dr. Rowinsky currently serves on the Board of Directors of Biogen Idec Inc., as well as several other public biopharmaceutical companies.

Strategy

Our goal is to maintain and fortify a leadership position in the discovery, acquisition and development of novel oncology therapies that target CSCs. The fundamental components of our business strategy to achieve this goal include the following:

- *Be the first anti-CSC-focused company to commercialize a CSC-directed oncology drug.* As the most clinically advanced anti-CSC-focused company, we aim to fortify our leadership position and be the first to commercialize a CSC-directed oncology drug.

- *Develop and commercialize SL-401 in multiple hematological cancers.* We plan to complete a pivotal Phase 2b single-arm trial of SL-401 in patients with relapsed or refractory BPDCN, with overall response rate as the primary endpoint. We also plan to advance SL-401 into a registration-directed randomized Phase 2b clinical trial, with CR rate and OS as co-primary endpoints, in AML patients as a third-line treatment. BPDCN and AML are orphan indications, i.e., rare diseases or conditions affecting fewer than 200,000 people in the United States and each represent an unmet medical need. The SL-401 target, IL-3R, is expressed on a wide variety of hematologic cancers including AML, BPDCN, CML, MDS, and acute lymphoid leukemia, as well as lymphomas, such as non-Hodgkin's lymphoma and Hodgkin's disease, and multiple myeloma. Accordingly, we believe that SL-401 should be active in multiple hematologic cancers. These indications could represent significant market opportunities for SL-401 **.**

- *Develop and commercialize SL-701 in brain cancer.* We plan to advance SL-701 into a Phase 2b clinical trial for the treatment of pediatric patients with brainstem and non-brainstem glioma. We also plan to initiate a Phase 2b clinical trial in adult second-line GBM.

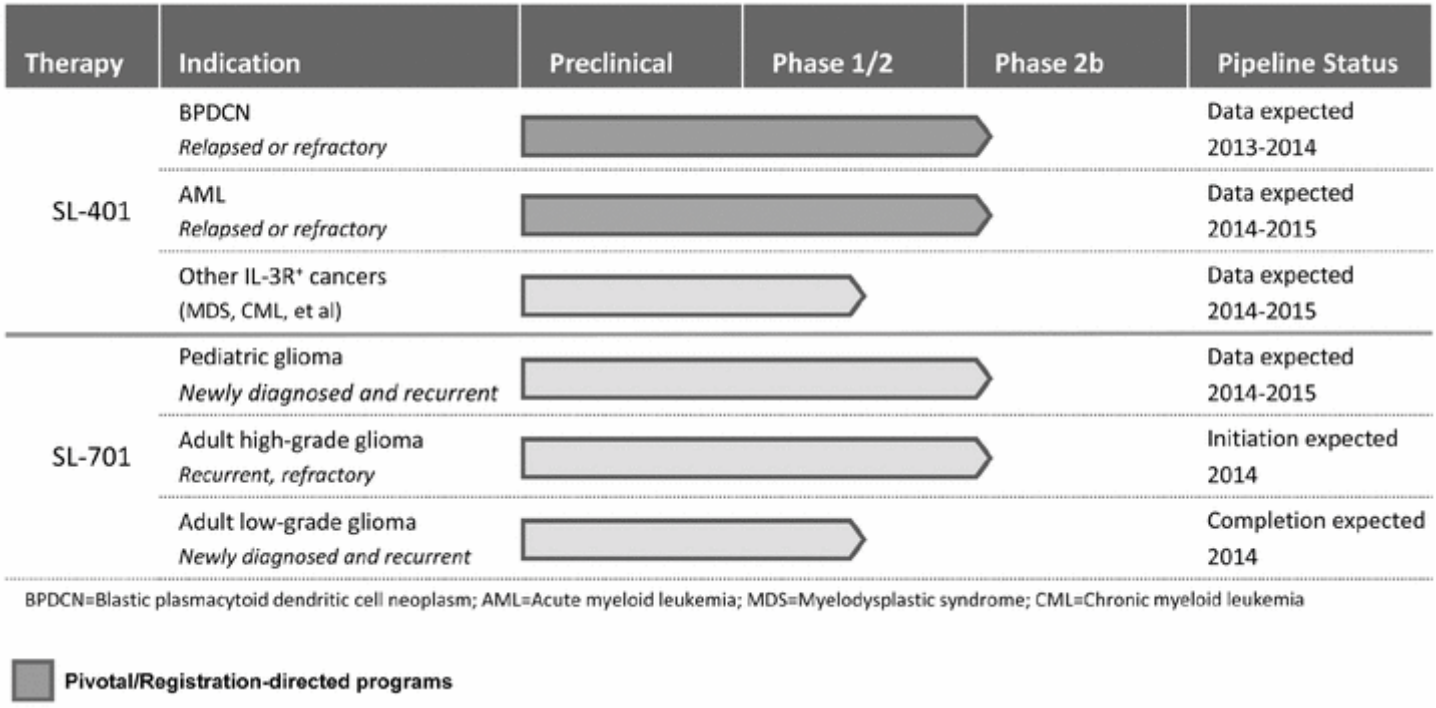
- *Leverage our proprietary drug discovery platform, StemScreen ® , to identify new therapeutics.* We intend to utilize our proprietary discovery platform to identify new CSC-targeted drug candidates. We may conduct some of these efforts internally and/or leverage our platform to forge strategic collaborations. To date, we have utilized StemScreen ® to identify a number of drug candidates.

- *Develop commercialization capabilities in North America and Europe.* We believe that the infrastructure required to commercialize our oncology products is relatively limited, which may make it cost-effective for us to internally develop a marketing effort and sales force. If SL-401 and SL-701 are approved by the FDA and other regulatory authorities for first use, we plan to commercialize these drugs ourselves in North America and 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 CSC intellectual property portfolio.* We believe that we have a strong intellectual property position relating to the development and commercialization of CSC-targeted therapeutics, diagnostics, and drug discovery. 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, in-license new intellectual property and take other steps to strengthen, leverage, and expand our intellectual property position.

Clinical Pipeline

The following table summarizes key information about our two most advanced product candidates:



SL-401 — An IL-3R-Directed Compound Targeting CSCs and Tumor Bulk

Overview

SL-401 is a clinically active biologic targeted therapy directed to the interleukin-3 receptor, or IL-3R, which is overexpressed on CSCs and more mature cancer cells derived from CSCs (i.e., tumor bulk) of multiple hematologic cancers. In AML, for example, IL-3R is overexpressed on both CSCs and tumor bulk of leukemia (i.e., blast cells). In a completed Phase 1/2 clinical trial in patients with advanced hematologic cancers, a single cycle of SL-401 alone demonstrated anti-tumor activity, including durable CRs, in relapsed or refractory patients. Specifically, a single cycle of SL-401 induced four durable CRs in relapsed or refractory patients: two CRs in BPDCN and two CRs in AML. Notably, a single cycle of SL-401 also improved the median OS of the 35 most heavily pretreated AML patients who had failed at least two previous therapies (i.e., third-line or greater) by more than two-fold compared with historical data. Moreover, a single cycle of SL-401 administered at therapeutically relevant doses (i.e., the maximum tolerated dose, or MTD, or one or two dose levels below the MTD) improved the median OS by more than three-fold compared with the historical median OS. Further, SL-401 was shown to be non-toxic to bone marrow, 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 bone marrow 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 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 is not toxic to the bone marrow, we hope to provide benefit to patients who historically have been difficult to treat with traditional therapies.

We plan to complete a pivotal Phase 2b single-arm trial in patients with relapsed or refractory BPDCN, with overall response rate as the primary endpoint. We also plan to advance SL-401 into a registration-directed randomized Phase 2b clinical trial, with CR rate and OS as co-primary endpoints, in AML patients as a third-line treatment. We also plan to evaluate the potential of SL-401 in additional hematologic cancer indications, including earlier stages of AML as well as other leukemia, lymphomas, and multiple myeloma.

In February 2011, SL-401 received 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 was previously classified by the World Health Organization, or WHO, as blastic NK cell lymphoma, agranular CD4+/CD56+ hematodermic neoplasm, and plasmacytoid dendritic cell cancer. BPDCN most commonly affects middle-aged and older patients and is approximately three times more common in men than women. BPDCN derives from plasmacytoid dendritic cells, which are specialized immune cells that express high levels of IL-3R, the target for SL-401. This malignancy most typically presents with skin lesions, as well as extracutaneous manifestations that may include the bone marrow, blood, lymph nodes, and spleen. BPDCN growth in the bone marrow results in decreased blood cell counts, thereby causing serious infections, bleeding, and invariably death. Although BPDCN can be controlled for brief periods with standard chemotherapy, including high dose chemotherapy with bone marrow transplantation, used to treat other hematologic cancers, durable clinical responses are rare and overall prognosis remains poor. There are currently no approved therapies for BPDCN, and an optimal therapeutic regimen for BPDCN has not yet been established.

Acute Myeloid Leukemia

AML 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 14,000 new AML cases occur annually in the United States, and approximately 16,000 to 18,000 new cases occur annually in Europe. The average age of an AML patient is 67 years. The National Cancer Institute estimated in 2007 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, daunorubicin and mitoxantrone. In certain circumstances, bone marrow 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. In third-line AML, there are currently no approved treatments, and these 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.

Myelodysplastic Syndrome

Myelodysplastic syndrome, or 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, 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

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.

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, genetically 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 including BPDCN and AML. Traditional therapies, such as chemotherapy, largely target rapidly dividing cells, whether malignant or normal, by 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 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 and dendritic cells, but not normal hematopoietic stem cells, and is involved in cell maturation, differentiation, and survival. IL-3R is overexpressed on multiple hematological malignancies including AML, BPDCN, MDS, CML, B cell acute lymphoid leukemia, hairy cell leukemia, Hodgkin's disease, and certain aggressive Non-Hodgkin's lymphomas. 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 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 IC_{50} (concentration that inhibits the growth of 50% of leukemia cells) values in the low picomolar range. Notably, normal bone marrow progenitor cells were relatively insensitive to SL-401. SL-401 also exhibited anti-CSC activity. In particular, SL-401 inhibited AML colony formation, an assay for stem cell activity, compared with normal human bone marrow. As further validation of an anti-CSC effect, SL-401 reduced the incorporation and growth (i.e., tumorigenicity) of AML cells, relative to normal human bone marrow, when treated *ex vivo* and reimplanted into immunodeficient mice — indicating activity at the level of the CSC. In addition, SL-401 prolonged the survival of mice implanted with human leukemia xenografts compared with untreated mice. In addition, SL-401 demonstrated high potency against BPDCN cells from patients, with an IC_{50} in the femtomolar range.

To support first-in-man clinical studies, repeat-dose animal safety studies were conducted in mice and monkeys. Toxicokinetic studies were performed to evaluate the relationships between toxicity and exposure to SL-401. Additionally, dose-limiting toxicity, or DLT, and maximum tolerated dose, or MTD, were determined from these studies to inform the subsequent Phase 1/2 human clinical trial.

SL-401 has also demonstrated preclinical activity against a variety of additional hematologic cancers. In particular, SL-401 has shown potent *in vitro* anti-leukemia activity against CML tumor bulk and CML CSCs, and increased survival in mouse models of human CML. SL-401 has also demonstrated potent *in vitro* anti-tumor activity against several lymphoid cancer types, including lymphoid leukemia, Hodgkin's and non-Hodgkin's lymphoma, and multiple myeloma.

Completed Phase 1/2 Clinical Trial — Advanced Hematologic Cancers

Overview

SL-401 was evaluated in a completed multi-center Phase 1/2 clinical trial of patients with advanced hematologic cancers, which we refer to as the 401 AHC Study. As described below, SL-401 demonstrated single agent anti-tumor activity, including durable CRs, and was well-tolerated at clinically active doses. Specifically, a single cycle of SL-401 induced four durable CRs in relapsed or refractory patients: two CRs in BPDCN and two CRs in AML. Although the study was designed so that all patients received only a single cycle of SL-401 treatment, the median OS was improved in the 35 most heavily pretreated AML patients who had failed at least two previous therapies (i.e., third-line or greater) by more than two-fold compared with historical data. Moreover, a single cycle of SL-401 administered at therapeutically relevant doses (i.e., the maximum tolerated dose, or MTD, or one or two dose levels below the MTD) improved the median OS by more than three-fold compared with the historical median OS of similar patients receiving traditional treatments. 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 was shown to be non-toxic to bone marrow, which we believe is a key differentiating feature relative to other hematologic cancer therapies.

The 401 AHC Study was undertaken in 80 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=3), with "n" representing the number of patients. The median patient age was 66 years, with a range of seven to 84 years of age. Patients received a single cycle of 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.

Dr. Arthur E. Frankel was the sponsor of the 401 AHC Study and the principal investigator at the Scott and White Cancer Research Institute/Texas A&M (Temple, TX). The other principal investigators and co-investigators of the 401 AHC Study have been Dr. Hagop M. Kantarjian and Dr. Marina Konopleva at MD Anderson Cancer Center (Houston, TX), Dr. David A. Rizzieri at Duke University (Durham, NC), and Dr. Donna E. Hogge at the British Columbia Cancer Agency (Vancouver, Canada). Updated 401 AHC Study results were presented at the American Society of Hematology (ASH) Annual Conference in December 2012.

Well-Tolerated at Clinically Active Doses

SL-401 was well-tolerated at clinically active doses. The side effect profile of SL-401 was similar to that of denileukin diftitox (Ontak ®), a compound comprised of human interleukin-2 linked to a shortened form of diphtheria toxin, which is FDA approved and has been marketed for certain forms of cutaneous T-cell lymphoma for over a decade. Similar to Ontak ®, the SL-401 profile consisted of mild to moderate fever and chills, which were manageable and not dose-limiting. Moderate to severe adverse events included liver enzyme elevations, which were mostly transient and not dose limiting, and manifestations of early capillary leak syndrome (e.g., reduced albumin, edema and weight gain) in fewer than 10% of patients. It is important to note that the side effects of Ontak ® decrease over time with each successive cycle administered. In contrast, the anticancer activity of Ontak ® is retained, and at times augmented, with each successive cycle in patients receiving multiple cycles. In particular, patients who partially responded in an initial or early cycle have been shown capable of converting to complete responders in subsequent cycles, and patients who do not respond in an initial cycle have also been shown to respond in later cycles. In fact, Ontak ® is approved on a daily for five-day schedule for up to eight cycles due to the improved safety and antitumor activity associated with multiple cycles.

The MTD of SL-401 was 16.6 µg/kg/day, with tolerable and active (i.e., therapeutically relevant) doses at 16.6 µg/kg/day as well as one and two dose levels below the MTD (12.5 and 9.4 µg/kg/day).

Non-Toxic to Bone Marrow

SL-401 was not toxic to the bone marrow, which is a key distinguishing feature relative to other hematologic cancer chemotherapies, such as nucleoside inhibitors and anthracyclines. Prior to starting treatment with SL-401, the majority of patients in the 401 AHC Study had pre-existing bone marrow suppression, likely due to the extent of their disease and/or previous exposure to myelosuppressive therapies. During and after SL-401 treatment, these patients exhibited largely stable bone marrow function relative to their pre-treatment condition, as determined by mean absolute neutrophil, hemoglobin and platelet counts of evaluable patients. As a result, we expect that SL-401, in contrast to traditional chemotherapy, may not increase a patient's susceptibility to infection, anemia, or bleeding, or increase the frequency of red blood cell or platelet transfusions or growth factor infusions. Further, because SL-401 does not appear to have overlapping toxicity with traditional hematologic cancer therapies, SL-401 may be potentially combined with more traditional agents, without the need to reduce the doses of any of the agents, in future studies involving earlier-stage AML.

Anti-Tumor Activity

In the 401 AHC Study, one cycle of SL-401 administered alone demonstrated anti-tumor activity, including reductions in leukemia blast cells in the bone marrow (i.e., reductions in tumor bulk) or disease stabilizations, in approximately half of all treated patients, the majority of whom were heavily pretreated, as summarized below. More specifically, reductions in leukemia blasts 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 67% of relapsed or refractory BPDCN patients. Durable CRs were induced in two patients with relapsed or refractory AML. There were also multiple additional cases of robust blast reductions in response to a single cycle of SL-401 treatment. Two additional CRs occurred after a single cycle of SL-401 in heavily pre-treated patients with BPDCN.

SL-401 Clinical Anti-Tumor Activity in Patients with Advanced Hematological Cancers After Only a Single Cycle of SL-401 Therapy

	AML (Relapsed refractory) (n=59)	AML (≥ 3rd line) (n=35*)	AML (Not chemo Candidate) (n=11)	MDS (High Risk) (n=7)	BPDCN (n=3)
Blast reductions/ disease stabilization	46%	43%	55%	43%	67%
Blast reductions	25% 2 Durable CRs	23% 1 Durable CR	27%	29%	67% 2 CRs

AML = Acute myeloid leukemia; MDS = Myelodysplastic syndrome; BPDCN = Blastic plasmacytoid dendritic cell neoplasm.
CR = Complete response
***Subpopulation of relapsed, refractory**

Of the two AML patients who sustained durable CRs following a single cycle of SL-401 treatment, one was a patient refractory to standard induction chemotherapy, and the other was a fourth-line AML patient. SL-401 induced a CR in an AML patient who failed standard induction chemotherapy prior to entry into the 401 AHC Study. Following SL-401 treatment, this patient's leukemic blast count decreased from 30% to undetectable levels and peripheral blood counts normalized. This CR was durable and lasted eight months. SL-401 also induced a CR in a fourth-line AML patient who had failed three previous treatment regimens, including two previous bone marrow transplantations prior to entry into the 401 AHC Study. Following SL-401 treatment, this patient's leukemic blast count decreased from 52% to undetectable levels and peripheral blood counts normalized. This CR currently exceeds 25 months in duration. It is notable that following only a single cycle of SL-401, both of these patients achieved durable CRs with normalization of blood counts and bone marrow examinations.

In addition, a single cycle of SL-401 induced CRs in two patients with heavily pre-treated BPDCN, a rare and aggressive hematologic malignancy that highly overexpresses IL-3R. The first BPDCN patient was third-line, having received several prior intensive treatment regimens including high-dose chemotherapy and bone marrow transplantation. Following SL-401 treatment, this patient's leukemic blasts, which had been in the bone marrow and bloodstream before treatment, were no longer detectable. Additionally, this patient's peripheral blood counts normalized. Furthermore, this patient's enlarged spleen

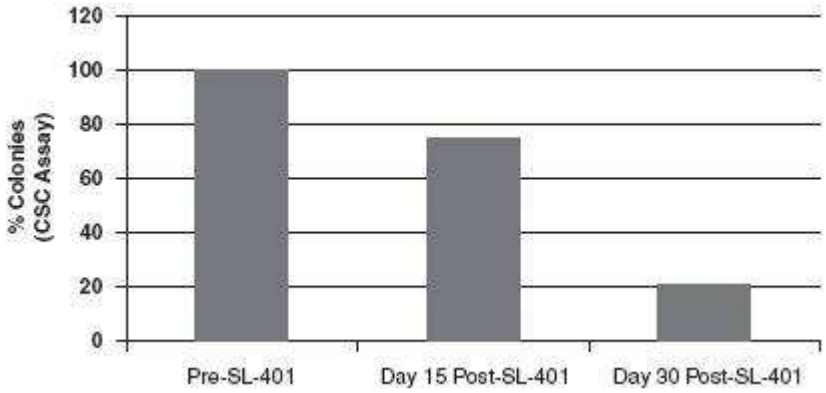
and lymph nodes, which were enlarged due to infiltration by malignant cells, also normalized. This CR is ongoing and currently exceeds four months.

The second BPDCN patient was fourth-line, having previously been treated with three intensive regimens of chemotherapy, including high-dose chemotherapy with bone marrow transplantation. The patient had malignant disease involving the skin and bone marrow, resulting in multiple cutaneous lesions and low blood counts. Following SL-401 treatment, the patient achieved a CR with no evidence of BPDCN in the skin, bone marrow, or bloodstream. In addition, the patient's blood cell counts returned to normal levels and no serious side effects were observed. This CR is ongoing and currently exceeds two months.

Anti-CSC Effect

In addition to SL-401's clinical activity, SL-401 was also shown to have activity against leukemic CSCs collected from three patients enrolled in the 401 AHC Study. In this translational study that was coordinated with the 401 AHC Study, bone marrow samples collected from several patients both before and after SL-401 treatment were tested for CSC activity in a colony formation assay (an assay that measures the ability of CSCs to form colonies). As demonstrated by Konopleva in *Blood* in 2010 describing a study of samples collected from patients enrolled in the 401 AHC Study, and as illustrated below, a substantial anti-CSC effect by SL-401 was observed, as demonstrated by considerable decreases in bone marrow CSC activity at 15 and 30 days after SL-401 treatment. At 30 days post-treatment, CSC activity decreased by an average of 79% of that measured at pretreatment. We believe that these studies also provided 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 outlived the historical median OS of heavily pretreated AML patients of 1.5 months by multiple fold, with overall survival values of 7.2 months and 13.6 months, respectively. We intend to follow-up on these positive preliminary data in future clinical trials.

SL-401 Demonstrates Clinical Anti-CSC Effect
(adapted from Konopleva et al. Blood 2010; 116:21: Abstract #3298)(1)

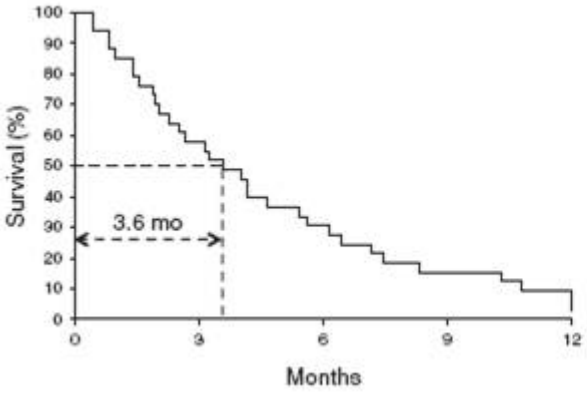


(1) *The study was conducted as a collaboration among us, MD Anderson Cancer Center and Scott and White Memorial Hospital and was completed after we licensed SL-401 from Scott and White Memorial Hospital in 2006.*

Survival Benefit

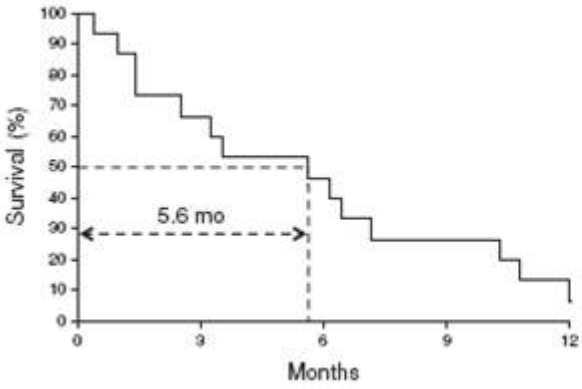
In the 401 AHC Study, SL-401, after only a single cycle of therapy, demonstrated an improvement in overall survival, or OS, of the 35 most heavily pretreated AML patients compared with historical survival results. In particular, in AML patients who had failed at least two previous therapies (i.e., third-line or greater), the median OS following a single cycle of SL-401 was 3.6 months, which is more than double the historical median OS of 1.5 months. Notably, the median OS following a single cycle of SL-401 was 5.6 months, which is more than three times the historical median OS of 1.5 months, in a cohort of 16 patients who received SL-401 at therapeutically relevant doses. The six-month and 12-month OS were also longer relative to comparable patients in a large contemporary series reported by Giles et al. in *Cancer* in 2005 and another large series reported by Keating et al., in the *Journal of Clinical Oncology* in 1989. These results are illustrated below.

SL-401 (Single Cycle): Overall Survival
Survival benefit in AML patients (≥ 3rd line) treated with
only a single cycle (all doses, n = 35 patients)
(Konopleva et al. American Society of Hematology 2012 Abstract #3625)



Median OS (historical)	Median OS (all doses)
1.5 mo	3.6 mo (95% CI: 2.3, 6.1 mo)

SL-401 (Single Cycle): Overall Survival
Survival benefit in AML patients (≥ 3rd line) treated with
only a single cycle (therapeutically relevant doses*; n = 16 patients)
(Konopleva et al. American Society of Hematology 2012 Abstract #3625)



Median OS (historical)	Median OS (all doses)
1.5 mo	5.6 mo (95% CI: 2.5, 10.8 mo)

**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 are based on the 401 AHC Study regimen of only one cycle of SL-401. We believe that 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 our planned Phase 2b clinical trials, as well as in other clinical evaluations of SL-401.

Planned Pivotal Program in BPDCN and Registration-Directed Program in Third-Line AML and Regulatory Strategy

We plan to complete a pivotal Phase 2b single-arm trial of patients with relapsed or refractory blastic plasmacytoid dendritic cell neoplasm, or BPDCN, with overall response rate as the primary endpoint. BPDCN, a rare hematologic cancer for which SL-401 has demonstrated clinical activity, is an orphan disease for which there is no approved or standard treatment. Accordingly, we believe that a registration path based on a relatively small nonrandomized trial with a surrogate endpoint can be pursued to potentially obtain accelerated approval of SL-401 in BPDCN. While we plan to enroll up to between 40 and 50 patients in the trial, if during the course of the trial the results are sufficiently robust, we will seek approval with even fewer patients.

We also plan to advance SL-401 into a registration-directed randomized Phase 2b clinical trial to treat relapsed or refractory AML patients who failed two previous treatments (i.e., third line AML). Patients with relapsed or refractory AML in the third-line setting will be randomized to treatment with either SL-401 or "physician's choice", which consists of either an available, non-investigational (i.e., "standard") therapeutic agent or combination regimen. The primary endpoints for the study will be CR rate and OS. Up to 240 patients will be randomized in a 2:1 manner whereby two patients will be treated with SL-401 for every one patient treated with physician's choice. The CR rate and OS will be evaluated in the course of various interim analyses throughout this study. Interim analyses are periodic evaluations throughout a clinical study to assess for efficacy and safety. If the study treatment is determined to be highly beneficial or futile, the study could be stopped early.

In contrast to the 401 AHC Study, which was designed so that all patients received only one cycle of treatment, multiple cycles of SL-401 will be administered in the planned trials to maximize efficacy. We believe that multiple cycle administration of SL-401 may increase the rate and duration of disease stabilization and response and, ultimately, further improve survival.

We also plan to evaluate the potential of SL-401 in additional hematologic cancer indications, including earlier stages of AML as well as other leukemias, lymphoma, and multiple myeloma.

SL-701 — A Multi-Epitope Brain Cancer Vaccine

Overview

SL-701, a clinically active therapeutic cancer vaccine comprised of synthetic peptides, is designed to direct the immune system to targets present on the CSCs and tumor bulk of brain cancer. High-grade gliomas, or HGGs, are the most aggressive brain cancers and have a poor prognosis. Treatment options are limited, particularly for pediatric patients with newly diagnosed HGG, including brainstem glioma, or BSG, and adult patients with recurrent or refractory HGG, including glioblastoma multiforme, or GBM. In two completed Phase 1/2 clinical trials, SL-701 demonstrated uncommon single agent anti-tumor activity in these indications, inducing tumor shrinkage or disease stabilization in 86% (19/22) of HLA-A2+ (as defined below) pediatric glioma patients (the 701 Ped-G Study), and 59% (13/22) of HLA-A2+ adult patients with recurrent or refractory HGG (the 701 Adult-RHGG Study). To date, there have been seven major objective tumor responses (i.e., tumor regressions) in these two studies, consisting of two CRs and five partial responses, or PRs.

Dr. Hideho Okada of the University of Pittsburgh School of Medicine was the sponsor of both the 701 Ped-G Study and the 701 Adult-RHGG Study. The principal investigators of the 701 Ped-G Study were Dr. Okada, Dr. Regina Jakacki of the Children's Hospital of Pittsburgh and Dr. Ian Pollack of the University of Pittsburgh School of Medicine. Dr. Okada was the principal investigator of the 701 Adult-RHGG Study. Trial results were delivered via oral presentation at the American Society of Clinical Oncology (ASCO) Annual Conference in June 2011. Trial results were also presented at the American Association for Cancer Research (AACR) Annual Meeting in April 2012.

We plan to advance SL-701 into a Phase 2b clinical trial to treat HLA-A2+ pediatric patients with malignant brainstem and non-brainstem glioma. We plan to fund this trial primarily through government funding, if available. We also plan to initiate a Phase 2b clinical trial in HLA-A2+ adult patients with second-line GBM.

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 astrocytoma, 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. Avastin ® is approved as a second-line therapy for adult GBM based on response. However, most recurrent patients receiving Avastin ® ultimately relapse, and the median OS for these second-line patients is approximately eight to nine months. Currently, no therapies have been approved for third-line treatment of GBM, which carries a median OS of three to four months.

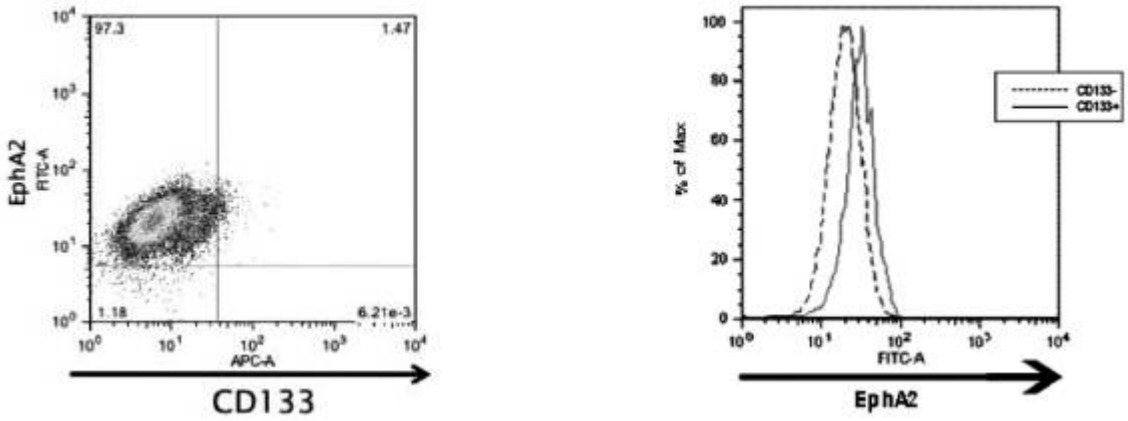
Pediatric HGG, which includes non-brainstem HGG and BSG, is a highly malignant disease with very poor outcomes. The annual incidence of pediatric HGG is approximately 1,600 to 2,000 in the United States and approximately 3,400 in Europe. No therapy has been shown to have a favorable outcome in this population 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 short synthetic peptides that correspond to epitopes of the brain cancer targets IL-13Rα2 and EphA2. The IL-13Rα2 synthetic peptide is a mutant specifically designed to be highly immunogenic to amplify the vaccine's anti-tumor immune response.

Both the IL-13Rα2 and EphA2 targets are overexpressed on brain cancer cells. We determined that EphA2 was overexpressed, not only on brain tumor bulk, but also on brain CSCs. In particular, EphA2 was found to be overexpressed on the surface of brain cancer cells expressing CD133, a marker of brain CSCs.

EphA2 Over-Expression on CSCs of GBM By Flow Cytometry
(Stemline Therapeutics, Inc.; unpublished data)



SL-701, like other cancer vaccines, is combined with additional elements designed to promote an immune response, including 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 injected into the patient concurrently with SL-701 administration.

Immune response analyses, including enzyme-linked immunosorbent spot, or ELISPOT, and tetramer assays, were used to assess peripheral blood immune responses of patients to SL-701 administration.

Completed Phase 1/2 Clinical Trial — Pediatric Glioma

In a completed Phase 1/2 trial, SL-701 was evaluated in pediatric patients with glioma. We refer to this trial as the 701 Ped-G Study. The 701 Ped-G Study was undertaken in 27 HLA-A2+ pediatric patients with glioma. Sixteen of these patients had newly diagnosed brainstem glioma, or BSG, five 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 SL-701 in the right or left upper arms associated with intact draining auxiliary lymph nodes once every three weeks for up to 24 weeks with a separate concurrent injection of an adjuvant. The 701 Ped-G Study was a single-arm trial whose objectives were to determine the general safety, dosage and efficacy of SL-701. Accordingly, all patients were treated with SL-701 and there was no comparative arm of patients receiving a control treatment or placebo. As such, this trial design was not intended to generate prospective comparative results conducive to calculating their statistical significance and, accordingly, no p-values were generated.

Well-Tolerated at Clinically Active Doses

SL-701 was well-tolerated at clinically active doses. Adverse effects included local injection site reactions and low grade fevers in almost all patients, which were generally mild and controlled with analgesics.

Clinical Activity

In the 701 Ped-G Study, SL-701 demonstrated single agent clinical activity. Eighty-six percent (19/22) 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. 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 four 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 SL-701 treatment stimulated the immune system in a highly specific fashion.

Completed Phase 1/2 Clinical Trial — Adult, Recurrent, High-Grade Glioma

In a completed Phase 1/2 clinical trial, SL-701 was evaluated in adult patients with recurrent or refractory HGG. We refer to this study as the 701 Adult-RHGG Study. 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 with Avastin ®. SL-701 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, in which SL-701 was administered to patients and demonstrated robust antitumor 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 SL-701. Accordingly, all patients were treated with SL-701 and there was no comparative arm of patients receiving a control treatment or placebo. We instead evaluated the results against published historical data for available therapies in the same indication. As such, this trial design was not intended to generate prospective comparative results conducive to calculating their statistical significance and, accordingly, no p-values were generated.

Well-Tolerated at Clinically Active Doses

SL-701 was well-tolerated at clinically active doses. 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 Avastin ®, which are mainstay therapies used to treat adult HGG. We believe that this implies that the development of SL-701-based combination regimens will likely be feasible.

Clinical Activity

In the 701 Adult-RHGG Study, SL-701 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 SL-701 treatment, this patient’s gadolinium enhanced tumor mass

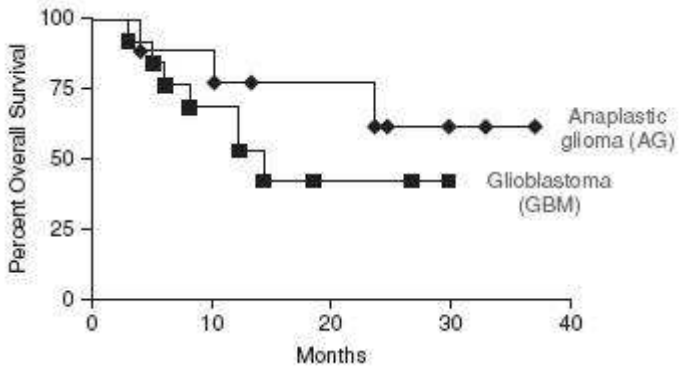
disappeared, and the patient was determined to have sustained a durable CR that exceeded 23 months. Notably, in this patient there was also a significant increase in target-specific T-cells by week 29 as determined by a tetramer assay, consistent with a positive immune response to SL-701. A recurrent 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-SL-701 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 SL-701 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, and that this patient experienced a tumor pseudoprogression prior to the PR. This activity is consistent with the proposed mechanism of action of SL-701 wherein SL-701 induces the immune system, and cytotoxic T cell in particular, to home to the tumor by crossing the blood-brain barrier and then attacking the 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 SL-701 treatment stimulated the immune system in a highly specific fashion.

Survival Benefit

SL-701 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 SL-701, 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 SL-701 also experienced an improvement in OS compared with historical results.

Kaplan-Meier Survival Curve
of Recurrent or Refractory Adult HGG Patients Treated with SL-701
(Okada et al., Journal of Clinical Oncology 2011; 29:330-336)



Low-Grade Glioma Trial in Adult Patients

There is currently a study of SL-701 open in adult patients with LGG. 24 HLA-A2+ patients have been enrolled, including 13 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 SL-701 via direct subcutaneous injection every three weeks for up to eight courses. SL-701 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. Although a thorough evaluation of progression-free survival requires a longer observation period, among 17 patients who completed eight courses, 10 had stable disease. Dr. Hideho Okada of the University of Pittsburgh School of Medicine is the sponsor of the study, and Dr. Frank Lieberman of the University of Pittsburgh School of Medicine is the principal investigator.

Planned Phase 2b Clinical Trials and Regulatory Strategy

Pediatric Trial

We have collaborated with the Pediatric Brain Tumor Consortium, or PBTC, to apply for funding from the National Cancer Institute, or NCI, for the SL-701 trial in pediatric patients with malignant brainstem and non-brainstem glioma. The letter of intent that we and the PBTC submitted for the pediatric trial was approved by the NCI in October 2012. We must still obtain approval of the full protocol, which we are pursuing now, before this pediatric trial may begin. If the final protocol is approved, we and the PBTC plan to oversee execution of the trial and management of clinical trial sites. In addition, we plan to provide SL-701 drug supply and submit a corporate IND. The PBTC was formed by the NCI and consists of participating academic centers and children's hospitals that are responsible for the diagnosis and treatment of children with primary brain tumors in the United States. The PBTC's primary objective is to rapidly conduct novel clinical evaluations of new therapeutic drugs and treatment strategies in pediatric patients from infancy to 21 years of age with primary central nervous systems tumors.

Adult Trial

We also plan to initiate a Phase 2b clinical trial in adult patients with second-line GBM. In this trial, which may include up to 30 patients, we plan to administer SL-701 in combination with the standard of care in this indication, which currently is bevacizumab (Avastin ®).

The Cancer Stem Cell Opportunity

Limitations of Current Cancer Therapies

According to the National Cancer Institute, cancer is the second leading cause of death in the United States and is responsible for nearly one quarter of all deaths in the United States. The National Institutes of Health estimated that the total cost of treating cancer in 2010 was $125 billion. Current cancer treatments, which often include chemotherapy and radiation as well as newer targeted therapies, have shown a limited overall survival benefit when used in advanced stages of the most common cancers. Moreover, the impact of current treatments on many other cancers, including AML, brain malignancies and multiple other cancer types has also been quite small, if any. We believe that it is becoming increasingly accepted within the oncology field, based on a progressively increasing body of supportive data, that a major reason for such failures is that available therapeutics fail to effectively eliminate CSCs, which continue to repopulate the cancer despite these standard treatments.

Cancer Stem Cell Overview

The field of CSCs is a rapidly emerging new area of cancer biology that we believe may fundamentally alter the approach to oncology drug development. CSCs comprise a highly malignant, self-renewing subpopulation of cancer cells within a tumor, often slow-growing, that is both highly tumorigenic, or tumor-producing, as well as resistant to traditional anti-cancer therapies relative to the rest of the largely fast-growing tumor bulk to which it gives rise.

CSCs have been identified in virtually all of the major tumor types including most of the common solid and hematologic cancer types. As shown in several examples below, researchers have identified numerous tumor types that harbor CSCs, including leukemia and cancers of the brain, breast, colon, prostate, pancreas, and others.

Examples of Tumor Types with CSCs

Tumor Types Harboring CSCs	Published Studies
Acute myeloid leukemia	Bonnet et al. *Nat Med* 1997; 3:730-737
Breast cancer	Al-Hajj et al. *PNAS* 2003; 100:3983-3988
Brain cancer	Singh et al. *Nature* 2004; 432:396-401
Acute lymphoblastic leukemia	Cox et al. *Blood* 2004; 104:2919-2925
Myeloma	Matsui et al. *Blood* 2004; 103:2332-2336
Chronic myeloid leukemia	Eisterer et al. *Leukemia* 2005; 19:435-441
Prostate cancer	Collins et al. *Cancer Res* 2005; 65:10946-10951
Lung cancer	Kim et al. *Cell* 2005; 121:823-835
Melanoma	Fang et al. *Cancer Res* 2005; 65:9328-9337
Ovarian cancer	Bapat et al. *Cancer Res* 2005; 65:3025-3029
Pancreatic cancer	Li et al. *Cancer Res* 2007; 67:1030-1037
Myelodysplastic syndrome	Nilsson et al. *Blood* 2007; 110:3005-3014
Liver cancer	Ma et al. *Oncogene* 2008; 27:1749-1758
Colon cancer	O'Brien et al. *Nature* 2007; 445:106-110
Bladder cancer	He et al. *Stem Cells* 2009; 27:1487-1495

CSCs are Tumorigenic

CSCs are a small subpopulation of highly malignant cells within a tumor that many within the oncology field believe are responsible for the tumorigenicity, meaning the source of growth, of the entire cancer. CSCs typically comprise approximately 1% to 5% of the entire cancer and give rise to, or "seed", the tumor bulk that comprises the remaining $\geq$ 95% of the tumor. In particular, isolated CSCs, not tumor bulk, have been shown capable of reconstituting the entire tumor anew when transplanted into immunocompromised mice and, importantly, are able to do so upon repeated serial retransplantation.

CSCs are Relatively Resistant to Traditional Therapies

In addition to being highly tumorigenic, CSCs are also resistant, relative to tumor bulk, to conventional anti-cancer therapies. This may be due to the many challenging characteristics of CSCs, including slow growth, presence of multi-drug resistance proteins, anti-cell death mechanisms, and increased activity of cellular mechanisms that repair damaged DNA. As shown in several examples below, researchers have shown that CSCs are resistant to chemotherapy, radiation, or targeted therapy relative to tumor bulk.

Examples of CSC Resistance to Traditional Therapies

CSC Type	Resistance to Therapy	Published Studies
Acute leukemia	Daunorubicin, Mitoxantrone	Wulf et al. *Blood* 2001; 98:1166-1173
Acute leukemia	AraC	Guzman et al., *Blood* 2001; 98:2301-2307
Brain cancer	BCNU	Kang and Kang, *Stem Cells Dev* 2007; 16:837-847
Brain cancer	Radiation	Bao et al. *Nature* 2006; 444:756-760
Breast cancer	Radiation	Phillips et al. *JNCI* 2006; 98:1777-1785
Chronic leukemia	Gleevec®	Graham et al. *Blood* 2002; 99:319-325
Colon cancer	5-FU, Oxaliplatin	Todaro et al. *Cell Stem Cell* 2007; 1:389-402
Liver cancer	Doxorubicin, 5-FU	Ma et al. *Oncogene* 2008; 27:1749-1758
Lung cancer	Cisplatin	Bertolini et al. *PNAS* 2009; 106:16281-16286
Myeloma	Velcade®	Matsui et al. *Cancer Res* 2008; 68:190-197
Pancreatic cancer	Gemcitabine	Hermann et al. *Cell Cycle* 2008; 7:188-193

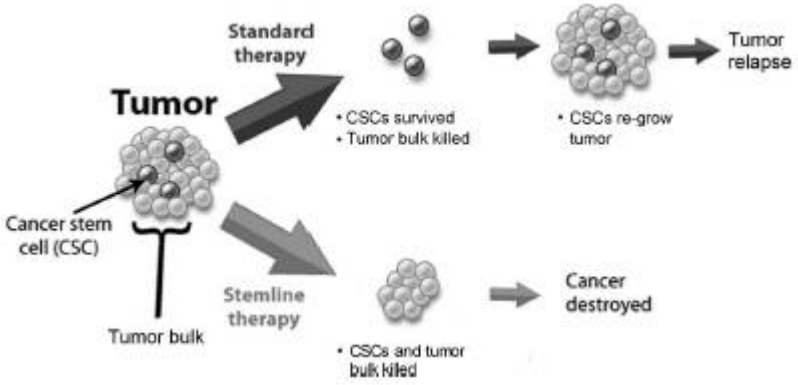
Not only have CSCs been shown to resist traditional therapies, but in some cases CSCs have also been shown to increase, as a percentage of total tumor cells, as a result of exposure to a traditional therapy. For example, as described by Bao et al. in *Nature* in 2006, CSCs of brain tumors increase as a percentage of the entire cancer following radiation treatment. Similarly, as shown by Hermann et al. in *Cell Stem Cell* in 2007, pancreatic CSCs increase following gemcitabine treatment in *in vivo* xenograft models.

CSCs Correlate with Prognosis

Consistent with their pivotal role in the development of tumors and relapse, higher amounts of CSCs in patient tumors as a percentage of their entire cancer have been shown to correlate with poor prognosis. For example, CSC fractions greater than 3.5% and 1% of the entire cancer correlate with poor survival outcomes in patients with AML and brain cancer, respectively, as shown by van Rhenen et al. in *Clinical Cancer Research* in 2005 (for AML) and Zeppernick et al. in *Clinical Cancer Research* in 2008 (for brain cancer).

Stemline's Anti-CSC Drug Development Opportunity

While standard therapies may initially shrink tumors by targeting the tumor bulk, we believe it is increasingly accepted within the oncology field that the failure of these therapies to eradicate CSCs is a major contributor to treatment failure, tumor relapse and poor survival. Accordingly, we believe that targeting CSCs, in addition to 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.



Since our inception, we have leveraged our knowledge of CSCs to anticipate and establish a leadership position in this new field of oncology. During this time, we have developed or strategically in-licensed key intellectual property, built and validated a drug discovery platform, and developed clinically active drug candidates. We believe that our early and comprehensive effort to develop the next generation of oncology therapeutics that target CSCs, as well as the tumor bulk, provides us with a significant competitive advantage.

Our Platform Technologies

We have developed an innovative platform technology, called StemScreen ®, currently consisting of StemScreen ® -1 and StemScreen ® -2, for the identification of novel CSC-directed compounds. This platform contrasts with traditional drug discovery methods in oncology that have been designed to identify compounds that target tumor bulk, not CSCs. StemScreen ® -1 is a technology developed to discover CSC-targeted compounds and involves the isolation of CSCs, the discovery of potential CSC targets through CSC gene expression analysis, and the identification and validation of compounds that impact candidate CSC targets. 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. We believe that this approach represents a major technological advance in oncology drug discovery. We have utilized StemScreen ® to discover several of our product candidates. We believe that this robust platform will be instrumental in the discovery of additional new therapies targeting a wide range of cancer types.

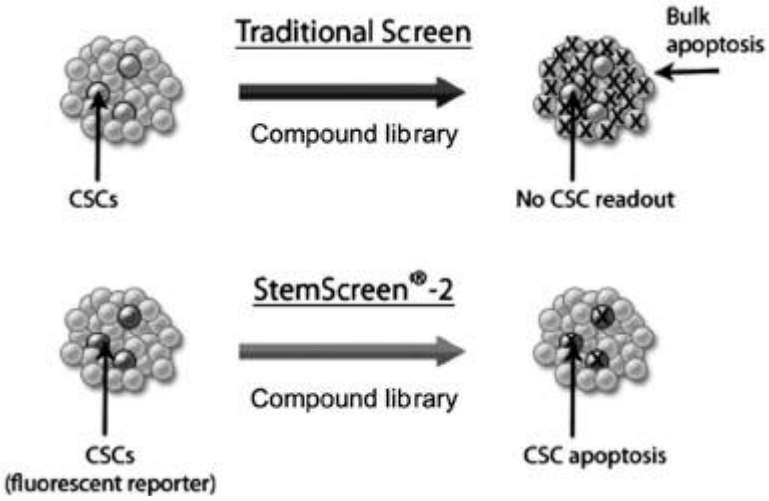
StemScreen ® -1

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. These include SL-201, SL-301, and SL-601. In addition, SL-401 demonstrated activity against CSCs as determined by both an *in vitro* colony formation and *in vivo* animal implantation assay, thereby validating certain StemScreen ® -1 anti-CSC assays.

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, covered by intellectual property controlled by Stemline, 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, 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.

StemScreen ® -2 also allows for further optimization, miniaturization, and screening in a high throughput manner for drug candidates with anti-CSC activity from large libraries of chemical or biologic compounds.

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 plan to further optimize StemScreen ® -2 for larger scale screening as well as expand its applicability for use in a broad range of tumor types either alone and/or in collaboration with a strategic partner.

Preclinical Pipeline

Stemline has assembled 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 discovery via our proprietary platforms as well as through in-licensing of certain key intellectual property.

We have also in-licensed certain 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, any or all of these mAbs.

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 13 issued patents and more than 30 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 consist of an issued U.S. patent (U.S. Patent 7,763,242) covering a method of treating MDS that expires in 2027 and pending U.S. and foreign applications directed to methods of using SL-401 to treat MDS, AML and other diseases that, if issued, would also expire in 2027. In addition, we have filed U.S. and foreign patent applications for the method of using SL-401 to treat MDS and AML, although there can be no assurances that such patents will be issued. In addition to patent protections, we also have the exclusivity afforded by the FDA's orphan designation of SL-401 for the treatment of AML and by the provisions of the Biologics Price Competition and Innovation Act of 2009. We plan to seek orphan designation from the FDA for SL-401 for the treatment of BPDCN. 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-13Rα2, and a non-exclusive worldwide license to SL-701 component, EphA2. These patent rights consist of an issued U.S. composition of matter patent (U.S. Patent 7,612,162) directed to an immunogenic mutant IL-13Rα2 peptide expiring in 2025 and issued U.S. method of use patent (U.S. Patents 7,297,337 and 8,114,407) directed to the use of EphA2 peptides used in SL-701 expiring in 2025 and 2024, respectively. We also have pending patent applications directed to methods of using SL-701 to treat certain diseases, which if issued would provide additional protection in the United States and certain non-U.S. territories and would expire in 2025.

We also in-licensed, our own, exclusive patent rights in the U.S. and abroad to our preclinical programs. For example, we in-licensed exclusive rights to SL-101, an antibody-based compound targeting CD123.

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;

- 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; and other pending applications. Patent protection, to the extent it issues, 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 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 have patent litigation thrust upon us. 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. 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 up, 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 $0.7 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 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-13Rα2 peptide (SL-701 component)

In September 2009, we entered into an exclusive license agreement with the University of Pittsburgh, or the University, for the composition of matter, and use with other components, of a proprietary immunogenic mutant analog peptide of IL-13Rα2, an active ingredient of SL-701, our brain cancer 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 $75,000 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-13Rα2 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-13Rα2 peptide license agreement with the University.

We must pay the University an initial license fee, and will pay the University annual license maintenance fees until the net sales of a licensed product exceed a specified amount. To date, we have paid an aggregate of approximately $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 from third parties to commercialize the licensed products. We must also pay a minimum annual royalty following the first commercial sale of a licensed product, but only to the extent the minimum annual royalty amount is greater than the annual royalty otherwise due.

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

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

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

In March 2012, we entered into a non-exclusive license agreement with the University. Pursuant to the agreement, we acquired a non-exclusive, worldwide license to use and reference certain know-how, information and data that is contained in the INDs covering the clinical trials of SL-701 that were conducted by the University for the development, manufacture, regulatory approval and commercialization of pharmaceutical products. We may grant sublicenses in conjunction with a sublicense to a permitted sublicensee under the exclusive IL-13Rα2 peptide license agreement with the University.

We paid the University an initial license fee, as well as payments following a regulatory milestone. To date, we have paid an aggregate of approximately $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-13Rα2 peptide license agreement with the University, or (iii) if we cease to carry out our business or become bankrupt or insolvent. We may terminate the agreement at any time prior to incorporating or referencing the data or University INDs, after a specified number of days following written notice. We may assign the agreement to an affiliate of

ours, a purchaser of all or substantially all of our assets or in connection with a merger, change in control or similar transaction by us.

Cambridge University Technical Services Limited

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

In September 2004, we entered into a license agreement with Cambridge University Technical Services Limited, or CUTS, relating to our StemScreen ® platform technology. Under the agreement, we acquired an exclusive, royalty-bearing, worldwide license under patent rights owned by CUTS to develop, manufacture, have manufactured, use, sell, offer to sell, market, have marketed, import, have imported, export and have exported products covered by the patent rights, including a platform technology to discover and screen for compounds that target CSCs. The patent rights exclusively licensed to us under the agreement are described in more detail above under "Business — Patents and Proprietary Rights." The license is subject to certain rights retained by CUTS for academic research and teaching. We also acquired a non-exclusive, worldwide license to know-how related to the licensed patent rights. The agreement provides us with full sublicensing rights. Under the agreement, we paid an upfront license fee and are obligated to make milestone payments of up to an aggregate of $1,700,000 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, 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, Dainippon Sumitomo Pharma Co. Ltd., Geron Corp., GlaxoSmithKline plc, ImmunoCellular Therapeutics, Ltd, 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 AML, 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., 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., 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 limited number of drugs approved for the treatment of adult AML, and these include the traditional chemotherapies cytarabine, daunorubicin, and other anthracyclines which have been marketed for many years and are both currently available in generic formulations. There are a number of companies working to develop new treatments for AML, including Cyclacel Pharmaceuticals, Inc., Sunesis Pharmaceuticals Inc., Genzyme Corporation (now a Sanofi company), Clavis Pharma ASA, Ambit Biosciences Corporation, Celgene Corporation, Eisai Co. Ltd. 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.

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. 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.

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.

U.S. Review and Approval Processes

The results of product development, preclinical studies and clinical trials, along with descriptions of the manufacturing process, analytical tests conducted on the chemistry of the 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 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. Unless otherwise required by regulation, PREA does not apply to any drug or biologic for an indication for which orphan designation has been granted.

The FDA 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 cGMP. 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.

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 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 combination of the product and the specific indication for which it is being studied. Unique to a Fast Track product, the FDA may consider for review sections of the NDA or BLA on a rolling basis before the complete application is submitted, if the sponsor provides a schedule for the submission of the sections of the NDA or BLA, the FDA agrees to accept sections of the NDA or BLA and determines that the schedule is acceptable, and 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. 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.

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 costs and continuing inspections over a period of many years, and possible withdrawal of the product from the market. 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 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 a budget proposal President Obama submitted to Congress in 2012, 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 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 ever 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 (including the Indian Health Service) designated entities 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.

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.

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 health care 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 health care 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. To date, all drug substance and drug product for SL-401 and SL-701 have been manufactured by our academic collaborators. We plan to work with qualified third-party contract manufacturers to produce sufficient quantities of SL-401 and SL-701 for our contemplated clinical trials and potential commercialization. Our manufacturing programs are being developed by our manufacturing team, which is comprised of full-time employees and consultants with experience manufacturing protein biologics and peptides and developing drug product formulations.

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. The initial supply of SL-401 that was used for the investigator-sponsored Phase 1/2 clinical trial was manufactured at Wake Forest University. We have optimized the plasmid vector and developed the fermentation and purification steps of our manufacturing process at third-party contract research organizations. We are currently preparing to transfer this technology to a third-party contract manufacturer with expertise in bacterial fermentation, where it will be process optimized and scaled-up for production. We cannot assure that the cGMP manufactured batches will be sufficient in quality and quantity to enable their use in corporate-sponsored clinical trials and commercialization.

SL-701 Manufacturing and Supply

SL-701 is a peptide vaccine that is comprised of short synthetic peptides. SL-701 can be administered as a peptide emulsion by direct subcutaneous injection into the patient, or by *ex vivo* delivery onto autologous dendritic cells which are then reinfused into the patient. We plan to focus largely on developing the direct peptide injection delivery method of SL-701 for future clinical trials and commercialization. Each of the component peptides of SL-701 is manufactured individually by solid-phase synthesis using standard Fmoc chemistry. We plan to mix and formulate the individual peptides to generate the

SL-701 drug product. SL-701 used in the investigator-sponsored Phase 1/2 trials was manufactured at a third-party contract manufacturer. We plan to select a qualified third-party contract manufacturer to produce SL-701 supply for our clinical trials and 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 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 $1,329,509 in 2010, $1,629,026 in 2011, and $3,415,668 in 2012. “Research and development expenses” consist of costs associated with the development of our product candidates and our platform technology, which include: clinical trial 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 December 31, 2012, we had eight full-time employees, four 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 previously submitted a biologics license application, or BLA, or a new drug application or NDA, to the FDA, or similar drug approval filings to comparable foreign authorities, for any product candidate. We cannot be certain that any of our product candidates will be successful in clinical trials or receive regulatory approval. 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 approval is similar in other countries, to obtain separate regulatory 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.

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 time period to complete. Its outcome is inherently uncertain. In addition, failure can occur at any time during clinical development. 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;
- our inability to manufacture, or obtain from third parties, adequate supply of drug product sufficient to complete our preclinical studies and clinical trials;
- the FDA requiring alterations to any of our study designs, our preclinical strategy or our manufacturing plans;
- delays in patient enrollment, and variability in the number and types of patients available for clinical trials, 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
- varying interpretations of data by the FDA and 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, for such trial, or by the FDA or other regulatory authorities. Such authorities may impose such a suspension or termination due to a number of factors, including 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 previously initiated or completed a corporate-sponsored clinical trial. Consequently, we may not have the necessary capabilities, including adequate staffing, to successfully manage the execution and completion of any clinical trials we initiate, including our planned clinical trials of SL-401 and SL-701, in a way that leads to our obtaining marketing approval for our product candidates in a timely manner, or at all.

In the event we are able to conduct a 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 complete response rate, the FDA may refuse to approve a BLA based on such pivotal trial. The FDA may require 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, and our ability to commence product sales and generate product revenues from any of our product candidates will be delayed. 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 clinical trials of product candidates may not be predictive of the results of later-stage clinical trials. Product candidates in later stages of clinical trials may fail to show the desired safety and efficacy results despite having progressed through preclinical studies and initial clinical trials. Many companies in the biopharmaceutical industry have suffered significant setbacks in advanced 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 the planned clinical trials compared to the completed clinical trials. As product candidates are developed through preclinical to early and late stage clinical trials towards approval and commercialization, it is customary that various aspects of the development program, such as manufacturing and methods of administration, 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 to initiate a dose escalation study and, if unsuccessful, could require us to complete additional preclinical or clinical studies of our product candidates.

- We plan to change the treatment regimen of SL-401 to a multi-cycle treatment regimen, in which the patient receives more than one treatment cycle, rather than a single-cycle treatment as used in the completed clinical trials. Although we anticipate that patients receiving multiple cycles of SL-401 will derive even greater clinical benefit than from a single cycle, there is always the risk of an unforeseen toxicity arising from multiple cycles.

- We plan to develop SL-701 as an injection delivered under the skin, or subcutaneously, in future trials. The 701 Ped-G 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, are removed from the patient, exposed to SL-701, and then re-injected into or near a lymph node of the patient (intra/peri-nodally). Thus, our plan continues the pediatric method and represents a change in the adult method.

- We plan to manufacture and formulate SL-701 as a mixture of IL-13Rα2, EphA2 and a helper peptide. In the 701 Ped-G and 701 Adult-RHGG Studies, SL-701 (which is comprised of IL-13Rα2 and EphA2) was mixed with additional peptides, including YKL-40 and GP-100 peptides in the adult study, and surviving peptide in the pediatric study. Given the clinical anti-tumor activity observed in both trials, we believe that the IL-13Rα2 and EphA2 peptides, the common feature of both trials, represent the active components. Thus, we believe that SL-701 need not be mixed with any additional peptides for clinical activity. Accordingly, while we will continue to evaluate the scientific merit of combining SL-701 with additional peptides, we plan to advance SL-701 into future trials without additional peptides.

- We plan to change the administration regimen of SL-701 to include a more commercially available and viable adjuvant than the adjuvant used in the completed clinical trials. An adjuvant is a substance administered to a patient to potentially help enhance the patient’s immune response to a vaccine.

- In some of our future trials, we may combine SL-401 or SL-701 with other therapies such as chemotherapy or anti-angiogenic therapy. We have not yet tested these combinations. In our planned Phase 2b clinical trial of SL-701 in adult second-line GBM, we plan to administer SL-701 in combination with the standard of care in this indication, which currently is bevacizumab (Avastin ®). Given that there do not appear to be overlapping toxicities between SL-701 and bevacizumab, we do not expect there to be unexpected side effects. We plan to conduct early safety analyses in our trial to confirm this conclusion.

Any of these changes could make the timing, including initiation, or the results of our planned clinical trials or other future clinical trials we may initiate 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 in BPDCN and SL-701 is being developed in pediatric brain cancer, both of which represent ultra-orphan indications for which there are very limited independently reported data on annual incidences. If the incidences are very low, this could significantly delay patient accrual to our SL-401 BPDCN program and SL-701 pediatric brain cancer program.

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 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 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.

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 approval 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, 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 products. 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 existence and importance of CSCs as an underlying cause of tumor initiation, propagation, recurrence, 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 products 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 commercially viable drugs to treat human patients with cancer.

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 a substantial amount of our efforts will focus 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. 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 likely would 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 obligations and continued regulatory review, which may result in significant additional expense. 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.

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, 2012 of $19.3 million. We do not know whether or when we will become profitable. To date, we have not commercialized any products or generated any revenues from product sales. Our losses have resulted principally from costs incurred in development and discovery activities. We anticipate that our operating losses will substantially increase over the next several years as we execute our plan to expand our discovery, research, development and potential commercialization activities. We believe that our existing cash, cash equivalents and marketable securities, together with the net proceeds from our initial public offering, will be sufficient to fund our operations and our capital expenditures for at least the next 24 months. 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.

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 substantial additional financing to achieve our goals, and a failure to obtain this necessary capital when needed could force us to delay, limit, reduce or terminate our product development or commercialization efforts. Specifically, in the case of our planned registration-directed randomized Phase 2b trial of SL-401 in third-line AML, we will require additional financing unless this trial is stopped early at an interim analysis.

Since our inception, most of our resources have been dedicated to the discovery, acquisition and preclinical and clinical development of our product candidates. In particular, we have expended and believe that we will continue to expend substantial resources for the foreseeable future developing our clinical 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 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, we expect to incur additional costs associated with operating as a public company.

We have no 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, we cannot reasonably estimate the actual amounts necessary to successfully complete the development and commercialization of our product candidates. 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 or debt financings or other sources, such as strategic partnerships and alliances and licensing arrangements. In addition, we may seek additional capital due to favorable market conditions or strategic considerations even if we believe we have sufficient funds for our current or future operating plans.

Our future capital requirements depend on many factors, including:

- the number and characteristics of the product candidates we pursue;

- the scope, progress, results and costs of researching and developing our product candidates, and conducting preclinical and clinical trials;
- the 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 of manufacturing our product candidates and any products we successfully commercialize;
- our ability to establish and maintain strategic partnerships, licensing or other arrangements and the financial terms of such agreements;
- our ability to obtain government funding for our planned clinical trial of SL-701 in pediatric patients;
- the costs involved in preparing, filing, prosecuting, maintaining, defending and enforcing patent claims, including litigation costs and the outcome of such litigation; and
- the timing, receipt and amount of sales of, or royalties on, our future products, if any.

Additional funds may not be available when we need them, on terms that are acceptable to us, or at all. If adequate funds are not available to us on a timely basis, we may be required to:

- delay, limit, reduce or terminate preclinical studies, clinical trials or other research and development activities for one or more of our product candidates; 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 the clinical development of SL-401 and SL-701 in their entirety. Specifically, in the case of our planned registration-directed randomized Phase 2b trial of SL-401 in third-line AML, we will require additional financing unless this trial is stopped early at an interim analysis.

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

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

As has been widely reported, global credit and financial markets have been experiencing extreme disruptions over the past several years, including severely diminished liquidity and credit availability, declines in consumer confidence, declines in economic growth, increases in unemployment rates, and uncertainty about economic stability. There can be no assurance that further deterioration in credit and financial markets and confidence in economic conditions will not occur. Our general business strategy may be adversely affected by the recent economic downturn and volatile business environment and

continued unpredictable and unstable market conditions. If the current equity and credit markets deteriorate further, or do not improve, 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; and
- manage our spending as costs and expenses increase due to drug discovery, preclinical development, clinical trials, regulatory approvals 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.

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, Dainippon Sumitomo Pharma Co. Ltd., Geron Corp., GlaxoSmithKline plc, ImmunoCellular Therapeutics, Ltd, 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 AML, 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., 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., 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 will 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, as well as other employees, consultants and scientific and medical collaborators. As of December 31, 2012, we had eight 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 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.

If our employees commit fraud or other misconduct, including noncompliance with regulatory standards and requirements and insider trading, 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 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 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 health care providers, health plans and health care 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 health care 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 may not comply with current or future statutes, regulations or case law involving applicable fraud and abuse or other healthcare laws and regulations. If our operations are found to be in violation of any of these laws or any other governmental regulations that may apply to us, we may be subject to significant civil, criminal and administrative penalties, damages, fines, exclusion from government funded healthcare programs, such as Medicare and Medicaid, and the curtailment or restructuring of our operations. If any of the physicians or other providers or entities with whom we expect to do business are found to be not in compliance with applicable laws, they may be subject to criminal, civil or administrative sanctions, including exclusions from government funded healthcare programs.

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

We 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 future products if and when they are approved, or enter into licensing or collaboration agreements to assist in the future development and commercialization of such products.

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; and

- unforeseen costs and expenses associated with creating an independent sales and marketing organization.

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.

Our commercial success depends upon attaining significant market acceptance of our product candidates, if approved, including SL-401 and SL-701, among physicians, patients, health care 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, health care 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 pay for and establish reimbursement levels. A primary trend in the U.S. healthcare industry and elsewhere is cost containment. Government authorities and third-party payors have attempted to control costs by limiting coverage and the amount of reimbursement for particular medications. Increasingly, third-party payors are requiring that drug companies provide them with predetermined discounts from list prices and are challenging the prices charged for medical products. We cannot be sure that reimbursement will be available for any product that we commercialize and, if reimbursement is available, the level of reimbursement. Reimbursement may impact the demand for, or the price of, any product candidate for which we obtain marketing approval. Obtaining reimbursement for our products may be particularly difficult because of the higher prices often associated with drugs administered under the supervision of a physician. If reimbursement is not available or is available only to limited levels, we may not be able to successfully commercialize any product candidate for which we obtain marketing approval.

There may be significant delays in obtaining reimbursement for newly approved drugs, and coverage may be more limited than the purposes for which the drug is approved by the FDA or similar regulatory authorities outside the United States. Moreover, eligibility for reimbursement does not imply that any drug will be paid for in all cases or at a rate that covers our costs, including research, development, manufacture, sale and distribution. Interim reimbursement levels for new drugs, if applicable, may also not be sufficient to cover our costs and may not be made permanent. Reimbursement rates may vary according to the use of the drug and the clinical setting in which it is used, may be based on reimbursement levels already set for lower cost drugs and may be incorporated into existing payments for other services. Net prices for drugs may be reduced by mandatory discounts or rebates required by government healthcare programs or private payors and by any future relaxation of laws that presently restrict imports of drugs from countries where they may be sold at lower prices than in the United States. Third-party payors often rely upon 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, potentially creating the opportunity for generic competition sooner than anticipated. Moreover, the extent to which a biosimilar, once approved, will be substituted for any one of our reference products in a way that is similar to traditional generic substitution for non-biological products is not yet clear, and will depend on a number of marketplace and regulatory factors that are still developing. In addition, a competitor could decide to forego the biosimilar route and submit a full BLA after completing its own preclinical studies and clinical trials. In such cases, any exclusivity to which we may be eligible under the BPCIA would not prevent the competitor from marketing its product as soon as it is approved.

Risks Related to Our Dependence on Third Parties

Third parties have conducted all clinical trials of SL-401 and SL-701 so far, and our ability to influence the design and conduct of such trials has been 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 the Scott and White Cancer Research Institute, MD Anderson Cancer Center, British Columbia Cancer Agency and Duke University have conducted and sponsored all clinical trials relating to SL-401 and faculty members at the University of Pittsburgh have conducted and sponsored all clinical trials relating to SL-701, in each case 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 corporate-sponsored trials, then our ability to design and conduct our planned corporate-sponsored clinical trials may be adversely affected. Additionally, the FDA may disagree with the sufficiency of our right of reference to the preclinical, manufacturing, or clinical data generated by these prior investigator-sponsored trials, or 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.

We intend to assume control over the clinical and regulatory development of SL-401 by either exercising our right under our agreement with Scott and White Memorial Hospital to have Scott and White transfer to us the existing IND for SL-401 or by filing our own IND for SL-401. We expect to either transfer the IND or file our own IND in the next twelve months. We intend to assume control over the clinical development of SL-701 by filing a corporate-sponsored IND, for which we may exercise our rights of reference under our agreements with the University of Pittsburgh with respect to the existing INDs for SL-701.

We plan to rely on government funding to complete our planned clinical trial of SL-701 in pediatric patients. If such funding is not available, we may be unable to complete this trial, which could harm our commercial prospects for SL-701.

We intend to rely on government funding to fund substantially all the costs of our planned clinical trial of SL-701 in pediatric patients with brainstem and non-brainstem glioma. We have collaborated with the Pediatric Brain Tumor Consortium, or PBTC, to apply for funding from the National Cancer Institute, or NCI, for this trial. The letter of intent that we and the PBTC submitted for the pediatric trial was approved by the NCI in October 2012. We must still obtain approval of the full protocol, which we are pursuing now, before this pediatric trial may begin. We anticipate requiring approximately $3 million to $5 million of funding from the NCI to complete this trial. The actual costs will vary based on a number of factors, including the degree of cost-savings that can be realized by the PBTC, as well as trial results and other factors.

Even if the full protocol for the pediatric trial receives approval, all government grants are typically subject to government appropriations. Government grants often contain provisions that allow for termination at the convenience of the government. Further, government grants are subject to complex federal guidelines and regulations. If federal agencies or regulatory authorities determine that we, or our planned pediatric trial of SL-701, do not qualify for these grants, we may be unable to complete this trial, which could harm our commercial prospects for SL-701 and delay our ability to commence product sales and generate product revenues from SL-701.

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 have less control over the timing and cost of these studies and the ability to recruit trial subjects than if we conducted these trials wholly by ourselves. Once we 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.

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 plan to work with third-party contract manufacturers to produce sufficient quantities of SL-401 and SL-701 on a timely basis for future clinical trials, preclinical testing and commercialization. If we are unable to arrange for such a third-party manufacturing source, 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. 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 have limited staffing and rely on our 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 any control over the process or timing of the acquisition of these materials by our manufacturers. Moreover, we currently do not have any agreements for the commercial production of these materials. 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 optimizing the manufacturing processes for SL-401 and SL-701 drug substance and drug product so that these product candidates may be produced in adequate quantities of adequate quality, and at an acceptable cost, to support our planned clinical trials and ultimate commercialization. Our manufacturers 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 manufacturers may not be able to manufacture our product candidates at a cost or in quantities or in a timely manner necessary to develop and commercialize them. If we successfully commercialize any of our drugs, we may be required to establish or access large-scale commercial manufacturing capabilities. In addition, assuming that our drug development pipeline increases and matures, we will have a greater need for clinical trial and commercial manufacturing capacity. To meet our projected needs for commercial manufacturing, third parties with whom we currently work will need to increase their scale of production or we will need to secure alternate suppliers.

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 United States 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 products 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 obtained a U.S. patent for the method of using SL-401 to treat MDS. In addition, we have filed U.S. and foreign patent applications for the method of using SL-401 to treat MDS and AML, although there can be no assurances that such patents will be issued over the prior art. Failure to obtain patents directed to the use of SL-401 to treat AML (or any other indication) 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 our mutant immunogenic IL-13Rα2 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 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.

Although we have various patent applications pending in the U.S. and abroad that we hope will result in additional protection for both SL-401 and SL-701, there can be no assurance that any of these applications will issue into a patent, or that if they issue, they will provide meaningful protection for SL-401 and SL-701. Our inability to obtain adequate patent protection for our product candidates or platform technology could adversely affect our business.

Issued patents covering one or more of our products 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 products, 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 novelty, obviousness 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 products 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 products 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 products, the use of our products, or our 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 as currently formulated. We may need to seek a license

with respect to one or more of these third party patents in order to commercialize SL-701. 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. For example, applications filed before November 29, 2000 and certain applications filed after that date that will not be filed outside the United States remain confidential until patents issue. Patent applications in the United States and elsewhere are published approximately 18 months after the earliest filing for which priority is claimed, with such earliest filing date being commonly referred to as the priority date. Therefore, 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 products, 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. 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.

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 products, programs, or intellectual property could be diminished. Accordingly, the market price of our common stock may decline.

SL-401 and SL-701 are protected by intellectual property exclusively licensed from academic institutions. If the licensors terminate the licenses or fail to maintain or enforce the underlying patents, our competitive position and market share will be harmed.

We are a party to several license agreements under which certain aspects of our business depend on patents and/or patent applications owned by other institutions. In particular, we hold exclusive licenses from Scott and White Memorial Hospital, or Scott and White, for SL-401 and three licenses, including an exclusive license, 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 to clinical trial data and information survives twenty years unless terminated earlier. We are likely to enter into additional license agreements as part of the development of our business in the future. Our 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 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 terminate the license agreements if we fail to meet specified milestones. If these in-licenses are terminated, or if the underlying patents fail to provide the intended market exclusivity, 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®. This platform is useful for identifying new potential product candidates. We have pending applications for StemScreen®, however, there is no guarantee that any of such pending patent applications, in the United States or elsewhere, will result in issued patents, and, even if patents eventually issue, there is no certainty that the claims in the eventual patents will have adequate scope to preserve our competitive position. Third parties might invent alternative technologies that would substitute for our platform technology while being outside the scope of the patents covering our platform technology. By successfully designing around our patented technology third parties could substantially weaken our competitive position in oncology research and development.

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

In addition to patents, we rely on trade secrets, technical know-how, and proprietary information concerning our business strategy in order to protect our competitive position in the field of oncology. In the course of our research and development activities and our business activities, we often rely on confidentiality agreements to protect our proprietary information. Such confidentiality agreements are used, for example, when we talk to vendors of laboratory or clinical development services or potential strategic partners. In addition, each of our employees is required to sign a confidentiality agreement upon joining our Company. We take steps to protect our proprietary information, and our confidentiality agreements are carefully drafted to protect our proprietary interests. Nevertheless, there can be no guarantee that an employee or an outside party will not make an unauthorized disclosure of our proprietary confidential information. This might happen intentionally or inadvertently. 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 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 products.

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, 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

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 stockholders beneficially own shares representing approximately 42% 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 stockholder meetings;
- authorize our board of directors to issue preferred stock without stockholder approval, which could be used to institute a “poison pill” that would work to dilute the stock ownership of a potential hostile acquirer, effectively preventing acquisitions that have not been approved by our board of directors; and
- require the approval of the holders of at least 75% of the votes that all our stockholders would be entitled to cast to amend or repeal certain provisions of our charter or bylaws.

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

We do not know whether a market will develop for our common stock or what the market price of our common stock will be and as a result it may be difficult for you to sell your shares of our common stock.

Prior to the completion of our initial public offer, there was no public market for our common stock. Although our common stock now trades on the NASDAQ Capital Market, an active trading market for our shares may not be sustained. It may be difficult for you to sell your shares without depressing the market price for the shares or at all. As a result of these and other factors, you may not be able to sell your shares of our common stock at or above the initial public offering price or at all. Further, an inactive market may also impair our ability to raise capital by selling shares of our common stock and may impair our ability to enter into strategic partnerships or acquire companies or products by using our shares of common stock as consideration.

We will incur increased 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 will incur significant legal, accounting and other expenses that we did not incur as a private company. We will be 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. We expect these rules and regulations to substantially increase our legal and financial compliance costs and to make some activities more time-consuming and costly. Our management and other personnel will need to 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 will be required to disclose changes made in our internal controls and procedures on a quarterly basis and our management will be 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.

If our stock price is volatile, our stockholders could incur substantial losses.

Our stock price is likely to be volatile. 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. As a result of this volatility, you may not be able to sell your common stock, if at all. The market price for our common stock may be influenced by many factors, including:

- our ability to commercialize our product candidates, if approved;
- 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;
- 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;
- our dependence on third parties, including CROs, clinical trial sponsors and clinical investigators;
- regulatory or legal developments in the United States and other countries;
- 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
- the other factors described in this “Risk Factors” section.

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.

A significant portion of our total outstanding shares are restricted from immediate resale but may be sold into the market in the near future, which could cause the market price of our common stock to drop significantly, even if our business is doing well.

Sales of a substantial number of shares of our common stock in the public market could occur at any time. These sales, or the perception in the market that the holders of a large number of shares intend to sell shares, could reduce the market price of

our common stock. We have outstanding 3,476,501 shares of common stock based on the number of shares outstanding as of December 31, 2012. Such shares are currently restricted as a result of securities laws and lock-up agreements.

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. Securities and industry analysts do not currently, and may never, publish research on our Company. If no or too few securities or industry analysts commence coverage of our Company, the trading price for our stock would likely be negatively impacted. In the event securities or industry analysts initiate coverage, if one or more of the analysts who cover us downgrade our stock or publish inaccurate or unfavorable research about our business, our stock price would likely decline. If one or more of these analysts cease coverage of our Company or fail to publish reports on us regularly, demand for our stock could decrease, which might cause our stock price and trading volume to decline.

Item 1B. Unresolved Staff Comments

None.

Item 2. Properties

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

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. As a result, we have not set forth quarterly information with respect to the high and low prices for our common stock for the two most recent fiscal years.

Holders

The number of record holders of our common stock as of March 27, 2013 was 48. 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, 2012.

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	1,819,839	$ 2.76	372,422
Restricted stock	34,507	N/A	—
Equity compensation plans not approved by security holders	—	—	—
Total	1,854,346	—	372,422

Common Stock Performance Graph

Not applicable.

Recent Sales of Unregistered Securities

Set forth below is information regarding securities sold by us during 2010, 2011 and 2012 that were not registered under the Securities Act of 1933, as amended, or the Securities Act. Also included is the consideration, if any, received by us for the securities and information relating to the section of the Securities Act, or rule of the Securities and Exchange Commission, under which exemption from registration was claimed.

On March 16, 2010, the Company entered into a Note Purchase Agreement with several investment funds (the “Pequot Funds”) that held the Company’s Series A preferred stock (the “Preferred Shares”) and NB Athyrium LLC. In exchange for 455,518 outstanding shares of the Preferred Shares, the Company paid $0.75 million of cash, issued 411,571 shares of its common stock valued at $1.2 million and issued a $1.25 million senior unsecured convertible note, represented by the 2.45% Convertible Note. See “Notes to the Financial Statements” for information regarding terms of conversion for the 2.45% Convertible Note.

On October 1, 2012, the Company agreed to issue to the representative of the underwriters in our initial public offering, warrants to purchase up to 99,529 shares of the Company’s common stock. The warrants are exercisable for cash or on a cashless basis at a price per share equal to $15.00.

No underwriters were involved in the foregoing sales of securities. The securities were issued to investors in reliance upon the exemption from the registration requirements of the Securities Act, as set forth in Section 4(2) under the Securities Act, including in some cases, Regulation D promulgated thereunder, relative to transactions by an issuer not involving any public offering, to the extent an exemption from such registration was required. All purchasers of securities described above represented to us in connection with their purchase that they were accredited investors and were acquiring the shares for their own account for investment purposes only and not with a view to, or for sale in connection with, any distribution thereof. The

purchasers received disclosures that the securities had not been registered under the Securities Act and that any resale must be made pursuant to a registration statement or an available exemption from such registration.

Purchase of Equity Securities

We have not purchase any of our registered equity securities during the period covered by this Annual Report on Form 10-K.

Use of Proceeds from Registered Securities

On January 31, 2013 we completed our 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 $32.5 million. Additionally, upon the closing of the IPO, our convertible debt, plus accrued interest thereon, was converted into 166,769 shares of common stock. The offering commenced on January 28, 2013 and did not terminate until the sale of all of the shares offered. None of the underwriting discounts and commissions or other offering expenses were incurred or paid to directors or officers of ours or their associates or to persons owning 10 percent or more of our common stock or to any affiliates of ours.

As of March 15, 2013, we have used approximately $3.1 million of the net proceeds primarily to fund the development of SL-401 and SL-701, to advance and expand the research and development of additional product candidates and for working capital, capital expenditures and other general corporate purposes. Following the offering, certain bonuses were paid to our officers and other employees in the amount of $976,323. There has been no material change in our planned use of the balance of the net proceeds from the offering as described in our final prospectus filed with the SEC pursuant to Rule 424(b) under the Securities Act.

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 statements of operations data for the years ended December 31, 2009, 2010, 2011 and 2012, and the balance sheet data as of December 31, 2010, 2011 and 2012 from our audited financial statement included in this Form 10-K. We have derived the statements of operations data for the years ended December 31, 2008 and the balance sheet data as of December 31, 2008 and 2009 from our financial statements not included in this Form 10-K. 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, 2012
	2008	2009	2010	2011	2012	
	(unaudited)					
Statement of operations data:						
Revenue	$ —	$ —	$ —	$ —	$ —	$ —
Operating expenses:						
Research and development	$ 1,016,702	$ 1,054,446	$ 1,329,509	$ 1,629,026	$ 3,376,962	$ 11,445,668
General and administrative	1,179,984	1,026,675	930,331	1,088,028	3,090,611	9,484,796
Total operating expenses	2,196,686	2,081,121	2,259,840	2,717,054	6,467,573	20,930,464
Loss from operations	(2,196,686)	(2,081,121)	(2,259,840)	(2,717,054)	(6,467,573)	(20,930,464)
Other income:	—	102,257	484,905	46,673	301,684	935,518
Other expense	—	—	—	(9,670)	(35)	(9,705)
Interest expense	—	—	(69,493)	(98,643)	(118,765)	(296,950)
Interest income	376,578	201,088	43,045	24,068	9,907	960,583
Net loss	$ (1,820,108)	$ (1,777,776)	$ (1,801,383)	$ (2,754,626)	$ (6,274,782)	$ (19,341,018)
Less: accretion of preferred stock dividends	(1,021,201)	(1,100,107)	(239,720)	—	—	(2,591,165)
Add: discount on redemption of preferred stock	—	—	12,171,765	—	—	12,171,765
Net (loss) / income attributable to common stockholders	$ (2,841,309)	$ (2,877,883)	$ 10,130,662	$ (2,754,626)	$ (6,274,782)	$ (9,760,418)
Net (loss) / income attributable to common stockholders per common share:						
Basic	$ (1.01)	$ (1.02)	$ 3.07	$ (0.80)	$ (1.82)	
Diluted	$ (1.01)	$ (1.02)	$ 2.81	$ (0.80)	$ (1.82)	
Weighted average number of common shares:						
Basic	2,824,647	2,824,647	3,298,793	3,441,995	3,441,995	
Diluted	2,824,647	2,824,647	3,607,030	3,441,995	3,441,995	

	As of December 31,				
	2008	2009	2010	2011	2012
	(unaudited)				
Balance sheet data:					
Cash and cash equivalents	$ 2,196,881	$ 9,236,395	$ 7,226,366	$ 5,829,886	$ 2,025,338
Total assets	$ 10,856,476	$ 9,329,341	$ 7,502,912	$ 6,453,096	$ 5,029,611
Long-term liabilities	$ —	$ —	$ 1,017,033	$ 1,665,346	$ 2,037,296
(Deficit)/earnings accumulated during development stage	$ (6,732,453)	$ (8,510,229)	$ 1,860,153	$ (894,473)	$ (7,169,255)
Total stockholders' (deficit)/equity	$ (3,355,509)	$ (6,162,215)	$ 5,851,561	$ 3,205,340	$ (2,508,420)

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 believe that we are developing a clinically advanced pipeline of anti-CSC therapeutics and that we hold a broad portfolio of CSC-focused intellectual property, establishing us as a leader in the CSC field. We are currently developing two clinical-stage product candidates, SL-401 and SL-701.

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 not generated any revenues and, to date, have funded our operations primarily through private sales of common stock and convertible preferred stock and issuances of convertible debt to our investors. From inception through December 31, 2012, we have received net proceeds of $3.8 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. Upon the completion of our initial public offering on January 31, 2013, we received net proceeds of $32.5 million, net of fees and expenses.

We have never been profitable and, from inception through December 31, 2012, our net losses from operations have been $19.3 million. Our net loss from operations was $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, upon the closing of the IPO, we recorded non-cash expense associated with the effect of the beneficial conversion charge of approximately $0.1 million that will be recorded upon the conversion of our convertible notes due 2017 and the recording of approximately $1.4 million of stock-based compensation expense associated with 573,424 options with a weighted average exercise price of $2.38 that fully vest upon the consummation of our initial public offering. In addition, we expect to record approximately $1.5 million of compensation expense relating to certain bonuses and salary increases payable upon continued employment and the occurrence of a specified financing, upon the consummation of our initial public offering. Further, 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 to date. 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. We expect that any revenue we generate will fluctuate from quarter to quarter as a result of the amount and timing of payments that we may recognize upon the sale of our products, to the extent that any products are successfully commercialized, and the amount and timing of fees, reimbursements, milestone and other payments received under any future licensing or collaboration arrangements. If we fail to complete the development of our product candidates in a timely manner or obtain regulatory approval for them, our ability to generate future revenue, and our results of operations and financial position, would be materially adversely affected.

Research and Development Expenses

The following table shows our research and development expenses for the years ended December 31, 2010 and 2011 and 2012:

	Year Ended December 31,		
	2010	2011	2012
Clinical (SL-401 and SL-701)	$ 1,226,738	$ 1,566,141	$ 3,292,724
Preclinical	102,771	62,885	84,238
Total	$ 1,329,509	$ 1,629,026	$ 3,376,962

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

- clinical trial 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 and 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. The clinical development and regulatory strategies for our lead product candidates are as follows:

- **SL-401.** We plan to complete a pivotal Phase 2b single-arm trial of SL-401 in patients with BPDCN. We also plan to advance SL-401 into a registration-directed randomized Phase 2b clinical trial to treat adult AML patients as a third-line treatment. We plan to use approximately $9 million for these programs.

- **SL-701.** We plan to advance SL-701 into a Phase 2b clinical trial to treat pediatric patients with malignant brainstem and non-brainstem glioma. We also plan to initiate a Phase 2b clinical trial of SL-701 in adult second-line glioblastoma multiforme, or GBM. We plan to use approximately $1 million, together with funding we are seeking to obtain from the National Cancer Institute, or NCI, for the pediatric trial, for these programs.

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;

- 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, operations, finance, investor relations, and business development functions. 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 increase in future periods to support increases in our research and development activities and as a result of 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 and cash equivalents.

Interest Expense

Interest expense consists primarily of cash and non-cash interest costs related to our 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.

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

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

Accrued Research and Development Expenses

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

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.

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. 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, 2012, we had U.S. federal net operating loss carryforwards of $14.7 million and research and development credits of $0.5 million which expire in 2023 through 2032.

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 filed a U.S. income tax return as well as 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.

Stock-Based Compensation

In accordance with ASC 718, *Stock Compensation* , we account for stock options issued to employees using a fair-value-based method, under which we measure the cost of employee services received in exchange for an award of equity

instruments, including stock options, based on the grant-date fair value of the award. The resulting cost is recognized for the awards expected to vest over the period during which an employee is required to provide service in exchange for the award, usually the vesting period.

In accordance with ASC 505-50, *Equity-Based Payments to Non-Employees* , we account for stock options issued to non-employees on a fair-value-based method as well; however, the fair value of the options granted to non-employees is remeasured each reporting period until the service is complete, and the resulting increase or decrease in value, if any, is recognized as income or loss during the period the related services are rendered.

The fair value of the stock options issued to employees and non-employees was estimated at each grant date using the Black-Scholes option-pricing model. One of the inputs to this model is the estimate of the fair value of the underlying common stock on the date of grant. The other inputs include an estimate of the expected volatility of the stock price, an option's expected term, the risk-free interest rate over the option's expected term, the option's exercise price, and our expectations regarding dividends. Stock-based compensation expense includes stock options granted to employees and non-employees and has been reported in our statements of operations as follows:

	Year Ended December 31,		
	2010	2011	2012
Research and development	$ 50,311	$ 79,955	$ 412,536
General and administrative	30,224	28,450	148,486
Total	$ 80,535	$ 108,405	$ 561,022

We do not have a history of market prices for our common stock because our stock was not publicly traded through December 31, 2012. We utilized the observable data for a group of public peer companies that grant options with substantially similar terms to assist in developing our volatility assumption. We derived our expected term assumption based on the simplified method, if applicable, which results in an expected term based on the midpoint between the vesting date and the contractual term of an option. The simplified method was chosen because we have limited historical option exercise experience because our Company was privately held. The expected term for options issued to non-employees was determined based on the contractual term of the awards. The weighted-average risk-free interest rate was based on a zero coupon U.S. Treasury instrument whose term was consistent with the expected life of the stock options. We have not paid and do not anticipate paying cash dividends on our shares of common stock; therefore, the expected dividend yield was assumed to be zero.

A summary of the significant assumptions used to estimate the fair value of employee and non-employee equity awards for the years ended December 31, 2010 and December 31, 2011 and December 31, 2012 is as follows:

	Year Ended December 31,		
	2010	2011	2012
Expected term	6.02	6.26	6.25
Risk-free interest rate	2.78%	2.66%	0.95%
Volatility	74.5%	72.9%	79.0%
Dividend yield	0%	0%	0%

If factors change and we employ different assumptions, stock-based compensation cost on future awards may differ significantly from what we have recorded in the past. Higher volatility and longer expected terms result in an increase to stock-based compensation determined at the date of grant. Future stock-based compensation cost and unrecognized stock-based compensation will increase to the extent that we grant additional equity awards to employees or we assume unvested equity awards in connection with acquisitions. If there are any modifications of the underlying unvested securities, we may be required to accelerate any remaining unearned stock-based compensation cost or incur incremental cost. Stock-based compensation cost affects our research and development, and selling, general, and administrative expenses.

The aggregate intrinsic value of all outstanding vested and unvested options to purchase shares of our common stock as of December 31, 2012 was $13.2 million based on a per share price of $10.00 for our common stock, the initial public offering price per share, with a weighted average exercise price of $2.76 per share.

We estimate our forfeiture rate based on an analysis of our actual forfeitures and will continue to evaluate the appropriateness of the forfeiture rate based on actual forfeiture experience, analysis of employee turnover behavior, and other factors. Changes in the estimated forfeiture rate can have a significant effect on reported stock-based compensation expense, because the cumulative effect of adjusting the rate for all expense amortization is recognized in the period the forfeiture estimate is changed. The effect of forfeiture adjustments during 2012, 2011 and 2010 was insignificant.

Significant Factors, Assumptions, and Methodologies Used in Estimating Fair Value of Common Stock

On March 26, 2009, our board of directors engaged in a review of the Company's valuation. The board of directors considered the status of the Company's product pipeline, the Company's StemScreen ® platform for identifying novel compounds that target and kill CSCs, the asset value of the Company (comprised of intellectual property rights and current cash on hand), the present value of future cash flows of the Company as a clinical and development stage company, the Company's capital structure, and the Company's acquisition potential. The board of directors also considered additional factors, including the Company's existing licensing and research agreements, intellectual property, funding prospects, the

funding prospects and valuations of similar companies, the ability of the management team and the Company's access to financing. Based on the foregoing review, the board of directors determined that the per share value of the Company's common stock was equal to $1.11 on March 26, 2009.

We also performed a valuation to estimate the fair value of our common stock for the options granted during the years ended December 31, 2011 and December 31, 2012. The per share exercise price, fair value of underlying shares and fair value of the option awards as of the respective dates of valuation are as follows:

Approval Date(1)	GAAP Measurement Date(2)	Number of Shares	Exercise price per share	Fair Value of Underlying Share of Common Stock	GAAP Measurement Amount ($)
March 8, 2011	March 8, 2011	224,067	$ 2.92	$ 3.10	$ 2.10
February 29, 2012	May 24, 2012	112,202	$ 3.30	$ 9.90	$ 8.18
March 5, 2012	May 24, 2012	81,814	$ 3.30	$ 9.90	$ 8.18
March 9, 2012	March 9, 2012	180,704	$ 3.30	$ 3.30	$ 2.20
March 9, 2012	May 24, 2012	247,108	$ 3.30	$ 9.90	$ 8.18

(1) The Approval Date is the date on which the board of directors authorized for issuance an option grant.

(2) The GAAP Measurement Date is the first date at which the key terms and conditions of the awards were communicated to the recipient. The GAAP Measurement Date occurs subsequent to the Approval Date due to timing of the notification of the award's approval by the board of directors to the recipient of the award.

The exercise prices for options granted were set by our board of directors, the members of which have extensive experience in the life sciences industry, based on the group's determination of the fair market value of our common stock at the time the grants were authorized. The board of directors considered the status of the Company's product pipeline and the progress since the last valuation date, the Company's StemScreen ® platform for identifying novel compounds that target and kill CSCs, the asset value of the Company (comprised of intellectual property rights and current cash on hand, which was less than the previous valuation), the present value of future cash flows of the Company as a clinical and development stage company, the Company's capital structure (including the repurchase during 2010 of all of its outstanding preferred stock and the removal of the corresponding liquidation preference), the valuation of comparable companies and the Company's acquisition potential. The board of directors also considered additional factors, including the Company's existing licensing and research agreements, intellectual property, funding prospects, the funding prospects and valuations of similar companies, the growth prospects of the biopharmaceutical industry in general and oncology companies focused on CSC targets specifically, expected regulatory and commercial hurdles to commercializing or licensing the Company's clinical candidates, the ability of the management team and the Company's access to financing. Based on the foregoing review, the board of directors determined that the fair market value of the common stock underlying options to purchase 224,067 shares granted on March 8, 2011 was determined to be $2.92 per share at the time of grant.

However, in connection with the preparation of the financial statements for a public offering, we performed a retrospective determination of fair value for accounting purposes of our common stock underlying stock options utilizing a combination of valuation methods described in the AICPA Technical Practice Aid, *Valuation of Privately-Held-Company Equity Securities Issued as Compensation* , or the Practice Aid, using a more sophisticated method to determine fair market value.

Determining the fair value of the common stock of a private enterprise requires complex and subjective judgments. Our retrospective estimate of enterprise value at March 31, 2010, March 31, 2011 and January 30, 2012 used results from both the income approach and the market approach.

Under the income approach, our enterprise value was based on the present value of our forecasted operating results. Our revenue forecasts were based on our estimates of expected annual growth rates following the anticipated commercial launch of our lead product candidates SL-401 and SL-701. Estimated operating expenses were based on our internal assumptions, including continuing research, development activities for SL-401 and SL-701 and other clinical and preclinical product candidates and our platform technology, and preparation and ongoing support for the commercialization of our lead product candidates. The assumptions underlying the estimates are consistent with our business plan. The risks associated with achieving our forecasts were assessed in selecting the appropriate discount rates, which were approximately 25%.

Under the market approach, our estimated enterprise value was developed based on a comparison of pre-money initial public offering, or IPO, values of recent biotechnology and emerging pharmaceutical companies at a similar stage of development to ours.

Once our enterprise value was established, the enterprise value was allocated to the different classes of equity instruments. Our board of directors engaged in a retrospective review during which we used the probability weighted expected returns, or PWER, method to allocate our enterprise value to our common stock. Under the PWER method, the value of common stock is estimated based upon an analysis of future values for the enterprise assuming various future outcomes. In our retrospective review, the future outcomes included three scenarios: (i) we become a public company, (ii) we merge with or are acquired by another company, and (iii) we sell our intellectual property and other assets. We used a low probability assumption for an IPO when valuing our 2011 grants, and this percentage was expected to increase over time as we continue to have discussions with our investment bankers and prepared for an IPO. An increase in the probability assessment for an IPO increases the value ascribed to our common stock while a decrease in that probability has the opposite effect on the value ascribed to our common stock.

Estimated future and present values for the common stock were calculated using assumptions including:

- our expected pre-IPO valuation;
- a risk-adjusted discount rate associated with the IPO scenario;
- the liquidation preferences of our redeemable convertible preferred stock;
- the appropriate discount for lack of marketability assuming we remain a private company;
- the expected probability of completing an IPO versus remaining a private company or completing a merger or acquisition; and
- the estimated timing of a potential IPO.

The retrospective fair value of our common stock on March 31, 2010 was determined based on the following factors: the outlook of the oncology market at such time and the likelihood of completing an IPO or merger or sale transaction, offset by general market conditions. Relying primarily on the PWER method, the retrospective fair value of our common stock on March 31, 2010 was determined to be $2.96 per share. The additional stock-based compensation recognized resulting from the 2010 retrospective valuation was approximately $13,453.

The retrospective fair values of our common stock increased throughout 2010 and into 2011. The increases in the fair value of the common stock took into account changes in the following factors: the improved outlook in the oncology market in general and oncology companies focused on CSC targets specifically, the advancement of our product candidates, the increased likelihood of completing an IPO or merger or sale transaction and the improvement in general market conditions. Relying primarily on the PWER method, the retrospective fair value of our common stock on March 31, 2011 was determined to be $3.10 per share. The additional stock-based compensation recognized resulting from the 2011 retrospective valuation was approximately $12,036.

The fair value of our common stock was estimated again as of January 30, 2012. The fair value of the common stock on that date took into account changes in the following factors:

- the presentation of SL-401 data at the annual American Society of Hematology (ASH) conference;
- the presentation of SL-701 data at the American Society for Clinical Oncology (ASCO) conference;
- the improvement of general market conditions, which increased the probability of an IPO; and
- the advancement of discussions with investment bankers and the drafting of a prospectus for an IPO.

Based on the foregoing factors, and relying primarily on the PWER method, the fair value of our common stock on January 30, 2012 was determined to be $3.30 per share.

On February 29, 2012, March 5, 2012 and March 9, 2012 we approved for issuance equity awards with an exercise price of $3.30 per share, which our board of directors determined to be equal to the fair market value of our common stock on the approval date. As described in the table above and in further detail below, the GAAP measurement date for certain equity awards approved on March 9, 2012 and all of the awards approved on February 29, 2012 and March 5, 2012 is later than the award's approval date due to timing of the notification of the award's approval by the board of directors to the recipient of the award.

At the time these awards were authorized to be granted, there remained substantial uncertainty regarding the regulatory pathway for our product candidates and the likelihood of a successful initial public offering. Specifically, at the time these awards were authorized to be granted no significant events had occurred regarding our product candidates or prospects of completing a successful initial public offering to impact the contemporaneous valuation that had been set as of January 30, 2012. In addition, at the time these awards were authorized, our underwriters had not yet communicated to us the proposed price range for this initial public offering. Based on these and other factors, including concern over whether the public equity markets would be receptive to pre-commercial biotechnology companies such as ours, and in light of the challenges that similarly situated companies have experienced in recent months in completing their own proposed initial public offerings, our board of directors determined that the fair market value of our common stock at the time these awards were authorized to be granted was equal to $3.30 per share.

On February 29, 2012, March 5, 2012 and March 9, 2012, our board of directors approved for issuance a total of 621,828 option grants. We, however, did not timely notify all recipients of these option grants. Specifically, we concluded that, among these awards 441,124 awards were not sufficiently communicated to the recipients to constitute a measurement date for accounting purposes until May 24, 2012. For accounting purposes, based on a retrospective assessment for the equity awards measured as of May 24, 2012, considering the achievement of certain milestones noted below, we applied a fair value of the underlying common stock of $9.90 per share, which is approximately 90% of the midpoint of the offering range of $10.00 to $12.00, and to incorporate the fair value calculated in this retrospective assessment into the Black-Scholes option pricing model when calculating the stock-based compensation expense. Of the remaining awards, we accounted for a 90,352 share service-based award issued to our Chief Executive Officer on March 9, 2012 in our financial statements for the quarter ended March 31, 2012, because there was a mutual understanding of the terms of this award on March 9, 2012 and the measurement date was determined to be March 9, 2012. We also determined there was a measurement date of March 9, 2012 for a 90,352 share performance-based award issued to our Chief Executive Officer, because there was a mutual understanding of the terms of this award on March 9, 2012.

The following developments increased the value of our common stock in the periods indicated below.

Between March 9 and May 24, 2012

Regulatory Development. On March 26, 2012, certain of our senior representatives, including our Chief Executive Officer and Chief Medical Officer, met with FDA representatives to discuss our clinical development plans for SL-401. As is customary, the minutes of the FDA meeting were made available to us and our advisors following the meeting. We and our advisors regarded the outcome of the FDA meeting as consistent with our envisioned clinical development plan for SL-401 and overall expectations at that time. Importantly, the FDA did not require us to conduct any clinical testing prior to the initiation of our proposed randomized Phase 2b clinical trial in third-line AML, potentially saving us significant capital and avoiding significant delays. As such, the results of the FDA meeting alleviated a significant threshold risk that the FDA would have a fundamentally different view than us of the proposed clinical development plan for SL-401 at that time, which might have made the development of SL-401 as contemplated at that time infeasible or more expensive and/or time-consuming than we had envisioned.

Intellectual Property Developments. Two significant intellectual property developments occurred with respect to SL-701. First, on March 21, 2012, we entered into a license agreement with the University of Pittsburgh that grants us the right to use, and reference for regulatory applications, clinical data, information and know-how generated by the University in its prior clinical trials of SL-701. Without this license, we would not have had the right to use these data in our own IND with the FDA. Second, on March 30, 2012, we entered into a license agreement with the University that grants us the right to practice patents claiming the use of EphA2 epitopes, one of the two primary active agents in SL-701. Without this license, we would

not have the freedom to pursue the development and commercialization of SL-701 absent a potential infringement claim. Both of these developments significantly improved the value of the SL-701 program. Please refer to "Business — License and Research Agreements" for a more detailed discussion of our license agreements with the University of Pittsburgh.

Clinical, Operational and Infrastructure Developments. Several clinical, operational and infrastructure developments occurred that further increased our readiness for the late-stage clinical development of our product candidates and commercialization.

- Additional clinical trial results for SL-701 were released by the investigator-sponsored researcher in April 2012. Such results were presented at the Annual Meeting of the American Association for Cancer Research (AACR) on April 2, 2012. These results were positive and reinforcing of previous results. The two clinical trials involving SL-701, administered to both adults and children with advanced brain cancer, were selected for late-breaking presentations at the AACR. The research team presenting the results concluded that SL-701 demonstrated both immunological and clinical activity in children with malignant glioma, and was well tolerated and demonstrated immune responses in adult patients with low-grade glioma.

- We selected a contract manufacturing organization to initiate process development, optimization and scale-up of SL-401 drug supply for late-stage clinical trials and commercialization.

Between May 24, 2012 and July 19, 2012

Intellectual Property Developments. On June 15, 2012, we entered into an assignment agreement with Dr. Bergstein, our Chairman, Chief Executive Officer and President, with respect to intellectual property that is significant to us. Pursuant to this agreement, upon the effectiveness of the registration statement related to our initial public offering, Dr. Bergstein transferred to us multiple therapeutic and diagnostic issued patents, pending patent applications and related know-how and technology in exchange for cash or a combination of cash and shares of our common stock. At such time, our current license arrangement with Dr. Bergstein terminated.

Results of Operations

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.

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.

Comparison of Years Ended December 31, 2011 and 2010

Research and development expense. Research and development expense was $1.6 million for the year ended December 31, 2011, compared with $1.3 million for the year ended December 31, 2010, an increase of $0.3 million or 23%. This increase was primarily attributable to increased costs pertaining to the continued development of our lead compound SL-401, including $0.2 million of consulting fees and $0.1 million of salary and related costs including stock-based compensation, partially offset by a decrease of $0.1 million of patent-related costs.

General and administrative expense. General and administrative expense was $1.1 million for the year ended December 31, 2011, compared with $0.9 million for the year ended December 31, 2010. This increase was primarily attributable to $0.2 million of corporate legal fees and professional fees.

Interest expense. Interest expense was $98,643 for the year ended December 31, 2011, compared with $69,493 for the year ended December 31, 2010. The $29,150 increase for 2011 was attributable to the convertible notes due 2015 that were outstanding for the full 2011 calendar year versus nine months during the calendar year 2010.

Other expense. Other expense was $9,670 for the year ended December 31, 2011, compared with none for the year ended December 31, 2010. The $9,670 increase in other expense for 2011 was attributed to the mark to market of the put option liability.

Interest income. Interest income was $24,068 for the year ended December 31, 2011, compared with $43,045 for the year ended December 31, 2010. The $18,977 decrease in interest income for 2011 as compared to 2010 reflected lower cash balances in 2011.

Other income. Other income was $46,673 for the year ended December 31, 2011, compared with $484,905 for the year ended December 31, 2010. The $438,232 decrease in other income for 2011 as compared to 2010 was due to $244,479 of income associated with the receipt of the Qualified Therapeutic Discovery grant program from the federal government and the receipt of $218,556 from the Biotechnology Tax Credit from the City of New York received during calendar year 2010. There are no continuing performance or refund obligations related to these grants.

Liquidity and Capital Resources

Sources of Liquidity

We have financed our operations to date primarily through proceeds from sales of common stock and convertible preferred stock and issuances of convertible debt. To date, we have not generated any revenue from the sale of products. We have incurred losses and generated negative cash flows from operations since inception.

Through December 31, 2012, we received net proceeds of $3.8 million from the sale of common stock and $12.5 million from the sale of convertible preferred stock and $0.9 million from the issuance of convertible notes. Upon the completion of our initial public offering on January 31, 2013, we received net proceeds of $32.5 million net of fees and expenses.

As of December 31, 2012, our cash, cash equivalents and marketable securities totaled $2.0 million. We primarily invest our cash and cash equivalents in commercial savings accounts. We believe that our existing cash, cash equivalents and marketable securities, together with the net proceeds from our initial public offering, will be sufficient to fund our operations and our capital expenditures for at least the next 24 months.

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,		
	2010	2011	2012
Net cash used in operating activities	$ (1,862,600)	$ (1,936,480)	$ (4,126,548)
Net cash provided by investing activities	—	—	—
Net cash (used in) provided by financing activities	(147,429)	540,000	322,000
Net increase (decrease) in cash and cash equivalents	$ (2,010,029)	$ (1,396,480)	$ (3,804,548)

Operating activities. The use of cash in all periods resulted primarily from our net losses adjusted for non-cash charges and favorable changes in the components of working capital. The net cash used in operating activities increased in 2010, 2011 and 2012 mainly due to the timing of payments to our suppliers in connection with supply and research agreements associated with the continued development of our product candidates. The cash used for the years ended December 31, 2010, 2011 and 2012 was also impacted by an increase in research and development expenses as we increased our research and development headcount and from costs associated with the development of our lead compound SL-401.

Financing activities. The cash used by financing activities for the year ended December 31, 2010 was due to the payment of $0.8 million in connection with the redemption of our Series A preferred stock offset by the receipt of $0.6 million of proceeds from the private placement of our common stock. The net cash provided by financing activities for the year ended December 31, 2011 and, 2012 were due to the issuance of $0.5 million and $0.4 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:

- 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;
- add operational, financial and management information systems and personnel, including personnel to support our product development and future commercialization efforts.

The net proceeds from our initial public offering, together with our existing cash and cash equivalents, will enable us to fund our operating expenses and capital expenditure requirements for at least the next 24 months. 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 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 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.

We will also incur costs as a public company that we have not previously incurred, including, but not limited to, costs and expenses for directors fees, increased personnel costs, increased directors and officers insurance premiums, audit and legal fees, investor relations fees, expenses for compliance with the Sarbanes-Oxley Act of 2002 and rules implemented by the SEC and The NASDAQ Stock Market, LLC and various other costs.

Contractual Obligations and Commitments

The following table summarizes our contractual obligations at December 31, 2012:

	Total	Less than 1 year	1 - 3 years	3 - 5 years	More than 5 years
Long-term debt obligations	$ 2,006,881	$ —	$ 1,133,964	$ 872,917	$ —
License agreements	692,225	129,425	258,900	197,600	106,300
Total	$ 2,699,106	$ 129,425	$ 1,392,864	$ 1,070,517	$ 106,300

(1) Included in the "1 to 3 Years" column is all of the outstanding principal amount, as of December 31, 2012, of our senior convertible note due 2015, which equals $1,250,000 in principal amount plus accrued interest. Upon the completion of our initial public offering, the holder of the senior convertible note elected to convert $625,000 in principal amount, plus accrued interest thereon, into shares of our common stock at the initial public offering price. Included in the "3 to 5 Years" column is all of the currently outstanding principal amount of our convertible notes due 2017, which equals approximately $862,000 plus accrued interest. See Note 6 to the financial statements appearing at the end of this Form 10-K.

(2) We have executed several license agreements, as discussed in Note 10 to the financial statements to our financial statement and in more detail in the section titled "Business — License and Research 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 arrangements include the following:

- Under a research and license agreement with Scott and White Memorial 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.

(3) We have several charges to earnings that are contingent on the successful completion of performance conditions. As such, since it is not known when the performance condition will be achieved, these charges are not included in the table above. These conditions include the following:

- the effect of a beneficial conversion charge of approximately $0.1 million that will be recorded upon the conversion of our convertible notes due 2017;
- the recording of approximately $1.4 million of stock-based compensation expense associated with 573,424 options with a weighted average exercise price of $2.84 that fully vested upon the consummation of our initial public offering; and
- the recording of approximately $1.5 million of compensation expense relating 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.

(4) 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.

(5) On June 15, 2012, we entered into an assignment agreement with Dr. Bergstein, our Chairman, President and Chief Executive Officer and owner of certain proprietary patent rights and related technology. The agreement was amended on November 7, 2012. The agreement replaces an existing license agreement. Dr. Bergstein agreed to assign sell, transfer and convey to us 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 our common stock. Pursuant to the agreement, as amended, such amounts are payable only if, within five years of the date of transfer, we either (i) have a change in control, as defined in the assignment agreement, or (ii) achieve a market capitalization of at least $200 million for a prescribed period. Under the terms of the agreement, as amended, 50% of such payment shall be paid in cash and the remaining 50% may be paid in shares of our common stock, or a combination of cash and common stock, as determined by us.

None of the assigned patent rights and related technology has alternative future uses, nor have they reached a stage of technological feasibility. We plan to account for this transaction as an asset acquisition because we did not acquire any processes or activities in addition to the assigned rights and technology. We will record the entire purchase price to acquired in-process research and development expense and will record a corresponding liability at the present value of such payments upon resolution of the contingencies above. The agreement does not contain any vesting or rescission/refund provisions.

Off-Balance Sheet Arrangements

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

Tax Loss Carryforwards

As of December 31, 2012, we had federal net operating loss carryforwards of $14.7 million, which are available to reduce future taxable income. We also had federal tax credits of $0.5 million, which may be used to offset future tax liabilities. The net operating loss and tax credit carryforwards will expire at various dates through 2032. 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, 2012, 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.

Item 7A. Quantitative and Qualitative Disclosures About Market Risk

We are exposed to market risk related to changes in interest rates. We had cash and cash equivalents of $5.8 million as of December 31, 2011 and $2.0 million as of December 31, 2012, consisting of cash and 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 contract manufacturers. 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, 2011 and 2012, all of our liabilities were denominated in our functional currency.

Item 8. Financial Statements and Supplementary Data

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

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

None.

Item 9A. Controls and Procedures

Evaluation of Disclosure Controls and Procedures. As of December 31, 2012, 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, 2012, our disclosure controls and procedures were effective.

Management's Annual Report on Internal Control over Financial Reporting. This annual report does not include a report of management's assessment regarding internal control over financial reporting or an attestation report of the company's registered public accounting firm due to a transition period established by rules of the Securities and Exchange Commission for newly public companies.

Changes in Internal Control Over Financial Reporting. There were no changes in our internal control over financial reporting during the quarter ended December 31, 2012, 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 2013 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 2013 Annual Meeting of Stockholders.

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

The information required by this Item is incorporated herein by reference from our Proxy Statement for our 2013 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 2013 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 2013 Annual Meeting of Stockholders.

Part IV

Item 15. Exhibits, Financial Statements Schedules.

(a) 1. Financial Statements

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

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.
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 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.
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.
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.

† 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.

STEMLINE THERAPEUTICS, INC.
(A DEVELOPMENT STAGE COMPANY)

Index to Financial Statements

Contents

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, 2011 and 2012, and the related statements of operations, preferred stock and stockholders' equity (deficit) and cash flows for each of the three years in the period ended December 31, 2012 and the period from August 8, 2003 (Inception) to December 31, 2012. 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, 2011 and 2012, and the results of its operations and its cash flows for each of the three years in the period ended December 31, 2012 and the period from August 8, 2003 (Inception) to December 31, 2012, in conformity with U.S. generally accepted accounting principles.

/s/ Ernst & Young LLP

MetroPark, New Jersey
April 1, 2013

STEMLINE THERAPEUTICS, INC.
(A DEVELOPMENT STAGE COMPANY)
Balance Sheets

	December 31, 2011	December 31, 2012
Assets		
Current assets:		
Cash and cash equivalents	$ 5,829,886	**$ 2,025,338**
Prepaid expenses and other current assets	223,210	**299,089**
Total current assets	6,053,096	**2,324,427**
Deferred financing fees	400,000	**2,705,184**
Total assets	$ 6,453,096	**$ 5,029,611**
Liabilities and stockholders' equity (deficit)		
Current liabilities:		
Accrued liabilities	$ 1,582,410	**$ 5,500,735**
Total current liabilities	1,582,410	**5,500,735**
Convertible notes	1,566,116	**2,006,881**
Put option liability	99,230	**30,415**
Total liabilities	3,247,756	**7,538,031**
Stockholders' equity:		
Preferred stock $0.0001 par value, 5,000,000 shares authorized, none issued and outstanding at December 31, 2011 and 2012	—	**—**
Common stock $0.0001 par value, 5,421,150 shares authorized at December 31, 2011 and 22,500,000 shares authorized at December 31, 2012, 3,441,995 shares issued and outstanding at December 31, 2011 and 3,476,501 shares issued and outstanding at December 31, 2012	344	**347**
Additional paid-in capital	4,099,469	**4,660,488**
Retained (deficit) accumulated during the development stage	(894,473)	**(7,169,255)**
Total stockholders' equity/(deficit)	3,205,340	**(2,508,420)**
Total liabilities and stockholders' equity/(deficit)	$ 6,453,096	**$ 5,029,611**

See accompanying notes.

STEMLINE THERAPEUTICS, INC.
(A DEVELOPMENT STAGE COMPANY)
Statements of Operations

	Year Ended December 31,			Period From August 8, 2003 (Inception) to December 31, 2012
	2010	2011	2012	
Operating expenses:				
Research and development	$ 1,329,509	$ 1,629,026	**$ 3,376,962**	$ 11,445,668
General and administrative	930,331	1,088,028	**3,090,611**	9,484,796
Total operating expenses	2,259,840	2,717,054	**6,467,573**	20,930,464
Loss from operations	(2,259,840)	(2,717,054)	**(6,467,573)**	(20,930,464)
Other income	484,905	46,673	**301,684**	935,518
Other expense	—	(9,670)	**(35)**	(9,705)
Interest expense	(69,493)	(98,643)	**(118,765)**	(296,950)
Interest income	43,045	24,068	**9,907**	960,583
Net loss from operations	(1,801,383)	(2,754,626)	**(6,274,782)**	(19,341,018)
Less accretion of preferred stock dividends	(239,720)	—	**—**	(2,591,165)
Add discount on redemption of preferred stock	12,171,765	—	—	12,171,765
Net income (loss) attributable to common stockholders	$ 10,130,662	$ (2,754,626)	**$ (6,274,782)**	$ (9,760,418)
Net income (loss) attributable to common stockholders per common share:				
Basic	$ 3.07	$ (.80)	**$ (1.82)**	
Diluted	$ 2.81	$ (.80)	**$ (1.82)**	
Weighted-average shares outstanding:				
Basic	3,298,793	3,441,995	**3,441,995**	
Diluted	3,607,030	3,441,995	**3,441,995**	

See accompanying notes.

Stemline Therapeutics, Inc.
(A Development Stage Company)
Statements of Preferred Stock and Stockholders' Equity (Deficit)
Period From August 8, 2003 (Inception) to December 31, 2012

	Preferred Stock		Common Stock		Subscription Receivable	Additional Paid-in Capital	Earnings (Deficit) Accumulated During the Development Stage	Total Stockholders' Equity (Deficit)
	Shares	Capital	Shares	Capital				
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
Stock-based compensation	—	—	—	—	—	(35,706)	—	(35,706)
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	**—**	**—**	**34,506**	**3**	**—**	**561,019**	**—**	**561,022**
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)**

See accompanying notes.

STEMLINE THERAPEUTICS, INC.
(A DEVELOPMENT STAGE COMPANY)
Statements of Cash Flows

	Year Ended December 31,			Period From August 8, 2003 (Inception) to December 31, 2012
	2010	2011	2012	
Cash flows from operating activities				
Net loss	$ (1,801,383)	$ (2,754,626)	**$ (6,274,782)**	$ (19,341,018)
Adjustments to reconcile net loss to net cash used in operating activities:				
Stock-based compensation expense	80,535	108,405	**561,022**	2,199,667
Non-cash interest expense	69,493	98,643	**118,765**	296,950
Mark to market of put option liability	(21,860)	9,670	**(68,815)**	(81,005)
Changes in operating assets and liabilities:				
Prepaid expenses and other current assets	(183,600)	53,336	**(75,879)**	(299,089)
Other assets	—	(400,000)	**(2,305,184)**	(2,705,184)
Accrued liabilities	(5,785)	948,092	**3,918,325**	5,500,735
Net cash used in operating activities	(1,862,600)	(1,936,480)	**(4,126,548)**	(14,428,944)
Cash flows from investing activities				
Purchase of marketable securities	—	—	**—**	(20,545,087)
Redemption of marketable securities	—	—	**—**	20,545,087
Net cash provided by investing activities	—	—	**—**	—
Cash flows from financing activities				
Proceeds from issuance of preferred stock, net	—	—	**—**	12,500,000
Redemption of preferred stock	(750,000)	—	**—**	(750,000)
Proceeds from issuance of common stock	602,571	—	**—**	3,842,282
Proceeds from issuance of convertible notes	—	540,000	**322,000**	862,000
Net cash (used in) provided by financing activities	(147,429)	540,000	**322,000**	16,454,282
Net (decrease) increase in cash and cash equivalents	(2,010,029)	(1,396,480)	**(3,804,548)**	2,025,338
Cash and cash equivalents at beginning of period	9,236,395	7,226,366	**5,829,886**	—
Cash and cash equivalents at end of period	$ 7,226,366	$ 5,829,886	**$ 2,025,338**	$ 2,025,338
Supplemental disclosure of non-cash transactions				
Discount on redemption of preferred stock	$ 12,921,765	$ —	**$ —**	$ 12,921,765
Issuance of common stock on redemption of preferred stock	$ 1,200,000	$ —	**$ —**	$ 1,200,000
Accretion of preferred stock dividend	$ 239,720	$ —	**$ —**	$ 1,339,827

See accompanying notes.

STEMLINE THERAPEUTICS, INC.
(A DEVELOPMENT STAGE COMPANY)
NOTES TO FINANCIAL STATEMENTS
December 31, 2012

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 $19.5 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's success depends primarily on the successful development and regulatory approval of its product candidates. The Company estimates that its cash and cash equivalents at December 31, 2012, its January 2013 initial public offering, of which the net proceeds from this offering were approximately $32.5 million are sufficient to fund its anticipated operating cash requirements for at least 24 months. 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.

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.

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.

2. Summary of Significant Accounting Policies

Use of Estimates

The preparation of financial statements in conformity with U.S. generally accepted accounting principles requires management to make estimates and assumptions that affect the amounts reported in the financial statements and accompanying notes. Actual results could differ from those estimates.

The Company utilizes estimates and assumptions in determining the fair value of its common stock. The Company granted stock options at exercise prices not less than the fair market value of its common stock as determined by the board of directors, with input from management. The board of directors has determined the estimated fair value of the Company's common stock based on a number of objective and subjective factors, including external market conditions affecting the biotechnology industry sector and the historic prices at which the Company sold shares of its common stock.

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, 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.

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.

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.

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 9), 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 follows the provisions of the ASC Topic 718, *Compensation — Stock Compensation* which requires the measurement and recognition of compensation expense for all stock-based payment awards made to employees and non-employee directors, including employee stock options. Stock compensation expense based on the grant date fair value estimated in accordance with the provisions of ASC 718 is generally recognized as an expense over the requisite service period.

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, will be re-measured using the fair value of the Company's common stock and the non-cash expense recognized during the period will be 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.

Prior to becoming a public company, the board of directors determined the estimated fair value of the Company's common stock based on a number of objective and subjective factors, including external market conditions affecting the biotechnology industry sector and the prices at which the Company sold shares of its common stock.

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 they do 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.

Stock-based compensation expense includes stock options granted to employees and non-employees and has been reported in the Company's statements of operations as follows:

	Year Ended December 31,		
	2010	2011	2012
Research and development	$ 50,311	$ 79,955	**$ 412,536**
General and administrative	30,224	28,450	**148,486**
Total	$ 80,535	$ 108,405	**$ 561,022**

No tax benefits related to the stock-based compensation costs have been recognized since the Company's inception. The weighted average fair value of the options granted during 2010, 2011 and 2012 was estimated at $0.84, $1.05 and $6.34, respectively, on the date of grant using the Black-Scholes option-pricing model with the following weighted average assumptions:

	Year Ended December 31,		
	2010	2011	2012
Risk-free interest rate	2.78%	2.66%	**0.95%**
Expected volatility	74.48%	72.86%	**78.95%**
Dividend yield	—	—	**—**
Expected life	6.02 years	6.26 years	**6.25 years**

Segment information

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

Other Income and Expenses

Other income includes funds received from the U.S. Treasury Department in 2010 for the Qualified Therapeutic Discovery Projects tax credit and grant program as well as funds received from the City of New York for the 2010 and 2011 Biotechnology Tax Credit program. The income from these programs was reimbursement of expenses directly related to specific qualifying research programs in accordance with the guidelines of the respective grant programs and there are no performance or refund obligations. These expenses were incurred in prior periods and therefore the grant income was recorded when the funds were received. Other expenses include the mark-to-market of a put option liability associated with the issuance of convertible notes.

Recent Accounting Pronouncements

In May 2011, the Financial Accounting Standards Board issued guidance that changed the requirement for presenting "Comprehensive Income" in the financial statements. The update requires an entity to present the components of other comprehensive income either in a single continuous statement of comprehensive income or in two separate but consecutive statements. The currently available option to disclose the components of other comprehensive income within the statement of stockholders' equity will no longer be available. The update is effective for fiscal years, and interim periods within those years, beginning after December 15, 2011 and should be applied retrospectively.

The Company adopted this pronouncement and elected to present a separate statement of comprehensive income. The Company did not incur any components of comprehensive income for the periods presented and therefore did not include a statement of comprehensive income in the financial statements.

3. Net Income (Loss) Per Common Share

Basic and diluted net income (loss) per common share is determined by dividing net income (loss) applicable to common stockholders by the weighted average common shares outstanding during the period. For the periods where there is a net loss attributable to common shareholders, the outstanding shares of Series A Preferred Stock, convertible long term debt and common stock options have been excluded from the calculation of diluted income (loss) per common shareholder because their effect would be anti-dilutive. Therefore, the weighted average shares used to calculate both basic and diluted loss per share would be the same.

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

	Year Ended December 31,		
	2010	2011	2012
Basic net income (loss) per common share calculation:			
Net loss	$ (1,801,383)	$ (2,754,626)	**$ (6,274,782)**
Less: Preferred dividends	(239,720)	—	**—**
Plus: Redemption of preferred stock at a discount to carrying value	12,171,765	—	**—**
Net income (loss) attributable to common shareholders — basic	10,130,662	(2,754,626)	**(6,274,782)**
Basic weighted-average common shares	3,298,793	3,441,995	**3,441,995**
Basic net income (loss) per share	$ 3.07	$ (0.80)	**$ (1.82)**
Diluted net income (loss) per common share calculation:			
Net income (loss) attributable to common shareholders — basic	$ 10,130,662	$ (2,754,626)	**$ (6,274,782)**
Plus: Preferred dividends	239,720	—	**—**
Net income (loss) attributable to common shareholders — diluted	10,370,382	(2,754,626)	**(6,274,782)**
Basic weighted-average common shares	3,298,793	3,441,995	**3,441,995**
Effect of dilutive securities:			
Redeemable preferred stock	46,800	—	**—**
Employee stock options	38,487	—	**—**
Weighted-average shares used to compute diluted net income (loss) per share	3,607,030	3,441,995	**3,441,995**
Diluted net income (loss) per share	$ 2.81	$ (0.80)	**$ (1.82)**

The following potentially dilutive securities have been excluded from the computations of diluted weighted average shares outstanding for the periods presented, as they would be anti-dilutive:

	Year Ended December 31		
	2010	2011	2012
Restricted stock	—	**—**	**34,506**
Options outstanding	1,009,007	1,233,074	**1,819,839**
Total	1,009,007	1,233,074	**1,854,345**

4. Deferred Financing Fees

Deferred financing fees include legal fees directly attributable to the Company's offering of its equity securities and have been reported in the Company's balance sheet as follows:

Balance at December 31, 2010	**$** —
Increase in deferred financing fees	400,000
Balance at December 31, 2011	$ 400,000
Increase in deferred financing fees	2,305,184
Balance as of December 31, 2012	$ 2,705,184

5. Fair Value Measurements

The Company's financial instruments consist of cash and cash equivalents, accrued liabilities and a Put Option (as defined in Note 6) for the 2.45% Convertible Note. Fair value estimates of these instruments are made at a specific point in time, based on relevant market information. These estimates may be subjective in nature and involve uncertainties and matters of significant judgment and therefore cannot be determined with precision. The carrying amount of cash and cash equivalents, and accrued liabilities are generally considered to be representative of their respective fair values because of the short-term nature of those instruments.

Fair value is defined as the exchange price that would be received for an asset or paid to transfer a liability (an exit price) in the principal or most advantageous market for the asset or liability in an orderly transaction between market participants at the measurement date. Assets and liabilities that are measured at fair value are reported using a three-level fair value

hierarchy that prioritizes the inputs used to measure fair value. This hierarchy maximizes the use of observable inputs and minimizes the use of unobservable inputs. The three levels of inputs used to measure fair value are as follows:

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

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

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

The following fair value hierarchy table presents information about each major category of the Company's financial assets and liability measured at fair value on a recurring basis as of December 31, 2011 and 2012:

	Quoted Prices in Active Markets for Identical Assets (Level 1)	Significant Other Observable Inputs (Level 2)	Significant Unobservable Inputs (Level 3)	Total
At December 31, 2012				
Cash	**$ 2,025,338**	**$ —**	**$ —**	**$ 2,025,338**
Put Option	**$ —**	**$ —**	**$ (30,415)**	**$ (30,415)**
At December 31, 2011				
Cash	$ 5,829,886	$ —	$ —	$ 5,829,886
Put Option	$ —	$ —	$ (99,230)	$ (99,230)

The Company measures the put option liability at fair value 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. The Company assesses these assumptions and estimates on an on-going basis as additional data impacting the assumptions and estimates are obtained. Changes in the fair value of the put option liability related to updated assumptions and estimates are recognized within the statements of operations.

The put option liability may change significantly as additional data is obtained, impacting the Company's assumptions regarding probabilities of outcomes used to estimate the fair value of the liability. In evaluating this information, considerable judgment is required to interpret the data used to develop the assumptions and estimates. The estimates of fair value may not be indicative of the amounts that could be realized in a current market exchange. Accordingly, the use of different market assumptions and/or different valuation techniques may have a material effect on the estimated fair value amounts, and such changes could materially impact the Company's results of operations in future periods.

Level 3 Disclosures

The following table provides quantitative information associated with the fair value measurement of the Company's Level 3 inputs:

	Fair Value as of December 31, 2012	Valuation Technique	Unobservable Input	Range (Weighted Average)
Put option liability	$ 30,415	Probability-adjusted discounted cash flow	Probabilities of success	25% — 45% (35)%
			Periods in which outcomes are expected to be achieved	2013
			Discount rate	12%

	Fair Value as of December 31, 2011	Valuation Technique	Unobservable Input	Range (Weighted Average)
Put option liability	$ 99,230	Probability-adjusted discounted cash flow	Probabilities of success	25% — 30% (45)%
			Periods in which outcomes are expected to be achieved	2014
			Discount rate	12%

The fair value of the put option liability represents the fair value of the Company's liability for all potential payments if the holder of the put option elected to convert into cash or shares of common stock. The significant unobservable inputs used in the fair value measurement of the Company's put option liability are the probabilities of successful outcomes, which would trigger conversion of the put option liability, probabilities as to the periods in which the outcomes are expected to be achieved and a discount rate. Significant changes in any of the probabilities of success would result in a significantly higher or lower fair value measurement, respectively. Significant changes in the probabilities as to the periods in which outcomes will be achieved would result in a significantly lower or higher fair value measurement, respectively.

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

	Level 3
Balance at December 31, 2009	$ —
Fair value adjustment of put option liability	111,420
Fair value adjustment to put option liability included in the other income	(21,860)
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

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. No transfers of assets between Level 1 and Level 2 of the fair value measurement hierarchy occurred during the year ended December 31, 2012.

6. Other Accrued Liabilities

Other accrued liabilities consist of the following:

	December 31, 2011	December 31, 2012
Accrued research and development costs	$ 268,547	**$ 972,218**
Accrued compensation	129,009	**100,000**
Accrued legal	1,043,018	**3,289,806**
Other accrued liabilities	141,836	**1,138,711**
Total	$ 1,582,410	**$ 5,500,735**

7. Convertible Notes

On March 16, 2010, in connection with the redemption of the Series A preferred stock, the Company issued a Senior Convertible Note ("the 2.45% Convertible Note") in the amount of $1.25 million. The 2.45% Convertible Note was initially recorded at fair value of $0.90 million. The 2.45% Convertible Note and the related interest expense are due on March 16, 2015. Interest is being charged at a rate of 2.45% per annum.

The carrying value of the 2.45% Convertible Note consists of $1.3 million of principal and accrued interest less $0.1 million of unamortized debt discount as of December 31, 2012 and $1.3 million of principal and accrued interest less $0.3 million of unamortized debt discount as of December 31, 2011.

Upon the occurrence of a qualified financing event as defined in the agreement, the 2.45% Convertible Note and any accrued interest are mandatorily convertible into shares of the same securities issued in the qualified financing at the same price per share used in the qualified financing. In addition, 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 recorded at approximately $111,000, its fair value on the date of issuance and is marked to fair value at each reporting period. During the years ended December 31, 2010, 2011 and 2012, changes in the fair value of the Put Option of approximately $(22,000), $10,000 and $(69,000), respectively, were recorded in Other Income and Other Expense in the Statement of Operations.

On July 26, 2012, the Company and NB Athyrium LLC, the holder of the 2.45% Convertible Notes, entered into an amendment to the 2.45% Convertible Notes pursuant to which NB Athyrium agreed to accelerate repayment of $625,000 in principal amount, plus accrued interest thereon, of such note in cash, at the time of the initial public offering, and convert the remaining $625,000 in principal amount, plus accrued interest thereon, into shares of our common stock at the initial public offering price. On November 14, 2012, the Company and NB Athyrium LLC, entered into an additional amendment to the 2.45% Convertible Notes pursuant to which NB Athyrium agreed to cancel the acceleration and defer the repayment of $625,000 in principal amount, plus accrued interest thereon, of such note in cash until February 28, 2014. The July 2012 amendment was not considered a significant amendment from an accounting perspective because there was no change in the cash flows resulting from the modification. The November 2012 amendment was not significant because the impact on cash flows on a present value basis is less than 10%.

In January 2012, the Company issued $0.9 million of convertible notes (the "1.27% Convertible Notes") at face value for cash. Of this amount, approximately $0.5 million was received on or before December 31, 2011 and before the note agreements were signed. These amounts were classified as long term liabilities on the balance sheet, consistent with the terms of the notes that were signed in January 2012.

The 1.27% Convertible Notes and the related interest expense are due in 5 years if the notes are not converted prior to that date. Interest is being charged at a rate of 1.27% per annum. The 1.27% Convertible Notes and related accrued interest are convertible into common stock at a conversion price equal to 87.5% of the IPO price per share upon the occurrence of an IPO, as defined in the 1.27% Convertible Notes agreement. Additionally, the 1.27% Convertible Notes are convertible upon

the occurrence of a qualified or non-qualified financing, as defined in the 1.27% Convertible Notes agreement, at a price equal to 85% of the price per share used in the each financing.

The 1.27% Convertible Notes also contain a beneficial conversion option such that immediately upon the occurrence of one of the financings discussed above, the 1.27% Convertible Notes shall convert into shares of newly issued common stock in the case of an IPO or the same securities issued in the case of a qualified or non-qualified financing. The 1.27% Convertible Notes holders shall be entitled to receive a number of shares determined by dividing the applicable 1.27% Convertible Note balance as of the conversion date by an amount equal to the share price as determined above. Upon a triggering event that forces conversion where both the price and quantity of the shares are known, a beneficial conversion charge will be determined representing the difference between the conversion price and the fair value of the new shares multiplied by the number of shares and a beneficial conversion charge will be recorded to earnings with a corresponding credit to additional paid-in capital.

During the years ended December 31, 2010, 2011 and 2012, the Company recorded interest expense of approximately $69,000, $99,000 and $77,224, respectively, related to the amortization of the debt discount.

The following table summarizes the convertible notes as of December 31, 2012.

	Year Ended December 31, 2012		
	Principal	Accrued interest and bond discount	Total
2.45% Convertible Notes	$ 1,250,00	$ (116,036)	**$ 1,133,964**
1.27% Convertible Notes	862,000	10,917	**872,917**
Total	$ 2,112,000	$ (105,119)	**$ 2,006,881**

8. Capital Structure

Common Stock

As of December 31, 2012, the Company was authorized to issue 22,500,000 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. Certain of the Company's stockholders have the right to appoint two directors, provided certain minimum ownership levels are maintained. These appointment rights terminate upon the closing of an IPO. 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 convertible notes and stock options. As of December 31, 2011 and 2012, the Company reserved 1,271,818 and 2,226,768 shares of common stock, respectively, for future issuance related to the exercise of the Company's outstanding stock options, and reserved an adequate number of shares of common stock for future issuance related to the conversion of the Company's Convertible Notes.

Preferred Stock

In October 2007, the Company sold 455,518 shares of 8% Series A Redeemable Convertible Preferred Stock at a purchase price of $27.44 per share, resulting in net proceeds of $12.5 million.

At any time on or after the fourth anniversary date of the issuance of the Series A Preferred Stock, the holders of the Series A Preferred Stock could require the Company to redeem the shares for a price per share equal to the original issuance price plus any accumulated but unpaid dividends. As a result, the carrying value of the Series A Preferred Stock was accreted to their redemption value by a charge to additional paid-in capital. For the years ended December 31, 2010, 2011 and 2012 and for the period from inception (August 8, 2003) to December 31, 2012, the Company recorded dividends amounting to $239,720, $0, $0 and $2,591,165, respectively, related to the accretion of the Series A Preferred Stock to their redemption value.

Redemption of Series A Preferred Stock

On March 16, 2010, the Company entered into a Note Purchase Agreement with several investment funds (the "Pequot Funds") that held the Company's Series A preferred stock (the "Preferred Shares") and NB Athyrium LLC. In exchange for 455,518 outstanding shares of the Preferred Shares, with a carrying value of $15.1 million, including $2.6 million for the accretion of dividends through the date of redemption, the Company paid $0.75 million of cash, issued 411,571 shares of its common stock valued at $1.2 million and issued a $1.25 million senior unsecured convertible note, represented by the 2.45% Convertible Note. This transaction was accounted for as an extinguishment of the Preferred Shares, as it resulted in the surrender and cancellation of the Preferred Shares. The 2.45% Convertible Note and common stock issuances were recorded at their issuance date fair value of $0.90 million and $1.2 million, respectively. The note and the common stock given for the redemption of the Preferred Shares were immediately transferred from the Pequot Funds to NB Athyrium LLC. The transaction resulted in a $12.2 million discount from the redemption of the Preferred Shares, including the cash payment over the carrying value of the Preferred Shares, and was accounted for in accordance with ASC Topic 260-10, Earnings per share. The $12.2 million discount was based on the ability of the Company to negotiate a favorable redemption of the Company's preferred stock due to business conditions of the preferred stock holders and restrictive transfer covenants in the investment documents governing the preferred stock. The value of the common stock issued as part of the redemption of the Company's preferred stock was based on a contemporaneous equity offering occurring in April 2010 at a value of $1.46 per share. This offering was comprised of new investors (57%) and existing investors (43%). The discount on the redemption of the Preferred Shares has been reflected as a reduction of the Accumulated Deficits in the Company's Statement of Preferred Stock and Stockholders Equity (Deficit) and also as an offset to the net operating losses in the Company's Statement of Operations to arrive at $10.1 million of net income available to common stockholders for the year ended December 31, 2010.

Before the redemption of the Series A Preferred Stock, the Company, the Pequot Funds and certain holders of the Company's common stock were party to an investors' rights agreement that provided the Pequot Funds with demand registration rights, piggyback registration rights, information rights and rights of first offer with respect to certain future issuances of the Company's securities. This agreement also required the approval of the directors designated by the Pequot Funds and certain holders of the Company's common stock for certain actions proposed to be taken. The parties to the investors' rights agreement terminated the agreement in connection with the redemption of the Series A Preferred Stock.

Representative's Warrants

On October 1, 2012, the Company agreed to issue to the representative of the underwriters in this offering 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. These warrants will be accounted for as a cost of issuance in the event of a successful public offering. There are no warrants outstanding as of December 31, 2012.

9. Stock-Based Compensation

The Company's 2004 Stock Option and Grant Plan (the "2004 Plan") was adopted by the board of directors in September 2003 and approved by the stockholders in September 2003. The 2004 Plan initially authorized the Company to grant up to 1,271,818 shares of common stock to eligible employees, directors, consultants and advisors to the Company in the form of options to purchase common stock in the Company at a price not less than the estimated fair value at the date of grant, or 110% of the estimated fair value at the date of grant if the optionee is a 10% owner of the Company. Under the provisions of the 2004 Plan, no option will have a term in excess of 10 years.

In March 2012, the Company amended its certificate of incorporation to increase the number of authorized shares of common stock from 5,421,150 shares to 6,351,780 shares. In addition, the Company amended its Amended and Restated 2004 Employee, Director and Consultant Stock Plan to increase the number of shares reserved for issuance under the plan from 1,271,818 shares to 2,226,768 shares.

The 2004 Plan is intended to encourage ownership of stock by employees and consultants of the Company and to provide additional incentives for them to promote the success of the Company's business. The board of directors is responsible for determining the individuals to receive option grants, the number of options each individual will receive, the option price per share and the exercise period of each option. Options granted pursuant to the 2004 Plan generally vest over four years and

have been granted at the estimated fair value of the Company's common stock, as determined by the board of directors, as of each grant date. In establishing its estimates of fair value of the Company's common stock prior to becoming a public company, the Company considered the guidance set forth in the AICPA Practice Aid, *Valuation of Privately-Held-Company Equity Securities Issued as Compensation* , and performed a retrospective determination of the fair value of its common stock for the years ended December 31, 2010 and 2011.

The Company performed a retrospective determination of the fair value of the Company's common stock for the years ended December 31, 2010 and 2011 and granted stock options with exercise prices as follows:

Grant Date	Number of Options Granted	Exercise Price	Retrospective Determination of Fair Value	Intrinsic Value
March 22, 2010	452,665	$ 2.21	$ 2.96	$ 0.37
March 8, 2011	224,067	$ 2.92	$ 3.10	$ 0.09

The following is a summary of stock option activity under the 2004 Plan through December 31, 2012:

	Options	Weighted-Average Exercise Price	Weighted-Average Remaining Contractual Life	Aggregate Intrinsic Value
Outstanding at January 1, 2010	556,345	$ 2.58		
Options granted	452,662	2.21		
Options exercised	—	—		
Options forfeited	—	—		
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**	**6.54**	**$ 13,167,542**
Options exercisable at December 31, 2012	**600,284**	**$ 2.52**	**3.30**	**$ 4,490,901**

As of December 31, 2012, there were 372,422 shares of common stock available for future grants under the 2004 Plan.

During the first quarter of 2012, the Company granted various employees, consultants and service providers options to purchase an aggregate of 621,828 shares of common stock. The options were granted at an exercise price of $3.30 per share, the fair market value of the Company's common stock on the date of grant as determined by the Company's board of directors. The options are subject to various vesting conditions.

Intrinsic value in the above table was calculated as the difference between the Company's estimated stock price on December 31, 2012 and the exercise price, multiplied by the number of options. For any of the Company's outstanding stock options with an exercise price equal to or greater than the Company's estimated stock price on December 31, 2012, the intrinsic value was considered to be zero.

As of December 31, 2012, there was $4.7 million of unrecognized compensation expense related to unvested stock options, which is expected to be recognized over a weighted average period of 1.89 years. There were no exercises of stock options during the years ended December 31, 2010, 2011 and 2012.

The Company periodically remeasures fair value of stock-based awards issued to non-employees and records expense over the requisite service period. The Company granted 97,796 options to non-employees and has recorded compensation expense of $29,397, $23,883 and $127,461 for the years ended December 31, 2010, 2011 and 2012, respectively.

Performance Share Awards

The following information relates to awards of performance shares and performance share, units, included in the preceding table, that have been granted to employees under the 2004 Plan.

In March 2010, the Company issued 406,131 options, with a weighted average exercise price of $2.21 per share, to consultants and key employees that fully vest upon the occurrence of an IPO or a specified financing. Also, in March 2011, the Company issued 112,036 options with a weighted average grant price of $2.92 per share that fully vest upon the occurrence of an IPO or a specified financing to directors, consultants, and key employees. Also, in March 2012, the Company issued 301,014 options with a weighted average grant price of $3.30 per share of which 281,895 options begin to vest and 19,119 options fully vest upon the occurrence of an IPO or a specified financing to directors, consultants, and key employees.

For awards with performance conditions, such as capital raises, an IPO, a change in control or a sale of the company, no expense will be 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.

10. Income Taxes

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

	2010	2011	2012
Deferred:			
Federal	$ (582,411)	$ (786,282)	$ (1,775,748)
State and local	(348,416)	(470,377)	(1,062,155)
	(930,827)	(1,256,659)	(2,837,903)
Increase in valuation allowance	930,827	1,256,659	2,837,903
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,		
	2010	2011	2012
Percent of pre-tax income:			
U.S. federal statutory income tax rate	(34.0)%	(34.0)%	**(34)%**
State taxes, net of federal benefit	(13.8)	(12.2)	**(11.3)**
Permanent items	(3.9)	0.6	**(0.3)**
Change in valuation allowance	51.7	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, 2011	December 31, 2012
Current deferred tax assets		
Accrued expenses	$ —	**$ 712,590**
Valuation allowance	—	**(712,590)**
Total noncurrent deferred tax assets	$ —	**$ —**
Noncurrent deferred tax assets		
Net operating loss carryforwards	$ 4,913,971	**$ 6,682,819**
Research credits	378,359	**473,380**
Convertible debt interest expense	44,167	**81,087**
Nonqualified stock compensation	600,374	**824,898**
Valuation allowance	5,936,871	**8,062,184**
Total noncurrent deferred tax assets	(5,936,871)	**(8,062,184)**
	$ —	**$ —**

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, 2010, 2011 and 2012.

The following table summarizes carryforwards of net operating losses and tax credits as of December 31, 2012:

	Amount	Expiration
Federal net operating losses	$ 14,707,000	2023 — 2032
State net operating losses	$ 15,085,000	2023 — 2032
Research and development credits	$ 473,000	2023 — 2032

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, 2012 and does not expect this to change significantly over the next twelve months. As of December 31, 2012, 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, 2012 are still subject to examination by major tax jurisdictions.

11. 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, 2012, the Company has paid or accrued $1.7 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 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.

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-13Rα2, 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-13Rα2 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

Certain bonuses and salary increases in the amount of $1,034,948 are contingent and payable upon approval of the board of directors, continued employment, and the occurrence of a specified financing, including the consummation of an initial public offering, with an additional $509,052 subject to the same contingencies and payable one year after the occurrence of a specified financing. No amounts have been recorded in respect of either the bonuses or salary increases as payment is not considered probable as of December 31, 2012.

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.

12. Related Party Transactions

Since January 1, 2009, the Company has engaged in the following transactions with its directors, executive officers, holders of more than 5% of voting securities, and affiliates or immediate family members of the directors, executive officers and holders of more than 5% of voting securities. The Company believes that all of these transactions were on terms as favorable as could have been obtained from unrelated third parties.

License Agreement and Assignment Agreement with the Company's Chief Executive Officer

The Company is party to a license agreement with Dr. Bergstein, dated December 1, 2003, pursuant to which Dr. Bergstein licensed to the Company his interest in the oncology-related patent rights, and all technology and know-how related to such patent rights, as well as improvements thereto. These patent rights do not relate to the Company's product candidates SL-401 and SL-701. The Company is required to pay Dr. Bergstein $2.0 million in cash or common stock the first time the Company obtains regulatory approval of each licensed product in the United States for certain major cancer indications, as well as a royalty in the low single digits as a percentage of net sales. As part of this license agreement, the Company granted Dr. Bergstein certain piggyback registration rights with respect to any common stock the Company issues him in connection with a milestone payment.

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 plans to account for this transaction as an asset acquisition when the company achieves a market capitalization of at least $200 million for a prescribed period because it did not acquire any processes or activities in addition to the assigned rights and technology. The Company will record the entire purchase price to acquired in-process research and development expense. The assignment agreement does not contain any vesting or rescission/refund provisions.

13. Subsequent Events

The Company evaluated events that occurred subsequent to December 31, 2012 through April 1, 2013, the date the financial statements were available to be issued.

Initial Public Offering

On January 31, 2013 the Company completed its 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 $32.5 million. Additionally, upon the closing of the IPO, our convertible debt, plus accrued interest thereon, was converted into 166,769 shares of common stock.

Stock-Based Compensation

The Company's 2012 Stock Equity Incentive Plan (the "2012 Plan") was adopted by the board of directors and approved by the stockholders in July 2012 effective immediately prior to the closing of the Company's initial public offering. In addition, the 2004 Plan was terminated effective immediately prior to the closing of the Company's initial public offering. Provided that the 1,819,839 options to purchase common stock and 34,507 restricted stock awards executed prior to the effective date of such termination shall 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 1,663,728 shares of common stock to eligible employees, directors, consultants and advisors to the Company in the form of options to purchase common stock in the Company at a price not less than the estimated fair value at the date of grant. Under the provisions of the 2004 Plan, no option will have a term in excess of 10 years.

Performance Share Awards

Subsequent to the closing of the IPO, certain options and restricted stock begin to vest and fully vest to directors, consultants and key employees. The Company will record 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 vest upon consummation of an IPO. In addition, 281,895 options will commence vesting based upon the consummation of an IPO and the Company will record $1.4 million on the vesting of these options.

Compensation Arrangement

Subsequent to the closing of the IPO, 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.

Contractual Agreement

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.

14. Selected Quarterly Financial Data (Unaudited)

	Quarters Ended			
	March 31	June 30	September 30	December 31
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)
2011				
Net loss attributable to common stockholders	$ (376,108)	$ (628,551)	$ (710,249)	$ (1,039,718)
Basic and diluted net loss per common share	$ (0.11)	$ (0.18)	$ (0.21)	$ (0.30)

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Index to Exhibits

Exhibit No.	Description
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.

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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: April 1, 2013

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, 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 April 1, 2013, 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/ Stephen P. Hall Stephen P. Hall	Vice President of Finance and Chief Accounting Officer (Principal Financial and Accounting Officer)
/s/ J. Kevin Buchi J. Kevin Buchi	Director
/s/ Kenneth Zuerblis Kenneth Zuerblis	Director
/s/ Ron Bentsur Ron Bentsur	Director
/s/ Eric L. Dobmeier Eric L. Dobmeier	Director

Exhibit 31.1

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

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

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

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

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

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

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

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

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

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

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

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

Date: April 1, 2013

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

Exhibit 31.2

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

I, Stephen P. Hall, certify that:

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

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

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

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

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

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

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

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

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

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

Date: April 1, 2013

/s/ Stephen P. Hall
Stephen P. Hall
Chief Accounting Officer
Principal Financial and Accounting Officer

Exhibit 32.1

STATEMENT OF CHIEF EXECUTIVE OFFICER OF
STEMLINE THERAPEUTICS, INC.
PURSUANT TO 18 U.S.C. SECTION 1350,
AS ADOPTED PURSUANT TO
SECTION 906 OF THE SARBANES-OXLEY ACT OF 2002

In connection with the annual report of Stemline Therapeutics, Inc. (the "Company") on Form 10-K for the period ended December 31, 2012 as filed with the Securities and Exchange Commission (the "Report"), I, Ivan Bergstein, Chief Executive Officer of the Company, certify, pursuant to 18 U.S.C. §1350, as adopted pursuant to §906 of the Sarbanes-Oxley Act of 2002, that, based on my knowledge:

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

2) The information contained in the Report fairly presents, in all material respects, the financial condition and results of operations of the Company.

Date: April 1, 2013

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

Exhibit 32.2

STATEMENT OF CHIEF ACCOUNTING OFFICER OF
STEMLINE THERAPEUTICS, INC.
PURSUANT TO 18 U.S.C. SECTION 1350,
AS ADOPTED PURSUANT TO
SECTION 906 OF THE SARBANES-OXLEY ACT OF 2002

In connection with the annual report of Stemline Therapeutics, Inc. (the "Company") on Form 10-K for the period ended December 31, 2012 as filed with the Securities and Exchange Commission (the "Report"), I, Stephen P. Hall, Chief Accounting Officer of the Company, certify, pursuant to 18 U.S.C. §1350, as adopted pursuant to §906 of the Sarbanes-Oxley Act of 2002, that, based on my knowledge:

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

2) The information contained in the Report fairly presents, in all material respects, the financial condition and results of operations of the Company.

Date: April 1, 2013

/s/ Stephen P. Hall
Stephen P. Hall
Chief Accounting Officer
Principal Financial and Accounting Officer