STEMLINE THERAPEUTICS INC
Form S-1
October 17, 2013

Table of Contents

As filed with the Securities and Exchange Commission on October 17, 2013

Registration No. 333-

UNITED STATES
SECURITIES AND EXCHANGE COMMISSION

Washington, D.C. 20549

FORM S-1

REGISTRATION STATEMENT
UNDER
THE SECURITIES ACT OF 1933

STEMLINE THERAPEUTICS, INC.

(Exact name of registrant as specified in its charter)

DELAWARE	**2834**	**45-0522567**
(State or other jurisdiction of incorporation or organization)	(Primary Standard Industrial Classification Code Number)	(I.R.S. Employer Identification Number)

750 Lexington Avenue
Eleventh Floor
New York, New York 10022
(646) 502-2311
(Address, including zip code, and telephone number, including area code, of registrant's principal executive offices)

Ivan Bergstein, M.D.
Chairman, President and Chief Executive Officer
Stemline Therapeutics, Inc.
750 Lexington Avenue
Eleventh Floor
New York, New York 10022
(646) 502-2311
(Name, address, including zip code, and telephone number, including area code, of agent for service)

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(212) 210-9400 **Boston, Massachusetts 02111**
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Approximate date of commencement of proposed sale to the public:
As soon as practicable after this Registration Statement is declared effective.

If any of the securities being registered on this Form are to be offered on a delayed or continuous basis pursuant to Rule 415 under the Securities Act of 1933, check the following box. o

If this Form is filed to register additional securities for an offering pursuant to Rule 462(b) under the Securities Act, check the following box and list the Securities Act registration statement number of the earlier effective registration statement for the same offering. o

If this Form is a post-effective amendment filed pursuant to Rule 462(c) under the Securities Act, check the following box and list the Securities Act registration statement number of the earlier effective registration statement for the same offering. o

If this Form is a post-effective amendment filed pursuant to Rule 462(d) under the Securities Act, check the following box and list the Securities Act registration statement number of the earlier effective registration statement for the same offering. o

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

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

CALCULATION OF REGISTRATION FEE

TITLE OF EACH CLASS OF SECURITIES TO BE REGISTERED	PROPOSED MAXIMUM AGGREGATE OFFERING PRICE(1)	AMOUNT OF REGISTRATION FEE(2)
Common Stock, $0.0001 Par Value Per Share	$90,000,000	$11,592.00

(1) Estimated solely for the purpose of calculating the registration fee pursuant to Rule 457(o) under the Securities Act of 1933, as amended.

(2) Calculated pursuant to Rule 457(o) based on an estimate of the proposed maximum aggregate offering price.

The registrant hereby amends this registration statement on such date or dates as may be necessary to delay its effective date until the registrant shall file a further amendment which specifically states that this registration statement shall thereafter become effective in accordance with Section 8(a) of the Securities Act of 1933 or until the registration statement shall become effective on such date as the Securities and Exchange Commission, acting pursuant to said Section 8(a), may determine.

Table of Contents

Subject to completion, Preliminary Prospectus dated October 17, 2013

The information in this preliminary prospectus is not complete and may be changed. We may not sell these securities until the registration statement filed with the Securities and Exchange Commission is effective. This preliminary prospectus is not an offer to sell these securities and it is not soliciting an offer to buy these securities in any jurisdiction where the offer or sale is not permitted.

Prospectus

shares

Common stock

We are offering shares of our common stock. Our common stock is listed on The NASDAQ Capital Market under the symbol "STML." The last reported sale price of our common stock on October 16, 2013 was $37.46 per share.

We are an "emerging growth company" as that term is used in the Jumpstart Our Business Startups Act of 2012, or the JOBS Act, and, as such, we elected to comply with certain reduced public company reporting requirements. Investing in our common stock involves a high degree of risk.

	Per share	Total
Public offering price	$	$
Underwriting discounts and commissions(1)	$	$
Offering proceeds to us, before expenses	$	$

(1) See "Underwriting" for additional compensation details.

We have granted a 30-day option to the underwriters to purchase up to additional shares of our common stock.

The underwriters are offering the common stock as set forth under "Underwriting." Delivery of the shares will be made on or about October , 2013.

Before investing in our common stock, you should carefully read the discussion of "Risk Factors" beginning on page 13 of this prospectus.

Neither the Securities and Exchange Commission nor any state securities commission has approved or disapproved of these securities or determined if this prospectus is truthful or complete. Any representation to the contrary is a criminal offense.

Joint Book-Running Managers

J.P. Morgan **Jefferies**

Lead Manager

Aegis Capital Corp

Roth Capital Partners **Ladenburg Thalmann & Co. Inc.** **H.C. Wainwright & Co., LLC**

, 2013

Table of Contents

Table of Contents

Table of contents

You should rely only on the information contained or otherwise incorporated by reference in this prospectus and any free writing prospectus prepared by or on behalf of us or to which we have referred you. We have not authorized anyone to provide you with information that is different. We are offering to sell, and seeking offers to buy, shares of common stock only in jurisdictions where offers and sales are permitted. The information contained in this prospectus, any free writing prospectus, or any document incorporated by reference herein is accurate only as of its date, regardless of the time of delivery of this prospectus or of any sale of common stock. To the extent there is a conflict between the information contained in this prospectus and the information contained in any document incorporated by reference herein filed prior to the date of this prospectus, you should rely on the information in this prospectus; provided that if any statement in one of these documents is inconsistent with a statement in another document having a later date for example, a document incorporated by reference in the prospectus the statement in the document having the later date modifies or supersedes the earlier statement.

It is important for you to read and consider all information contained in this prospectus, including the documents incorporated by reference herein, in making your investment decision. You should also read and consider the information in the documents to which we have referred you in the sections entitled "Where You Can Find More Information" and "Incorporation of Documents by Reference" in this prospectus.

For investors outside of the United States: neither we nor any of the underwriters have done anything that would permit this offering or possession or distribution of this prospectus in any jurisdiction where action for that purpose is required, other than in the United States. You are required to inform yourselves about and to observe any restrictions relating to this offering and the distribution of this prospectus.

Table of Contents

We further note that the representations, warranties and covenants made by us in any agreement that is filed as an exhibit to the registration statement of which this prospectus is a part or to any document that is incorporated by reference herein were made solely for the benefit of the parties to such agreement, including, in some cases, for the purpose of allocating risk among the parties to such agreements, and should not be deemed to be a representation, warranty or covenant to you. Moreover, such representations, warranties or covenants were accurate only as of the date when made. Accordingly, such representations, warranties and covenants should not be relied on as accurately representing the current state of our affairs.

Table of Contents

Prospectus summary

This summary provides an overview of selected information contained elsewhere in this prospectus or incorporated by reference into this prospectus from our Annual Report on Form 10-K for the year ended December 31, 2012, and our other filings with the Securities and Exchange Commission listed in the section of this prospectus entitled "Incorporation of Documents by Reference" and does not contain all of the information you should consider before investing in our common stock. You should carefully read this prospectus, the registration statement of which this prospectus is a part and the information incorporated by reference herein in their entirety before investing in our common stock, including the information discussed under "Risk Factors" in this prospectus and our financial statements and notes thereto that are incorporated by reference herein.

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 the treatment of blastic plasmacytoid dendritic cell neoplasm, or BPDCN, third-line acute myeloid leukemia, or AML, and multiple other hematologic cancers. In February 2011, SL-401 received Orphan Drug designation from the FDA for the treatment of AML. In June 2013, SL-401 received Orphan Drug designation from the FDA for the treatment of BPDCN. SL-701 is a subcutaneously-administered therapeutic cancer vaccine comprised of synthetic peptides, and is currently being developed for the treatment of advanced adult and pediatric brain cancer. In completed investigator-sponsored Phase 1/2 clinical trials, both SL-401 and SL-701 have demonstrated single agent activity, including durable complete responses, or CRs, and longer median overall survival, or OS, in heavily pretreated patients compared with that achieved in the past with traditional therapies.

Based upon favorable clinical data observed to date with SL-401 and SL-701 across multiple liquid and solid tumor types, we plan to initiate multiple clinical trials in 2014 of SL-401 in a variety of hematological cancers and of SL-701 in adult and pediatric brain cancers. These include pivotal programs with SL-401 in patients with BPDCN, and in patients with AML who failed two previous treatments, i.e., third-line AML. In addition, given the breadth of SL-401 clinical activity seen to date, including clinical response in three indications: BPDCN, AML, and myelodysplastic syndrome, or MDS; the robust preclinical activity demonstrated in multiple leukemia and lymphoma indications; and the widespread expression of the target of SL-401, the interleukin-3 receptor, or IL-3R, across the majority of hematologic cancers, we plan to initiate Phase 2 trials in many of these indications. These may include multiple myeloma, earlier stages of AML, MDS, chronic myeloid leukemia, or CML, Hodgkin's and certain non-Hodgkin's lymphomas, and other hematologic indications, including several rare malignancies and proliferative disorders, such as hairy cell leukemia, mastocytosis, basophilic leukemia and eosinophilic leukemia. In addition to SL-401, we have developed next generation product candidates, SL-501 and SL-101, directed to the same clinically-validated IL-3R target. Each of these preclinical stage compounds has distinguishing characteristics SL-501 is a high affinity variant of SL-401 with elevated potency against IL-3R+ malignancies, and SL-101 is a monoclonal antibody-conjugate directed to the IL-3R alpha chain (CD123) also with high potency against IL-3R+ malignancies. We plan to advance SL-501, SL-101, and possibly other preclinical candidates, through investigational new drug, or IND, enabling studies and potentially into clinical trials. We plan to complete a Phase 2b trial of SL-701 in adult patients with glioblastoma multiforme, or GBM, who failed one previous

Table of Contents

treatment, i.e., second-line GBM. In addition, we plan to complete a Phase 2 trial of SL-701 in pediatric patients with brainstem and non-brainstem high-grade glioma.

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

As a leader in the CSC field, we intend to develop and commercialize our lead product candidates, advance our preclinical pipeline, and utilize our discovery platform, StemScreen®, to identify new drug candidates for additional indications. Our most advanced product candidates are SL-401 and SL-701.

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 including many leukemias and lymphomas. In a completed investigator-sponsored Phase 1/2 clinical trial in 83 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 six CRs in patients: four CRs in BPDCN and two CRs in AML. Notably, 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 of the 16 most heavily pretreated AML patients by more than three-fold compared with the historical median OS. Further, SL-401 has not demonstrated toxicity 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 both tumor bulk and CSCs, and are often toxic to

Table of Contents

the bone marrow. Accordingly, by pursuing hematologic cancer indications with SL-401, a therapeutic that uniquely targets both CSCs and tumor bulk and has not demonstrated toxicity to the bone marrow, we hope to provide benefit to patients who historically have been difficult to treat with traditional therapies.

We plan to initiate Company-sponsored clinical trials in 2014 with SL-401 in multiple hematologic cancers. We plan to initiate a Phase 2b trial in BPDCN, with overall response rate, or ORR, as the primary endpoint, and a Phase 3 trial in AML patients as a third-line treatment, with OS as the primary endpoint, and believe that these studies can serve as pivotal trials. In addition, we plan to initiate Phase 2 trials of SL-401 in additional hematologic cancers, which may include multiple myeloma, earlier stages of AML, MDS, CML, certain lymphomas, and other hematologic indications including several rare malignancies and proliferative disorders, such as hairy cell leukemia, mastocytosis, basophilic leukemia, and eosinophilic leukemia.

SL-701 is a clinically active subcutaneously-administered therapeutic cancer vaccine comprised of synthetic peptides, designed to direct the immune system to targets present on the CSCs and tumor bulk. In two completed investigator-sponsored Phase 1/2 clinical trials, SL-701 demonstrated single agent anti-tumor activity, which is uncommon for a cancer vaccine, including inducing tumor shrinkage or disease stabilization in 59% (13/22) of adult patients with recurrent or refractory high-grade glioma, or HGG (the 701 Adult-RHGG Study), and 87% (26/30) of pediatric glioma patients (the 701 Ped-G Study). To date, there have been eight major objective tumor responses (i.e., tumor regressions) in these two studies, consisting of two CRs and six partial responses, or PRs.

We plan to initiate Company-sponsored trials in 2014 with SL-701 in adult and pediatric brain cancers. We plan to advance SL-701 into a Phase 2b trial of adult patients with second-line GBM. The design of this study may enable SL-701 to obtain accelerated regulatory approval or serve as the foundation for a subsequent Phase 3 pivotal trial in this indication. We also plan to initiate a Phase 2 trial of SL-701 in pediatric patients with brainstem and non-brainstem high-grade glioma, which are areas of unmet medical need.

We plan to build out and advance our pipeline of product candidates. In particular, we plan to advance SL-501 and SL-101, our next generation IL-3R-targeting agents, through IND-enabling studies and potentially into clinical trials.

SL-501 is a rationally designed variant of SL-401 that binds to IL-3R with high affinity and has shown elevated potency against hematologic cancer cells both *in vitro* and in *in vivo* xenograft experiments.

SL-101 is a mAb-conjugate that binds to the alpha chain of IL-3R (CD123). SL-101 has demonstrated potent *in vitro* cytotoxic activity against several hematologic cancer cell lines.

We have developed an innovative platform technology, called StemScreen®, currently consisting of StemScreen®-1 and StemScreen®-2, for the identification of novel CSC-targeted compounds. This platform is differentiated from traditional drug discovery methods in oncology that have been designed to identify compounds that target only 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 can track and follow CSCs in their natural state during high throughput screening to permit the rapid testing of many compounds for potential anti-CSC activity. We believe that this approach represents a major technological advance in oncology drug discovery. We have utilized StemScreen® to discover several of our preclinical product candidates. We believe that this

Table of Contents

robust platform will be instrumental in the discovery of additional new therapies targeting a wide range of cancer types.

Our intellectual property portfolio contains 15 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 patent applications around (i) Stemline's drug candidates and (ii) general CSC-focused intellectual property directed to CSC therapeutics, diagnostics, including companion diagnostics, and drug discovery.

Management

We are led by a team with extensive experience in managing biopharmaceutical companies and in oncology drug development, including:

Ivan Bergstein, M.D. Chairman, Chief Executive Officer and President. Dr. Bergstein is Chief Executive Officer and Founder of Stemline Therapeutics. He led Stemline through multiple rounds of private financing and ultimately its successful initial public offering and subsequent follow-on offering, raising over $100 million as a public company. Prior to founding Stemline, Dr. Bergstein was Medical Director of Access Oncology, Inc., a clinical stage oncology-focused biotechnology company where he was a key member of a small team responsible for the acquisition and development of the company's clinical stage assets and ultimately the sale of the company. Previously, he was a senior biopharmaceuticals analyst at a Wall Street-based firm that advised mutual funds and hedge funds on investments in public companies with late clinical stage assets. He completed an internal medicine residency and hematology-oncology fellowship at the New York Presbyterian Hospital Weill Medical College of Cornell University.

Eric K. Rowinsky, M.D. Executive Vice President, Chief Medical Officer and Head of Research and Development. Dr. Rowinsky was previously the Chief Medical Officer for ImClone Systems, Inc. Dr. Rowinsky has more than 25 years of experience managing clinical trials and developing drugs in oncology, including leading the FDA approval of Erbitux® for head and neck and colorectal cancers and advancing eight other biological therapeutics through clinical development while at ImClone. He has also played integral roles in the development and registration of a wide range of cancer therapeutics, including paclitaxel, docetaxel, irinotecan, topotecan, erlotinib, gefitinib, panitumumab, lapatinib, and temsirolimus, among others. Dr. Rowinsky currently serves on the Board of Directors of Biogen Idec Inc., as well as several other public biopharmaceutical companies.

Kenneth Hoberman Chief Operating Officer. Mr. Hoberman was previously Vice President of Corporate and Business Development of Keryx Biopharmaceuticals, Inc., where he was instrumental in securing multiple sources of capital including over $200 million in equity investments through public and private offerings. He also initiated and executed a $100 million strategic alliance and originated, negotiated and closed dozens of licensing and operational contracts, and helped grow the company to a $900 million market capitalization.

Stephen P. Hall Vice President of Finance and Chief Accounting Officer. During the past 20 years, Mr. Hall has held senior positions, including Chief Financial Officer, Chief Accounting Officer, and Treasurer of multiple life science companies. These include Orthocon Inc, Helicos Biosciences and TriPath Imaging, a public oncology company that was subsequently acquired by Becton Dickinson and Company where Mr. Hall continued to serve as a Senior Advisor. He was also founder and managing director of Deimos Consulting LLC, a consulting firm specializing in life sciences. He earned an A.B. degree from Harvard College and an MBA from the Stanford Graduate School of Business.

Table of Contents

Strategy

Our goal is to maintain and fortify a leadership position in the discovery, acquisition and development of novel oncology therapies that target CSCs and to build a fully integrated pharmaceutical company with commercial infrastructure to support the marketing of our CSC targeted oncology drugs, if approved. The fundamental components of our business strategy to achieve this goal include the following:

Develop and commercialize SL-401 in multiple hematological cancers. We plan to initiate Company-sponsored clinical trials in 2014 with SL-401 in multiple hematologic cancers. We plan to initiate pivotal programs in BPDCN and in third-line AML. In particular, we plan to initiate a Phase 2b single-arm trial in patients with BPDCN with overall response rate, or ORR, as the primary endpoint, and a Phase 3 randomized trial as a third-line treatment for patients with AML, with OS as the primary endpoint. We intend to conduct these trials in North America and Europe, and we believe these trials will serve as pivotal trials in these territories. BPDCN and AML are orphan indications, which are rare diseases or conditions affecting fewer than 200,000 people in the United States, and each represents an unmet medical need. We also plan to initiate multiple Phase 2 trials of SL-401 in additional hematologic cancers, which may include multiple myeloma, earlier stages of AML (i.e., second- and first-line; either combined with standard treatment regimens or as a single agent as maintenance therapy for minimum residual disease, or MRD), MDS, CML, Hodgkin's and certain non-Hodgkin's lymphomas, and other hematologic indications including several rare diseases, such as hairy cell leukemia, mastocytosis, basophilic leukemia, and eosinophilic leukemia. We believe that some of these trials could be expanded to serve as platform trials for potential registration. Accordingly, we believe that SL-401 could be active in many hematologic cancer indications, thereby representing significant market opportunities.

Develop and commercialize SL-701 in brain cancer. We plan to initiate Company-sponsored trials in 2014 with SL-701 adult and pediatric brain cancers. We plan to initiate a Phase 2b trial of SL-701 in adult patients with second-line GBM. The design of the Phase 2b study may enable SL-701 to obtain accelerated regulatory approval or serve as the foundation for a subsequent Phase 3 pivotal trial in this indication. We also plan to initiate a Phase 2 trial of SL-701 in pediatric patients with brainstem and non-brainstem high-grade glioma, which are areas of unmet medical need.

Advance and build out our pipeline. We also plan to advance and build out our pipeline of product candidates. In particular, we plan to advance SL-501 and SL-101, our next generation IL-3R-targeting agents through IND-enabling studies and potentially into clinical trials.

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

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

Table of Contents

Continue to both leverage and fortify our intellectual property portfolio. We believe that we have a strong intellectual property position relating to the development and commercialization of our product candidates and technology. 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 the status of our two most advanced product candidates:

Risks associated with our business

Our business is subject to a number of risks of which you should be aware before making an investment decision. These risks are discussed more fully in the "Risk Factors" section of this prospectus beginning on page 11. In particular:

We currently have no commercial products, and we have not received regulatory approval for any of our product candidates.

We are heavily dependent on the success of our two lead product candidates, SL-401 and SL-701. Positive results in the completed Phase 1/2 clinical trials of SL-401 and SL-701 may not be predictive of the results in our planned clinical trials of SL-401 and SL-701.

Our clinical trials may not be successful. If

Table of Contents

we are unable to obtain required regulatory approvals of, commercialize, manufacture, obtain or maintain patent protection for, or gain sufficient market acceptance by physicians, patients and healthcare payors of our product candidates, or experience significant delays in doing so, our business will be materially harmed and our ability to generate revenue will be materially impaired.

Our approach to the discovery and development of product candidates that target CSCs is unproven. Research on CSCs is an emerging field and, consequently, there is ongoing debate regarding the importance of CSCs as an underlying cause of tumor initiation, propagation, recurrence and metastasis (the cancer spreading from the initial site to other parts of the body), resistance, as well as the defining characteristics and origins of CSCs. To date, we do not believe that any drugs have been successfully developed to target CSCs for the treatment of cancer.

We will require additional financing, in addition to the net proceeds of this offering, to achieve our goals including generation of revenue for the Company. The proceeds from this offering will not be sufficient to complete all of our planned clinical trials in their entirety. A failure to obtain additional financing when needed could force us to delay, limit, reduce or terminate our product development or commercialization efforts.

We face substantial competition, which may result in others discovering, developing or commercializing products before, or more successfully, than we do. We expect any product candidates that we commercialize will compete with products from other companies in the biotechnology and pharmaceutical industries. Our potential competitors may have greater commercial infrastructures and financial, technical and personnel resources than we have. If we are not able to compete effectively against our competitors, our business will not grow and our financial condition and operations will suffer.

Our inability to obtain or maintain adequate patent protection for our product candidates or platform technology or 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. In addition, SL-401 and SL-701 are protected by patents exclusively licensed from third parties. If the licensors terminate the licenses or fail to maintain or enforce the underlying patents, our competitive position will be materially harmed. Any challenges relating to our intellectual property may require us to spend a substantial amount of time and money to resolve.

We have incurred net operating losses since our inception and, to date, we have not generated any revenues. We expect to incur net operating losses for the foreseeable future and may never achieve or maintain profitability.

Our corporate information

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

We are an "emerging growth company," as defined in the Jumpstart Our Business Startups Act of 2012. We will remain an "emerging growth company" until the earliest of (i) the last day of the fiscal year in which we have total annual gross revenues of $1 billion or more; (ii) December 31, 2018; (iii) the date on which we have issued more than $1 billion in nonconvertible debt during the previous three years; or (iv) the date on which we are deemed to be a large accelerated filer under the rules of the Securities and Exchange Commission, or the SEC. We refer to the Jumpstart Our Business Startups Act of 2012 herein as

Table of Contents

the "JOBS Act," and references herein to "emerging growth company" shall have the meaning associated with it in the JOBS Act.

Our website address is www.stemline.com. The information contained on, or that can be accessed through, our website is not a part of this prospectus. We have included our website address in this prospectus solely as an inactive textual reference. We are not including the information on our website as a part of, nor incorporating it by reference into, this prospectus.

In this prospectus, unless otherwise stated or the context otherwise requires, references to "Stemline," "we," "us," "our," "Company" and similar references refer to Stemline Therapeutics, Inc. The Stemline name and logo and StemScreen® are our trademarks. The other trademarks, trade names and service marks appearing in this prospectus are the property of their respective owners.

This prospectus 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.

Table of Contents

The offering

Common stock offered by us	shares of our common stock
Common stock to be outstanding after this offering	shares
Option to purchase additional shares	The underwriters have an option for a period of 30 days to purchase up to additional shares of our common stock.
Use of proceeds	We intend to use the net proceeds from this offering for clinical development of SL-401 in multiple hematologic cancer indications, including planned pivotal programs in BPDCN and third-line AML; clinical development of SL-701 in adult second-line GBM and in pediatric brainstem and non-brainstem glioma; development of our preclinical pipeline, including advancing SL-501 and SL-101; development of our drug discovery platform; and other general corporate purposes. See "Use of Proceeds" beginning on page 41.
Risk factors	You should read the "Risk Factors" section starting on page 11 of this prospectus for a discussion of factors to consider carefully before deciding to invest in shares of our common stock.
NASDAQ Capital Market symbol	STML

The number of shares of our common stock outstanding after this offering is based on 12,890,965 actual shares of our common stock outstanding as of September 30, 2013.

The number of shares of our common stock outstanding after this offering excludes:

- 1,484,315 shares of our common stock issuable upon the exercise of stock options outstanding as of September 30, 2013, at a weighted-average exercise price of $4.27 per share, of which 956,174 shares were vested as of such date;
- 99,529 shares of common stock issuable upon the exercise of warrants outstanding as of September 30, 2013, at an exercise price of $15.00 per share, none of which were vested as of such date; and
- 1,290,613 shares of our common stock available for future issuance under our 2012 Equity Incentive Plan, or the 2012 Equity Plan, as of September 30, 2013, plus any future increases in the number of shares reserved for issuance under the 2012 Equity Plan pursuant to evergreen provisions.

Unless otherwise indicated, all information in this prospectus assumes:

- no exercise of the outstanding options or warrants; and
- no exercise by the underwriters of the option to purchase up to additional shares of our common stock.

Table of Contents

Summary financial data

You should read the following summary financial data together with our financial statements and the related notes and "Management's Discussion and Analysis of Financial Condition and Results of Operations" incorporated by reference from our Annual Report on Form 10-K for the year ended December 31, 2012, and our Quarterly Report on Form 10-Q for the quarter ended June 30, 2013, together with the sections entitled "Capitalization," "Selected Financial Data" and "Risk Factors" included in this prospectus.

We have derived the statements of operations data for the years ended December 31, 2010, 2011 and 2012 and the balance sheet data as of December 31, 2012 from our audited financial statements included in our Annual Report on Form 10-K for the year ended December 31, 2012, which is incorporated by reference into this prospectus. We have derived the statements of operations data for the six months ended June 30, 2012 and 2013, and the balance sheet data as of June 30, 2013 from our unaudited financial statements included in our Quarterly Report on Form 10-Q for the quarter ended June 30, 2013, which is incorporated by reference into this prospectus. In our opinion, such unaudited financial statements include all adjustments, consisting only of normal recurring adjustments that we consider necessary for a fair presentation of the financial information set forth in those statements. Our historical results for any prior period are not necessarily indicative of results to be expected in any future period and the results for the six months ended June 30, 2013 are not necessarily indicative of the results that may be expected for the full fiscal year. For more details on how you can obtain the documents incorporated by reference in this prospectus, see "Where You Can Find More Information" and "Incorporation of Documents by Reference."

Table of Contents

	Year ended December 31,			Six months ended June 30,		Period from August 8, 2003 (inception) to June 30,
	2010	2011	2012	2012 (unaudited)	2013 (unaudited)	2013 (unaudited)
Statement of operations data:						
Operating expenses:						
Research and development	$ 1,329,509	$ 1,629,026	$ 3,376,962	$ 1,613,340	$ 7,246,247	$ 18,691,915
General and administrative	930,331	1,088,028	3,090,611	1,372,152	3,238,893	12,723,689
Total operating expenses	2,259,840	2,717,054	6,467,573	2,985,492	10,485,140	31,415,604
Loss from operations	(2,259,840)	(2,717,054)	(6,467,573)	(2,985,492)	(10,485,140)	(31,415,604)
Other income:	484,905	46,673	301,684	12,460	30,649	966,167
Other expense		(9,670)	(35)	(35)		(9,705)
Interest expense	(69,493)	(98,643)	(118,765)	(50,038)	(505,037)	(801,987)
Interest income	43,045	24,068	9,907	7,882	3,244	963,827
Net loss	$ (1,801,383)	$ (2,754,626)	$ (6,274,782)	$ (3,015,223)	$ (10,956,284)	$ (30,297,302)
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 (loss) / income attributable to common stockholders	$ 10,130,662	$ (2,754,626)	(6,274,782)	$ (3,015,223)	$ (10,956,284)	$ (20,716,702)
Net (loss) / income attributable to common stockholders per common share:						
Basic	$ 3.07	$ (0.80)	$ (1.82)	$ (0.88)	$ (1.37)	
Diluted	$ 2.81	$ (0.80)	$ (1.82)	$ (0.88)	$ (1.37)	
Weighted average number of common shares:						
Basic	3,298,793	3,441,995	3,441,995	3,441,995	8,014,529	
Diluted	3,607,030	3,441,995	3,441,995	3,441,995	8,014,529	

The following summary unaudited balance sheet data as of June 30, 2013 is presented:

on an actual basis; and

on an as adjusted basis to give effect to our sale of shares of common stock in this offering at the assumed offering price of $ per share, which was the last reported closing price of our common stock on The NASDAQ Capital Market on October , 2013, after deducting underwriting discounts and commissions and estimated offering expenses payable by us.

Table of Contents

The summary unaudited as adjusted balance sheet is for informational purposes only and does not purport to indicate balance sheet information as of any future date.

		June 30, 2013
	Actual (unaudited)	As adjusted(1)
Balance sheet data:		
Cash and cash equivalents	$ 92,685,912	$
Total assets	93,848,091	
Deficit accumulated during development stage	(18,125,539)	(18,125,539)
Total stockholders' equity	89,675,368	

(1) Each $1.00 increase (decrease) in the assumed public offering price of $ per share, the last reported sale price of our common stock on The NASDAQ Capital Market on October , 2013, would increase (decrease) the as adjusted amount of cash and cash equivalents, total stockholders' equity and total capitalization by approximately $ million , assuming the number of shares offered by us, as set forth on the cover page of this prospectus, remains the same and after deducting the estimated underwriting discounts and commissions and estimated offering expenses payable by us. We may also increase or decrease the number of shares we are offering. Each increase (decrease) of 100,000 shares in the number of shares offered by us would increase (decrease) the as adjusted amount of cash and cash equivalents and investment securities, total assets, and total stockholders' equity by approximately $ million, assuming that the assumed public offering price remains the same, and after deducting the estimated underwriting discounts and commissions and estimated offering expenses payable by us. The as adjusted information discussed above is illustrative only and will be adjusted based on the actual public offering price and other terms of this offering determined at pricing.

Recent developments

On September 30, 2013, we had cash and cash equivalents of approximately $87.7 million, as compared to $2.0 million and $92.7 million, as of December 31, 2012 and June 30, 2013, respectively. The increase from December 31, 2012 was primarily the result of our initial public offering completed in January 2013 and our secondary offering completed in May 2013.

Table of Contents

Risk factors

Investing in our common stock involves a high degree of risk. You should carefully consider the risks and uncertainties described below, together with all of the other information contained in this prospectus before deciding to invest in our common stock. The risk factors set forth below supersede in their entirety the risks and uncertainties discussed under "Risk Factors" in our Annual Report on Form 10-K for the year ended December 31, 2012, and our Quarterly Reports on Form 10-Q for the quarters ended March 31, 2013 and June 30, 2013. If any of the following risks actually occurs, our business, prospects, operating results and financial condition could suffer materially, the trading price of our common stock could decline and you could lose all or part of your investment.

Risks related to development, clinical testing and regulatory approval of our product candidates

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

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

Before we generate any revenues from product sales, we must complete preclinical and clinical development of one or more of our product candidates, conduct human subject research, submit clinical and manufacturing data to the FDA, qualify a third party contract manufacturer, receive regulatory approval in one or more jurisdictions, satisfy the FDA that our contract manufacturer is capable of manufacturing the product in compliance with cGMP, build a commercial organization, make substantial investments and undertake significant marketing efforts ourselves or in partnership with others. We are not permitted to market or promote any of our product candidates before we receive regulatory approval from the FDA or comparable foreign regulatory authorities, and we may never receive such regulatory approval for any of our product candidates.

We have not submitted a biologics license application, or BLA, or a new drug application, or NDA, to the FDA, or similar market approval applications to comparable foreign authorities, for any of our product candidates. We cannot be certain that any of our product candidates will be successful in clinical trials or receive regulatory approval. Further, our product candidates may not receive regulatory approval even if they are successful in clinical trials. If we do not receive regulatory approvals for our product candidates, we may not be able to continue our operations. Even if we successfully obtain regulatory approvals to market one or more of our product candidates, our revenues will be dependent, in part, upon the size of the markets in the territories for which we gain regulatory approval and have commercial rights. If the markets for patient subsets that we are targeting are not as significant as we estimate, we may not generate significant revenues from sales of such products, if approved.

We plan to seek regulatory approval to commercialize our product candidates in the United States, the European Union and potentially in additional foreign countries. While the scope of regulatory review and approval is similar in other countries, to obtain separate regulatory review and approval in many other countries we must comply with numerous and varying regulatory requirements of such countries regarding

Table of Contents

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

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

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

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

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

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

- the FDA requiring alterations to any of our study designs, overall strategy or manufacturing plans;

- delays in patient enrollment, variability in the number and types of patients available for clinical trials, poor accrual, or high drop-out rates of patients in our clinical trials;

- clinical trial sites deviating from trial protocol or dropping out of a trial and our inability to add new clinical trial sites;

- difficulty in maintaining contact with patients after treatment, resulting in incomplete data;

- poor effectiveness of our product candidates during clinical trials;

- safety issues, including serious adverse events associated with our product candidates and patients' exposure to unacceptable health risks;

- receipt by a competitor of marketing approval for a product targeting an indication that one of our product candidates targets, such that we are not "first to market" with our product candidate;

- governmental or regulatory delays and changes in regulatory requirements, policy and guidelines; or

differing interpretations of data by the FDA or similar foreign regulatory agencies.

We could also encounter delays if a clinical trial is suspended or terminated by us, by the IRBs of the institutions in which such trial is being conducted, by the Data Safety Monitoring Board, or DSMB, if one is utilized for any such trial, or by the FDA or other regulatory authorities. Such authorities may suspend or terminate a clinical trial due to a number of factors, including, among other things, failure to conduct the clinical trial in accordance with regulatory requirements or our clinical protocols, inspection of the clinical

Table of Contents

trial operations or trial site by the FDA or other regulatory authorities resulting in the imposition of a clinical hold, unforeseen safety issues or adverse side effects, failure to demonstrate a benefit from using a drug, changes in governmental regulations or administrative actions or lack of adequate funding to continue the clinical trial.

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

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

If we experience delays in the completion of, or termination of, any clinical trial of our product candidates, the commercial prospects of our product candidates will be harmed, which will have a negative impact on our ability to commence product sales and generate product revenues from any of our product candidates. In addition, any delays in completing our clinical trials will increase our costs and slow down our product candidate development and approval process. Delays in completing our clinical trials could also allow our competitors to obtain marketing approval before we do or shorten the patent protection period during which we may have the exclusive right to commercialize our product candidates. Any of these occurrences may harm our business, financial condition and prospects significantly. In addition, many of the factors that cause, or lead to, a delay in the commencement or completion of clinical trials may also ultimately lead to the denial of regulatory approval of our product candidates.

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

The results of preclinical studies and early-stage clinical trials of product candidates may not be predictive of the results of subsequent later-stage clinical trials. Product candidates in later-stage clinical trials may fail to show the safety and efficacy results demonstrated in earlier studies despite having progressed through preclinical studies and earlier clinical trials. Many companies in the biopharmaceutical industry have suffered significant setbacks in later-stage clinical trials due to adverse safety profiles or lack of efficacy, notwithstanding promising results in earlier studies. Similarly, our future clinical trial results may not be successful for these or other reasons.

This drug candidate development risk is heightened by any changes in planned clinical trials compared to completed clinical trials. As product candidates are advanced through preclinical studies to early and later stage clinical trials towards approval and commercialization, it is customary that various aspects of the development program, such as manufacturing and methods of administration and dosing, are altered along the way in an effort to optimize processes and results. While these types of changes are common and are intended to optimize the product candidates for late stage clinical trials, approval and commercialization,

Table of Contents

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 patients receive 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 greater clinical benefit than from a single cycle, there is always the risk of unforeseen toxicity or a lack of efficacy arising from multiple cycles.

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

- 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 Survivin 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 adjuvant used in the injection of SL-701 to include a commercially available and more 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, radiation, targeted therapy, or anti-angiogenic therapy. We have not yet clinically tested these combinations. While there do not appear to be overlapping toxicities with these combinations, there is always the risk of unforeseen toxicities. Accordingly, we plan to conduct early analyses of safety in such trials and make any appropriate adjustments, if necessary.

Any of these aforementioned, or other, 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

Table of Contents

our clinical trials, delay approval of our product candidates, and/or jeopardize our ability to commence product sales and generate revenues.

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

We may not be able to initiate or continue clinical trials for our product candidates if we are unable to locate and enroll a sufficient number of eligible patients to participate in these trials as required by the FDA or other regulatory authorities. Patient enrollment, a significant factor in the timing of clinical trials, is affected by many factors including the size and nature of the patient population, the proximity of patients to clinical sites, the eligibility criteria for the trial, the design of the clinical trial, competing clinical trials and clinicians' and patients' perceptions as to the potential advantages of the drug being studied in relation to other available therapies, including any new drugs that may be approved for the indications we are investigating. SL-401 and SL-701 are being developed in hematologic and brain cancers, respectively, both of which are orphan indications (i.e., rare diseases). In particular, SL-401 is being developed initially in BPDCN and AML; and SL-701 is being developed in adult and 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 of these diseases are very low, including lower than our estimates or estimates of our third party contractors, this could significantly delay patient enrollment in any one or more of our planned clinical trials.

If we fail to enroll and maintain the number of patients for which the clinical trial was designed, the statistical power of that clinical trial may be reduced, which would make it harder to demonstrate that the product candidate being tested in such clinical trial is safe and effective. Additionally, enrollment delays in our clinical trials may result in increased development costs for our product candidates, which would cause the value of our common stock to decline and limit our ability to obtain additional financing. Our inability to enroll a sufficient number of patients for any of our current or future clinical trials would result in significant delays or may require us to abandon one or more clinical trials altogether.

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

The time required to obtain approval by the FDA and comparable foreign authorities is unpredictable and depends upon numerous factors, including the substantial discretion of the regulatory authorities. In addition, review and 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;

Table of Contents

- 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 the data may not be sufficient to support the submission of a BLA or an NDA or other submission or to obtain regulatory approval in the United States or elsewhere;

- the FDA or comparable foreign regulatory authorities may fail to approve the manufacturing processes or facilities of third-party manufacturers with which we contract for clinical and commercial supplies;

- the FDA or comparable foreign regulatory authorities may fail to approve any companion diagnostics we develop; and

- the approval policies or regulations of the FDA or comparable foreign regulatory authorities may significantly change in a manner rendering our clinical data insufficient for approval.

This lengthy and costly review process as well as the unpredictability of future clinical trial results may result in our failing to obtain regulatory approval to market SL-401 and SL-701, or any of our other product candidates that we may advance into clinical trials, which would significantly harm our business.

In addition, even if we were to obtain approval, regulatory authorities may approve any of our product candidates for fewer or more limited indications than we request, may grant approval contingent on the performance of costly post-marketing clinical trials, or may approve a product candidate with a label that does not include the labeling claims necessary or desirable for the successful commercialization of that product candidate. In addition, we may not be able to ultimately achieve the price we intend to charge for our product candidates. Moreover, in many foreign countries, a product candidate must be approved for reimbursement before it can be approved for sale in that country. Any of the foregoing scenarios could materially harm the commercial prospects for our product candidates.

Our approach to the discovery and development of product candidates that target CSCs is unproven, and we do not know whether we will be able to develop any products of commercial value.

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

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

Table of Contents

that, at times or always, targeting CSCs should be coupled with targeting tumor bulk for a positive clinical outcome.

We believe that SL-401 and SL-701 target both tumor bulk and CSCs. However, it is conceivable that SL-401, SL-701 and any other product candidates that we develop, including SL-101 and SL-501, may not effectively target tumor bulk or CSCs or, even if they do, they may not have a beneficial clinical outcome. In addition, it is conceivable that our platform technology may ultimately fail to identify any commercially viable drugs to treat human patients with cancer.

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

Although we expect to focus a substantial amount of our efforts on the continued clinical testing and potential approval of SL-401 and SL-701, another key element of our strategy is to identify and test additional compounds that target CSCs in a variety of different types of cancer, including SL-501 and SL-101. A portion of the research that we are conducting involves new and unproven drug discovery methods, including our proprietary StemScreen® platform technology, as well as the testing of new compounds and potential new uses of existing compounds. The drug discovery that we are conducting using our StemScreen® platform technology may not be successful in identifying compounds that are useful in treating humans with cancer. Research programs designed to identify product candidates require substantial technical, financial and human resources, whether or not any product candidates are ultimately identified. Our research programs may initially show promise in identifying potential product candidates, yet fail to yield product candidates for clinical development or commercialization for many reasons, including the following:

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

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

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

Any regulatory approvals that we or our potential strategic partners receive for our product candidates may also be subject to limitations on the approved indicated uses for which the product may be marketed or to the conditions of approval, or contain requirements for potentially costly post-marketing testing,

Table of Contents

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 June 30, 2013 of $30.3 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 and cash equivalents, together with the net proceeds from this offering, will be sufficient to fund our operations and our capital expenditures for at least the next several years. If our cash is insufficient to meet future operating requirements, we will have to raise additional funds. If we are unable to obtain additional funds on terms favorable to us or at all, we may be required to cease or reduce our operating activities or sell or license to third parties some or all of our intellectual property. If we raise additional funds by selling additional shares of our capital stock, the ownership interests of our stockholders will be diluted. If we need to raise additional funds through the sale or license of our intellectual property, we may be unable to do so on terms favorable to us, if at all. In addition, if we do not continue to meet our diligence obligations under our license agreements for our product candidates that we have in-licensed, including

Table of Contents

SL-401, SL-701, SL-501 and SL-101, we will lose our rights to develop and commercialize those product candidates.

If we do not successfully develop and obtain regulatory approval for our existing and future product candidates and effectively manufacture, market and sell any product candidates that are approved, we may never generate product sales, and even if we do generate product sales, we may never achieve or sustain profitability on a quarterly or annual basis. Our failure to become and remain profitable would depress the market price of our common stock and could impair our ability to raise capital, expand our business, diversify our product offerings or continue our operations. A decline in the market price of our common stock also could cause you to lose all or a part of your investment.

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

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

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

Our future capital requirements depend on many factors, including:

- the number and characteristics of the product candidates we pursue;
- the scope, progress, results and costs of researching and developing our product candidates, and conducting preclinical and clinical trials;
- the ability of our product candidates to progress through clinical development successfully;
- the timing of, and the costs involved in, obtaining regulatory approvals for our product candidates;
- the cost of commercialization activities if any of our product candidates are approved for sale, including marketing, sales and distribution costs;
- the cost associated with securing and establishing commercialization and manufacturing capabilities for our product candidates and any products we successfully commercialize;

Table of Contents

- our ability to establish and maintain strategic partnerships, licensing or other arrangements and the economic and other terms of such agreements;

- the costs involved in preparing, filing, prosecuting, maintaining, defending and enforcing patent claims, including litigation costs and the outcome of such litigation;

- the timing, receipt and amount of sales of, or royalties on, our future products, if any;

- our need and ability to hire additional management and scientific and medical personnel;

- the effect of competing technological and market developments; and

- our need to implement additional internal systems and infrastructure, including financial and reporting systems.

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

- delay, limit, reduce or terminate preclinical studies, clinical trials or other research and development activities for one or more of our product candidates; or

- delay, limit, reduce or terminate our establishment of sales and marketing capabilities or other activities that may be necessary to commercialize our product candidates.

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

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

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

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

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

Table of Contents

a risk that one or more of our current service providers, manufacturers and other partners may not survive these difficult economic times, which could directly affect our ability to attain our operating goals on schedule and on budget. There is a possibility that our stock price may decline, due in part to the volatility of the stock market and the general economic downturn.

Risks related to our business and industry

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

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

- execute product candidate development activities;
- obtain required regulatory approvals for the development and commercialization of our product candidates;
- maintain, leverage and expand our intellectual property portfolio;
- build and maintain robust sales, distribution and marketing capabilities, either on our own or in collaboration with strategic partners;
- gain market acceptance for our products;
- develop and maintain any strategic relationships we elect to enter into;
- satisfy our obligations under our in-license agreements; and
- manage our spending as costs and expenses increase due to drug discovery, preclinical development, clinical trials, regulatory approvals 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,

Table of Contents

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, 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., Northwest Biotherapeutics, Inc., Agenus, Inc., Merck & Co. Inc., Novartis AG, Roche Holding AG and others.

Many of our competitors have substantially greater commercial infrastructures and financial, technical and personnel resources than we have. We may not be able to compete successfully unless we successfully:

- design and develop products that are superior to other products in the market;
- attract qualified scientific, medical, sales and marketing and commercial personnel;
- obtain patent and/or other proprietary protection for our processes and product candidates;
- obtain required regulatory approvals; and
- collaborate with others in the design, development and commercialization of new products.

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

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

Our success depends in part on our continued ability to attract, retain and motivate highly qualified management, clinical and scientific personnel. We are highly dependent upon our senior management, particularly Ivan Bergstein, M.D., our Chairman, Chief Executive Officer and President, and Eric K. Rowinsky, M.D., our Executive Vice President, Chief Medical Officer and Head of Research and Development, as well as other employees, consultants and scientific and medical collaborators. As of September 30, 2013, we had 12 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 senior management has informed us to date that he or she intends to retire or resign in the near future, the loss of services of any of these individuals or one or more of our other members of senior management could delay or prevent the successful development of our product pipeline, completion of our planned clinical trials or the commercialization of our product candidates.

Although we have not historically experienced unique difficulties attracting and retaining qualified employees, we could experience such problems in the future. For example, competition for qualified personnel in the biotechnology and pharmaceuticals field is intense. We will need to hire additional

Table of Contents

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, our business may experience serious adverse consequences.

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

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

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

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

We face an inherent risk of product liability as a result of the clinical testing of our product candidates and will face an even greater risk if we commercialize any products. For example, we may be sued if any product we develop allegedly causes injury or is found to be otherwise unsuitable during product testing,

Table of Contents

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

Table of Contents

are false or fraudulent or making a false statement to avoid, decrease or conceal an obligation to pay money to the federal government and also includes provisions allowing for private, civil whistleblower or "qui tam" actions;

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

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

the federal transparency requirements under the Health Care Reform Law requires manufacturers of drugs, devices, biologics and medical supplies to report to the Department of Health and Human Services information related to physician payments and other transfers of value and physician ownership and investment interests;

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

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

Efforts to ensure that our business arrangements with third parties will comply with applicable healthcare laws and regulations will involve substantial costs. It is possible that governmental authorities will conclude that our business practices do not comply with current or future statutes, regulations or case law involving applicable fraud and abuse or other healthcare laws and regulations. If our operations are found to be in violation of any of these laws or any other governmental regulations that may apply to us, we may be subject to significant civil, criminal and administrative penalties, damages, fines, exclusion from government funded healthcare programs, such as Medicare and Medicaid, and the curtailment or restructuring of our operations. If any of the physicians or other providers or entities with whom we expect to do business are found to be not in compliance with applicable laws, they may be subject to criminal, civil or administrative sanctions, including exclusions from government funded healthcare programs.

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

Table of Contents

materials and wastes. Our operations involve the use of hazardous and flammable materials, including chemicals and biological materials. Our operations also produce hazardous waste products. We generally contract with third parties for the disposal of these materials and wastes. We cannot eliminate the risk of contamination or injury from these materials. In the event of contamination or injury resulting from our use of hazardous materials, we could be held liable for any resulting damages, and any liability could exceed our resources. We also could incur significant costs associated with civil or criminal fines and penalties.

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

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

Risks related to commercialization of our product candidates

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

We currently have a relatively small number of employees and do not have a sales or marketing infrastructure, and we, including our executive officers, do not have any significant sales, marketing or distribution experience. We will be opportunistic in seeking to either build our own commercial infrastructure to commercialize SL-401, SL-701 and any future product candidates if and when they are approved, or enter into licensing or collaboration agreements to assist in the future development and commercialization of such product candidates.

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

- our inability to recruit and retain adequate numbers of effective sales and marketing personnel;
- the inability of sales personnel to obtain access to physicians or persuade adequate numbers of physicians to prescribe any future products;
- the lack of complementary products to be offered by sales personnel, which may put us at a competitive disadvantage relative to companies with more extensive product lines; 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

Table of Contents

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 and other healthcare providers, patients, third-party payors and, in the cancer market, acceptance by the major operators of cancer clinics.

Even if SL-401, SL-701 or any other product candidate that we may develop or acquire in the future obtains regulatory approval, the product may not gain market acceptance among physicians, healthcare 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.

Table of Contents

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

The regulations that govern marketing approvals, pricing and reimbursement for new drug products vary widely from country to country. In the United States, recently passed legislation may significantly change the approval requirements in ways that could involve additional costs and cause delays in obtaining approvals. Some countries require approval of the sale price of a drug before it can be marketed. In many countries, the pricing review period begins after marketing or product licensing approval is granted. In some foreign markets, prescription pharmaceutical pricing remains subject to continuing governmental control even after initial approval is granted. As a result, we might obtain marketing approval for a product in a particular country, but then be subject to price regulations that delay our commercial launch of the product, possibly for lengthy time periods, and negatively impact the revenues we are able to generate from the sale of the product in that country. Adverse pricing limitations may hinder our ability to recoup our investment in one or more product candidates, even if our product candidates obtain marketing approval.

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

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

Table of Contents

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

Our business may be affected by the efforts of government and third-party payors to contain or reduce the cost of healthcare through various means. For example, the Patient Protection and Affordable Care Act and the Health Care and Education Affordability Reconciliation Act of 2010 (collectively, the Affordable Care Act or ACA), enacted in March 2010, substantially changed the way healthcare is financed by both governmental and private insurers, and significantly impacted the pharmaceutical industry. With regard to pharmaceutical products, among other things, the ACA is expected to expand and increase industry rebates for drugs covered under Medicaid programs and make changes to the coverage requirements under the Medicare Part D program. Although the Supreme Court has upheld the ACA in the main challenge to the constitutionality of the statute and the 2012 elections maintained divided government at the federal level, Congressional efforts to repeal the ACA continue. In addition, there may be Congressional efforts to expand the Medicaid drug rebate program to the Medicare Part D program (or to provide authority for the government to negotiate drug prices under the Medicare Part D program). This adds to the uncertainty of the legislative changes enacted as part of the ACA, and we cannot predict the impact that the ACA or any other legislative or regulatory proposals will have on our business. Regardless of whether or not the ACA is repealed, we expect both government and private health plans to continue to require healthcare providers, including healthcare providers that may one day purchase our products, to contain costs and demonstrate the value of the therapies they provide.

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

With the enactment of the Biologics Price Competition and Innovation Act of 2009, or BPCIA, as part of the Health Care Reform Law, an abbreviated pathway for the approval of biosimilar and interchangeable biological products was created. The new abbreviated regulatory pathway establishes legal authority for the FDA to review and approve biosimilar biologics, including the possible designation of a biosimilar as "interchangeable." The FDA defines an interchangeable biosimilar as a product that, in terms of safety or diminished efficacy, presents no greater risk when switching between the biosimilar and its reference product than the risk of using the reference product alone. Under the BPCIA, an application for a biosimilar product cannot be submitted to the FDA until four years, or approved by the FDA until 12 years, after the original brand product identified as the reference product was approved under a BLA. The new law is complex and is only beginning to be interpreted by the FDA. As a result, its ultimate impact, implementation and meaning are subject to uncertainty. While it is uncertain when any such processes may be fully adopted by the FDA, any such processes could have a material adverse effect on the future commercial prospects for our biological products.

We believe that if any of our product candidates, such as SL-401 or SL-701, were to be approved as biological products under a BLA, such approved products should qualify for the 12-year period of exclusivity. However, there is a risk that the U.S. Congress could amend the BPCIA to significantly shorten this exclusivity period as proposed by President Obama or that may be proposed by his successors, potentially creating the opportunity for generic competition sooner than anticipated. Moreover, the extent to which a biosimilar, once approved, will be substituted for any one of our reference products in a way that is similar to traditional generic substitution for non-biological products is not yet clear, and will depend on a number of marketplace and regulatory factors that are still developing. In addition, a competitor could decide to forego the biosimilar route and submit a full BLA after completing its own preclinical studies and clinical trials. In such cases, any exclusivity to which we may be eligible under the BPCIA would not prevent the competitor from marketing its product as soon as it is approved.

Table of Contents

Risks related to our dependence on third parties

Third parties have conducted all clinical trials of SL-401 and SL-701 so far, and our ability to influence the design and conduct of such trials was limited. Our plans to assume control over the future clinical and regulatory development of such product candidates will entail additional expenses and require us to rely on additional third parties. Any failure by a third party to meet its obligations with respect to the clinical and regulatory development of our product candidates may delay or impair our ability to obtain regulatory approval for our products.

To date, we have not sponsored any clinical trials relating to SL-401 or SL-701. Instead, faculty members at academic institutions have conducted and sponsored all clinical trials relating to SL-401 and SL-701 under their own INDs. Because the completed SL-401 and SL-701 clinical trials were investigator-sponsored, we did not control the design or conduct of the previous trials, and it is possible that the FDA will not view these previous trials as providing adequate support for future clinical trials, whether controlled by us or third parties, for any one or more reasons, including elements of the design or execution of the previous trials or safety concerns or other trial results.

While we plan to assume control of the overall clinical and regulatory development of SL-401 and SL-701 going forward, we have so far been dependent on contractual arrangements with each investigator and their respective academic institutions, and will continue to be until we assume control. Such arrangements provide us certain information rights with respect to the completed trials, including access to and the ability to use and reference the data, including for our own regulatory filings, resulting from the completed trials. If these obligations are breached by the investigators or institutions, or if the data prove to be inadequate compared to the first-hand knowledge we might have gained had the completed trials been Company-sponsored trials, then our ability to design and conduct our planned Company-sponsored clinical trials may be adversely affected. Additionally, the FDA may disagree with the adequacy of our interpretation of preclinical, manufacturing, or clinical data from these clinical trials. If so, the FDA may require us to obtain and submit additional preclinical, manufacturing, or clinical data before we may initiate our planned trials and/or may not accept such additional data as adequate to initiate our planned trials.

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 submitting our own IND for SL-401. We intend to assume control over the clinical development of SL-701 by submitting a Company-sponsored IND, for which we expect to exercise our rights of reference under our agreements with the University of Pittsburgh with respect to the existing INDs for 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 had less control over the timing and cost of these studies and the ability to recruit trial subjects than if we had conducted these trials wholly by ourselves. If we successfully

Table of Contents

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.

Table of Contents

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

We do not currently own or operate any manufacturing facilities, and we lack sufficient internal staff to produce clinical and preclinical product candidate supplies ourselves. As a result, we work with third-party contract manufacturers in an effort to produce sufficient quantities of SL-401 and SL-701 for future clinical trials, preclinical testing and commercialization. If we are unable to arrange for or maintain such 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, including SL-101 and SL-501. If we are unable to arrange for third-party manufacturing sources, or to do so on commercially reasonable terms or on a timely basis, we may not be able to complete development of such other product candidates or market them.

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

We rely on our third-party manufacturers to purchase from third-party suppliers the materials necessary to produce our product candidates for our clinical studies. There are a small number of suppliers for certain capital equipment and materials that we use to manufacture our drugs. Such suppliers may not sell these materials to our manufacturers at the times we need them or on commercially reasonable terms. We do not have 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

Table of Contents

commercial launch of our product candidates would be delayed or there would be a shortage in supply, which would impair our ability to generate revenues from the sale of our product candidates.

We are working with our contract manufacturer to optimize the manufacturing processes for SL-401 and SL-701 drug substance and drug product so that these product candidates may be produced in adequate quantities of adequate quality, and at an acceptable cost, to support our planned clinical trials and ultimate commercialization. Our manufacturer may not be able to adequately demonstrate that an optimized product candidate is comparable to a previously manufactured product candidate, which could cause significant delays and increased costs to our programs. Our manufacturer may not be able to manufacture our product candidates at a cost or in quantities or in a timely manner necessary to develop and commercialize them. If we successfully commercialize any of our drugs, we may be required to establish or access large-scale commercial manufacturing capabilities. In addition, assuming that our drug development pipeline increases and matures, we will have a greater need for clinical trial and commercial manufacturing capacity. To meet our projected needs for commercial manufacturing, third parties with whom we currently work will need to increase their scale of production or we will need to secure alternate suppliers.

We rely on a single third-party to manufacture and supply our product candidates. Any problems experienced by this vendor could result in a delay or interruption in the supply of our product candidates to us until this vendor cures the problem or until we locate and qualify an alternative source of supply.

The manufacturer of our product candidates requires specialized equipment and utilizes complicated production processes that would be difficult, time-consuming and costly to duplicate. We currently rely on a single third party to purchase from third-party suppliers the materials necessary to produce our product candidates for our clinical studies. Any prolonged disruption in the operations of our third-party manufacturer could have a significant negative impact on our ability to manufacture products on our own and would cause us to seek additional third-party manufacturing contracts, thereby increasing our development and any commercialization costs. We may suffer losses as a result of business interruptions that exceed coverage under our manufacturer's insurance policies. Events beyond our control, such as natural disasters, fire, sabotage or business accidents could have a significant negative impact on our operations by disrupting our product candidate development and commercialization efforts until our third-party manufacturer can repair its facility or put in place third-party contract manufacturers to assume this manufacturing role, which we may not be able to do on reasonable terms, if at all. In addition, if we are required to change manufacturers for any reason, we will be required to verify that the new manufacturer maintains facilities and procedures that comply with quality standards and with all applicable regulations and guidelines. The delays associated with the verification of a new manufacturer or the reverification of an existing manufacturer could negatively affect our ability to develop product candidates or produce approved products in a timely manner. Any delay or interruption in our clinical studies for the validation and commercialization of our product candidates could negatively affect our business.

To the extent we elect to enter into licensing or collaboration agreements to 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

Table of Contents

collaboration agreements, collaborations may involve greater uncertainty for us, as we have less control over certain aspects of our collaborative programs than we do over our proprietary development and commercialization programs. We may determine that continuing a collaboration under the terms provided is not in our best interest, and we may terminate the collaboration. Our collaborators could delay or terminate their agreements, and our products subject to collaborative arrangements may never be successfully commercialized.

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

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

Risks related to our intellectual property rights

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

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

Our patents and patent applications may not be sufficient to protect our products and product candidates from commercial competition. For example, we cannot obtain a composition of matter patent for SL-401 due to earlier published prior art. We have however obtained U.S. patents for certain methods of using SL-401 to treat AML and MDS. In addition, we have filed U.S. and foreign patent applications for the method of using SL-401 to treat AML, MDS, and BPDCN although there can be no assurances that such patents will be issued over the prior art. Failure to obtain patents directed to all approved uses of SL-401 would enable a competitor to market SL-401 for such unpatented indication(s), which could lead to price erosion for sales of SL-401 for our patented indications through off-label use. Although we have an issued U.S. patent directed to the composition of matter for 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

Table of Contents

increase a patient's immune response to SL-701, we do not have any foreign composition of matter patent protection. We do not expect that we will be able to obtain such protection outside the U.S. in the future although we do have foreign pending patent applications that seek to cover certain uses of this peptide. While we have a non-exclusive license to issued U.S. patents directed to methods of use for the EphA2 peptide used in SL-701, we do not have any composition of matter patent protection. We do not expect that we will be able to obtain such protection in the future. While we have patent applications pending in the United States and Canada directed to our StemScreen® technology, we currently have no issued patents covering StemScreen®.

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

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

If we were to initiate legal proceedings against a third party to enforce a patent covering one of our product candidates, the defendant could counterclaim that our patent is invalid and/or unenforceable. In patent litigation in the United States, defendant counterclaims alleging invalidity and/or unenforceability are commonplace. Grounds for a validity challenge could be an alleged failure to meet any of several statutory requirements, for example, lack of 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 product candidates or certain aspects of our platform technology, StemScreen®. Such a loss of patent protection could have a material adverse impact on our business.

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

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

Table of Contents

litigation, which could result in us having to cease commercialization of our products and subject us to money damages in such territories.

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

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

Table of Contents

liabilities, or require us to seek licenses that may not be available on commercially acceptable terms, if at all.

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

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

SL-401, SL-701, some of our other product candidates and our platform technology are protected by intellectual property licensed from academic institutions. If the licensors terminate the licenses or fail to prosecute patent applications or maintain or enforce the underlying patents, our competitive position, market share, and business prospects will be harmed.

We are a party to several license agreements relating to certain patents and/or patent applications owned by other institutions, upon which certain aspects of our business depend. In particular, we hold an exclusive license from Scott and White Memorial Hospital, or Scott and White, for SL-401 and three licenses, including an exclusive license and two non-exclusive licenses, from the University of Pittsburgh for SL-701. Our license agreement with Scott and White survives, unless earlier terminated, until the later of the expiration of the last to expire licensed patent or the date on which we owe no further payments to Scott and White. Our exclusive and our non-exclusive patent license agreements with the University of Pittsburgh survive, unless earlier terminated, until the expiration of the last to expire licensed patent, and our non-exclusive license with the University of Pittsburgh to use and reference certain clinical trial data and information survives for a term of twenty years unless earlier terminated. We also hold licenses from academic institutions relating to intellectual property underlying our SL-501 and SL-101 product candidates and our StemScreen platform technology. We expect to enter into additional license agreements as part of the development of our business. Our current or future licensors may not successfully prosecute certain patent applications under which we are licensed and on which our business depends. Even if patents issue from these applications, our licensors may fail to maintain these patents, may decide not to pursue litigation against third party infringers, may fail to prove infringement, or may fail to defend against counterclaims of patent invalidity or unenforceability. In addition, in spite of our best efforts, our licensors might conclude that we have materially breached our license agreements and might therefore seek to terminate the license agreements, thereby removing our ability to obtain regulatory approval and to market products covered by these license agreements. Our licensors may also seek to terminate the license agreements if we fail to satisfy our diligence obligations and/or meet specified milestones or upon insolvency. From time to time, we have had to request extensions of our development obligations contained in some of our license agreements, and we may need to seek further extensions in the future. Although we have obtained such extensions in the past, there can be no assurance that our licensors will continue to extend the development timelines or other milestones contained in our license agreements. If these in-licenses are terminated, or if the underlying patents fail to provide the intended market exclusivity, we could lose our rights to develop and commercialize the product candidates governed by the licenses and competitors would have the freedom to seek regulatory approval of, and to market, products identical to ours. This could have a material adverse effect on our competitive business position and our business prospects.

Table of Contents

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

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

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

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

Table of Contents

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

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

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

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

As is the case with other biopharmaceutical companies, our success is heavily dependent on intellectual property, particularly patents. Obtaining and enforcing patents in the biopharma industry involve both technological complexity and legal complexity. Therefore, 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.

Table of Contents

Risks related to our common stock and this offering

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

The trading price of our common stock may be highly volatile, and could be subject to wide fluctuations in response to various factors, some of which are beyond our control. Since our initial public offering which occurred in January 2013, the price of our common stock on The NASDAQ Capital Market has ranged from $10.33 per share to $47.25 per share. The stock market in general and the market for biopharmaceutical companies in particular have experienced extreme volatility that has often been unrelated to the operating performance of particular companies. As a result of this volatility, you may not be able to sell your common stock at a price higher than the offering price. The market price for our common stock may be influenced by many factors, including:

- results from or delays of clinical trials of our product candidates, as well as results of regulatory reviews relating to the approval of our product candidates;
- our decision to initiate a clinical trial, not to initiate a clinical trial or to terminate an existing clinical trial;
- our dependence on third parties, including CROs and CMOs, clinical trial sponsors and clinical investigators;
- our ability to commercialize our product candidates, if approved;
- the results of our efforts to discover, develop, acquire or in-license additional product candidates or products;
- new products, product candidates or new uses for existing products or technologies introduced or announced by our competitors and the timing of these introductions or announcements;
- regulatory or legal developments in the United States and other countries;
- our ability to maintain the license agreements for SL-401, SL-701 and other in-licensed product candidates;
- developments or disputes concerning patent applications, issued patents or other proprietary rights;
- the recruitment or departure of key scientific or management personnel;
- the level of expenses related to any of our product candidates or clinical development programs;
- actual or anticipated changes in estimates as to financial results, development timelines or recommendations by securities analysts;
- variations in our financial results or those of companies that are perceived to be similar to us;
- sales of common stock by us or our stockholders in the future, as well as the overall trading volume of our common stock;

changes in the structure of healthcare payment systems;

market conditions in the pharmaceutical and biotechnology sectors;

Table of Contents

general economic, industry and market conditions and other factors that may be unrelated to our operating performance or the operating performance of our competitors, including changes in market valuations of similar companies; and

the other factors described in this "Risk Factors" section.

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

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

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

Upon the closing of this offering, our executive officers, directors and principal stockholders will beneficially own shares representing approximately % 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;

Table of Contents

limit the manner in which stockholders can remove directors from the board;

establish advance notice requirements for stockholder proposals that can be acted on at stockholder meetings and nominations to our board of directors;

require that stockholder actions must be effected at a duly called stockholder meeting and prohibit actions by our stockholders by written consent;

limit who may call special stockholder meetings and the matters transacted at such meetings;

authorize our board of directors to issue preferred stock without stockholder approval, which could be used to institute a "poison pill" that would work to dilute the stock ownership of a potential hostile acquirer, effectively preventing acquisitions that have not been approved by our board of directors; and

require the approval of the holders of at least 75% of the votes that all our stockholders would be entitled to cast to amend or repeal certain provisions of our charter or bylaws.

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

We have broad discretion in the use of net proceeds from this offering and may not use them effectively.

We intend to use substantially all of the net proceeds from this offering to fund (i) clinical development of SL-401 and SL-701; (ii) further development of our preclinical pipeline, including SL-101 and SL-501; (iii) our drug discovery platform; and (iv) other general corporate purposes, including general and administrative expenses, capital expenditures, working capital and prosecution and maintenance of our intellectual property. Although we currently intend to use the net proceeds from this offering in such a manner, we will have broad discretion in the application of the net proceeds. Our failure to apply these funds effectively could affect our ability to continue to develop and commercialize our product candidates.

If you purchase shares of common stock in this offering, you will suffer immediate dilution in the book value of your shares.

The offering price of our common stock is substantially higher than the net tangible book value per share of our common stock. Therefore, if you purchase shares of our common stock in this offering, you will pay a price per share that substantially exceeds our net tangible book value per share after this offering. To the extent outstanding options or warrants are exercised, you will incur further dilution. If you purchase shares in this offering, you will experience immediate dilution of $ per share, representing the difference between our as adjusted net tangible book value per share after giving effect to this offering, assuming the issuance and sale of shares of our common stock at an assumed offering price of $, which is the last reported public sale price of our common stock on October , 2013. For a further description of the dilution that you will experience immediately after this offering, see "Dilution" elsewhere in this prospectus.

Table of Contents

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

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

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

Effective internal controls over financial reporting are necessary for us to provide reliable financial reports and, together with adequate disclosure controls and procedures, are designed to prevent fraud. Any failure to implement required new or improved controls, or difficulties encountered in their implementation could cause us to fail to meet our reporting obligations. In addition, any testing by us conducted in connection with Section 404 of the Sarbanes-Oxley Act, or the subsequent testing by our independent registered public accounting firm, may reveal deficiencies in our internal controls over financial reporting that are deemed to be material weaknesses or that may require prospective or retroactive changes to our financial statements or identify other areas for further attention or improvement. Inferior internal controls could also cause investors to lose confidence in our reported financial information, which could have a negative effect on the trading price of our common stock.

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

We do not anticipate paying any cash dividends on our capital stock in the foreseeable future.

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

Table of Contents

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. After this offering, we will have outstanding shares of common stock based on the number of shares outstanding as of October , 2013. This includes the shares that we are selling in this offering, which may be resold in the public market immediately without restriction, unless purchased by our affiliates. There will be shares currently restricted as a result of securities laws or lock-up agreements. The restricted shares may be sold in the public market only if registered or pursuant to an exemption from registration, such as Rule 144 or Rule 701 under the Securities Act. Once we register these shares, they can be freely sold in the public market upon issuance, subject to volume limitations applicable to affiliates and the lock-up agreements described in the "Underwriting" section of this prospectus.

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

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

Our issuance of the common stock pursuant to this offering might result in an "ownership change" at the time of issuance, which will increase the risk that we could experience an ownership change in the future. Any ownership change would significantly limit our ability to utilize our net operating losses.

As of December 31, 2012, we had over $14.7 million of net operating losses for tax purposes that we can use in certain circumstances to offset future taxable income and thus reduce our federal income tax liability. Our ability to utilize these net operating losses to offset future taxable income may be significantly limited if we experience an "ownership change" as defined in Section 382 of the Internal Revenue Code of 1986, as amended, or the Code. In general, an ownership change will occur if there is a cumulative change in our ownership by "5-percent shareholders" (as defined in the Code) that exceeds 50 percentage points over a rolling three-year period. A corporation that experiences an ownership change will generally be subject to an annual limitation on the corporation's subsequent use of net operating loss carryovers that arose from pre-ownership change periods and use of losses that are subsequently recognized with respect to assets that had a built-in-loss on the date of the ownership change. The amount of the annual limitation generally equals the value of the corporation immediately before the ownership change multiplied by the long-term tax-exempt interest rate (subject to certain adjustments). To the extent that the limitation in a post-ownership-change year is not fully utilized, the amount of the limitation for the succeeding year will be increased.

We do not expect to experience an ownership change as a result of our issuance of common stock in this offering. Nevertheless, the rules regarding the determination of whether an ownership change exists are complicated and are subject to differing interpretations, and it is possible that such issuances might be treated as resulting in an ownership change. Even if there will be no immediate ownership change as a result of such issuance, the issuance of stock pursuant to this offering will be taken into account in determining the cumulative change in our ownership for Section 382 purposes. As a result, this offering materially increases the risk that we could experience an ownership change in the future. If we experience an ownership change, we may not be able to fully utilize our net operating losses, resulting in additional income taxes and a reduction in our stockholders' equity.

Table of Contents

Special cautionary notice regarding forward-looking statements

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

By their nature, forward-looking statements involve risks and uncertainties because they relate to events, competitive dynamics, and healthcare, regulatory and scientific developments and depend on the economic circumstances that may or may not occur in the future or may occur on longer or shorter timelines than anticipated. Although we believe that we have a reasonable basis for each forward-looking statement contained in this prospectus, 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 prospectus. 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 prospectus, 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 use of the proceeds from this offering;

Table of Contents

our available cash;

the accuracy of our estimates regarding expenses, future revenues, capital requirements and needs for additional financing;

our ability to obtain additional funding;

our ability to obtain and maintain intellectual property protection for our product candidates;

our ability to maintain the license agreements for SL-401, SL-701 and our other in-licensed product candidates;

the successful development of our sales and marketing capabilities;

the performance of third-party manufacturers, CROs, clinical trial sponsors and clinical trial investigators; and

our ability to successfully implement our strategy.

Any forward-looking statements that we make in this prospectus 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 prospectus. You should also read carefully the factors described in the "Risk Factors" section of this prospectus 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 prospectus 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 prospectus 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.

Table of Contents

Use of proceeds

We estimate that the net proceeds from our issuance and sale of shares of our common stock in this offering will be approximately $ million, after deducting underwriting discounts and commissions and estimated offering expenses payable by us. If the underwriters exercise the option to purchase an additional shares of our common stock in full, we estimate that the net proceeds from this offering will be approximately $ million, after deducting underwriting discounts and commissions and estimated offering expenses payable by us.

We intend to use the net proceeds from this offering for clinical development of SL-401 and SL-701, further development of our preclinical pipeline, including SL-501 and SL-101, and drug discovery platform, and other general corporate purposes, as follows:

SL-401. We plan to initiate Company-sponsored trials in 2014 with SL-401 in multiple hematologic cancers. Among these studies, we intend to initiate planned pivotal programs with SL-401 in patients with BPDCN and in patients with third-line AML. We also plan to initiate Phase 2 trials in multiple additional hematologic indications. These may include multiple myeloma, earlier stages of AML, MDS, CML, Hodgkin's and certain non-Hodgkin's lymphomas, and other hematologic indications, including several rare malignancies and proliferative disorders (e.g., hairy cell leukemia, mastocytosis, basophilic leukemia, eosinophilic leukemia).

SL-701. We plan to initiate a Phase 2b clinical trial of SL-701 in adult patients with second-line GBM. The design of the Phase 2b study may enable SL-701 to obtain accelerated regulatory approval or serve as the foundation for a subsequent Phase 3 pivotal trial in this indication. We also plan to initiate a Phase 2 clinical trial of SL-701 in pediatric patients with brainstem and non-brainstem high-grade glioma.

Pipeline. We plan to continue to build out and develop our pipeline. In particular, we have developed follow-on product candidates, SL-501 and SL-101, directed to IL-3R, the target clinically validated by SL-401. We plan to advance SL-501, SL-101, and possibly other preclinical candidates, through investigational new drug, or IND, enabling studies and potentially into clinical trials.

Drug Discovery (StemScreen®). We intend to optimize and utilize our proprietary discovery platform, StemScreen®, to identify novel CSC-targeted drug candidates. We may conduct some of these efforts by using third party contractors or by acquiring/building internal laboratories. We believe that using our own laboratories could facilitate our research and development efforts.

The remaining proceeds will be used for general corporate purposes.

This expected use of the net proceeds from this offering represents our intentions based upon our current plans and business conditions. The amounts and timing of our actual expenditures may vary significantly depending on numerous factors, including the progress of our development efforts, the status of and results from clinical trials, as well as any collaborations that we may enter into with third parties for our product candidates, and any unforeseen cash needs. As a result, our management will retain broad discretion over the allocation of the net proceeds from this offering.

We believe that our existing cash and cash equivalents, together with the net proceeds from this offering, will be sufficient to fund our operations and our capital expenditures for at least the next several years.

Pending our use of the net proceeds from this offering, we intend to invest the net proceeds in a variety of capital preservation investments, including short-term, investment grade, interest bearing instruments and U.S. government securities.

Table of Contents

Capitalization

The following table sets forth our cash and cash equivalents and capitalization as of June 30, 2013:

- on an actual basis; and
- on an as adjusted basis to give further effect to the issuance and sale of shares of our common stock in this offering at an assumed offering price of $ per share, which was the last reported sale price of our common stock on October , 2013, after deducting underwriting discounts and commissions and estimated offering expenses payable by us.

You should read this table together with "Selected Financial Data" included elsewhere in this prospectus, and our financial statements and related notes and the "Management's Discussion and Analysis of Financial Condition and Results of Operations" in our Annual Report on Form 10-K for the year ended December 31, 2012 and Quarterly Reports on Form 10-Q for the quarters ended March 31, 2013 and June 30, 2013, all of which are incorporated by reference in this prospectus.

		June 30, 2013	
		Actual (unaudited)	As adjusted(1)
Cash and cash equivalents	$	92,685,912	$
Common stock, $0.0001 par value, 33,750,000 shares authorized and 12,539,031 shares issued and outstanding, actual; and 33,750,000 shares authorized and shares issued and outstanding, as adjusted		1,254	
Additional paid-in capital		107,799,653	
Accumulated deficit during the development stage		(18,125,539)	(18,125,539)
Total stockholders' equity		89,675,368	
Total capitalization	$	89,675,368	$

(1) Each $1.00 increase (decrease) in the assumed public offering price of $ per share, the last reported sale price of our common stock on The NASDAQ Capital Market on October , 2013, would increase (decrease) the as adjusted amount of cash and cash equivalents, total stockholders' equity and total capitalization by approximately $ million , assuming the number of shares offered by us, as set forth on the cover page of this prospectus, remains the same and after deducting the estimated underwriting discounts and commissions and estimated offering expenses payable by us. We may also increase or decrease the number of shares we are offering. Each increase (decrease) of 100,000 shares in the number of shares offered by us would increase (decrease) the as adjusted amount of cash and cash equivalents, total stockholders' equity and total capitalization by approximately $ million, assuming that the assumed public offering price remains the same, and after deducting the estimated underwriting discounts and commissions and estimated offering expenses payable by us. The as adjusted information discussed above is illustrative only and will be adjusted based on the actual public offering price and other terms of this offering determined at pricing.

The table above does not include:

- 1,879,573 shares of our common stock issuable upon the exercise of stock options outstanding as of June 30, 2013 at a weighted-average exercise price of $3.97 per share;

99,529 shares of common stock issuable upon the exercise of warrants outstanding as of June 30, 2013, at an exercise price of $15.00 per share; and

1,352,379 shares of our common stock reserved for future issuance under the 2012 Equity Plan as of June 30, 2013, plus any future increases in the number of shares reserved for issuance under the 2012 Equity Plan pursuant to evergreen provisions

Table of Contents

Dilution

If you invest in our common stock in this offering, your ownership interest will be diluted immediately to the extent of the difference between the offering price per share of our common stock and the as adjusted net tangible book value per share of our common stock after the offering.

Our historical net tangible book value as of June 30, 2013 was $89.7 million, or $7.15 per share of our common stock. Historical net tangible book value per share represents the amount of our total tangible assets less total liabilities, divided by the number of shares of our common stock outstanding.

After giving effect to our issuance and sale of $ million of shares of our common stock in this offering at the assumed offering price of $ per share, which was the last reported sale price of our common stock on The NASDAQ Capital Market on October , 2013, and after deducting underwriting discounts and commissions and estimated offering expenses payable by us, our as adjusted net tangible book value as of June 30, 2013 would have been $ million, or $ per share. This represents an immediate increase in as adjusted net tangible book value per share of $ to existing stockholders and immediate dilution of $ in as adjusted net tangible book value per share to new investors purchasing common stock in this offering.

Dilution per share to new investors is determined by subtracting as adjusted net tangible book value per share after this offering from the offering price per share paid by new investors. The following table illustrates this dilution on a per share basis.

Assumed Offering Price Per Share	$
Historical Net Tangible Book Value Per Share as of June 30, 2013	7.15
Increase in Net Tangible Book Value Per Share Attributable to New Investors	
As Adjusted Net Tangible Book Value Per Share After the Offering	
Dilution Per Share to New Investors	

If the underwriters exercise the option to purchase an additional $ million of shares of our common stock in full, the as adjusted net tangible book value per share, after giving effect to the offering, would be $ per share. This represents an immediate increase in as adjusted net tangible book value of $ per share to existing stockholders and an immediate dilution in as adjusted net tangible book value of $ per share to new investors purchasing common stock in this offering. Moreover, if any additional shares are issued in connection with outstanding options or warrants, you will experience further dilution. In addition, we may choose to raise additional capital due to market conditions or strategic considerations even if we believe we have sufficient funds for our current or future operating plans. To the extent that additional capital is raised through the sale of equity or convertible debt securities, the issuance of these securities may result in further dilution to our stockholders.

Each $1.00 increase in the assumed public offering price of $ per share would increase our net tangible book value after this offering by approximately $ million, or approximately $ per share, and the dilution per share to new investors by approximately $ per share, assuming that the number of shares offered by us, as set forth on the cover page of this prospectus, remains the same and after deducting the estimated underwriting discounts and commissions and estimated offering expenses payable by us. Each $1.00 decrease in the assumed public offering price of $ per share would decrease our net tangible book value after this offering by approximately $ million, or

Table of Contents

approximately $ per share, and the dilution per share to new investors by approximately $ per share, assuming that the number of shares offered by us, as set forth on the cover page of this prospectus, remains the same and after deducting the estimated underwriting discounts and commissions and estimated offering expenses payable by us. We may also increase or decrease the number of shares we are offering. An increase of 100,000 shares in the number of shares offered by us would increase our net tangible book value after this offering by approximately $ million, or $ per share, and decrease the dilution per share to new investors by approximately $ per share, assuming that the assumed public offering price remains the same, and after deducting the estimated underwriting discounts and commissions and estimated offering expenses payable by us. Similarly, a decrease of 100,000 shares in the number of shares offered by us would decrease our net tangible book value after this offering by approximately $ million, or $ per share, and increase the dilution per share to new investors by approximately $ per share, assuming that the assumed public offering price remains the same, and after deducting the estimated underwriting discounts and commissions and estimated offering expenses payable by us. The information discussed above is illustrative only and will adjust based on the actual public offering price and other terms of this offering determined at pricing.

The information above excludes:

- 1,879,573 shares of our common stock issuable upon the exercise of stock options outstanding as of June 30, 2013 at a weighted-average exercise price of $3.97 per share;

- 99,529 shares of common stock issuable upon the exercise of warrants outstanding as of June 30, 2013, at an exercise price of $15.00 per share; and

- 1,352,379 shares of our common stock reserved for future issuance under the 2012 Equity Plan as of June 30, 2013, plus any future increases in the number of shares reserved for issuance under the 2012 Equity Plan pursuant to evergreen provisions.

Table of Contents

Selected financial data

The following table sets forth our selected financial data for the periods and as of the dates indicated. You should read the following selected financial data in conjunction with our audited and unaudited financial statements and the related notes thereto and the "Management's Discussion and Analysis of Financial Condition and Results of Operations" section of our Annual Report on Form 10-K for the year ended December 31, 2012 and our Quarterly Reports on Form 10-Q for the quarters ended March 31, 2013 and June 30, 2013 incorporated by reference herein.

We have derived the statements of operations data for the years ended December 31, 2010, 2011 and 2012 and the balance sheet data as of December 31, 2011 and 2012 from our audited financial statement incorporated by reference in this prospectus. We have derived the statements of operations data for the years ended December 31, 2008 and 2009 and the balance sheet data as of December 31, 2008, 2009 and 2010 from our financial statements not included in this prospectus. We have derived the statements of operations data for the six months ended June 30, 2012 and 2013, and the balance sheet data as of June 30, 2013 from our unaudited financial statements incorporated by reference in this prospectus. In our opinion, such unaudited financial statements include all adjustments, consisting only of normal recurring adjustments that we consider necessary for a fair presentation of the financial information set forth in those statements. Our historical results for any prior period are not necessarily indicative of results to be expected in any future period and the results for the six months ended June 30, 2013 are not necessarily indicative of the results that may be expected for the full fiscal year. For more details on how you can obtain the documents incorporated by reference in this prospectus, see "Where You Can Find More Information" and "Incorporation of Documents by Reference."

Table of Contents

	Year ended December 31,					Six months ended June 30,		P
	2008 (unaudited)	2009	2010	2011	2012	2012 (unaudited)	2013 (unaudited)	(
f operations data:								
	$	$	$	$	$	$	$	$
penses:								
l development	$ 1,016,702	$ 1,054,446	$ 1,329,509	$ 1,629,026	$ 3,376,962	$ 1,613,340	$ 7,246,247	$
administrative	1,179,984	1,026,675	930,331	1,088,028	3,090,611	1,372,152	3,238,893	
ng expenses	2,196,686	2,081,121	2,259,840	2,717,054	6,467,573	2,985,492	10,485,140	
erations	(2,196,686)	(2,081,121)	(2,259,840)	(2,717,054)	(6,467,573)	(2,985,492)	(10,485,140)	
e:		102,257	484,905	46,673	301,684	12,460	30,649	
se				(9,670)	(35)	(35)		
nse			(69,493)	(98,643)	(118,765)	(50,038)	(505,037)	
me	376,578	201,088	43,045	24,068	9,907	7,882	3,244	
	$(1,820,108)	$(1,777,776)	$ (1,801,383)	$(2,754,626)	$(6,274,782)	$(3,015,223)	$(10,956,284)	$
on of preferred stock dividends	(1,021,201)	(1,100,107)	(239,720)					
t on redemption of preferred stock			12,171,765					
ncome attributable to common	$(2,841,309)	$(2,877,883)	$10,130,662	$(2,754,626)	(6,274,782)	$(3,015,223)	$(10,956,284)	$
ncome attributable to common per common share:								
	$ (1.01)	$ (1.02)	$ 3.07	$ (0.80)	$ (1.82)	$ (0.88)	$ (1.37)	
	$ (1.01)	$ (1.02)	$ 2.81	$ (0.80)	$ (1.82)	$ (0.88)	$ (1.37)	
erage number of common shares:								
	2,824,647	2,824,647	3,298,793	3,441,995	3,441,995	3,441,995	8,014,529	
	2,824,647	2,824,647	3,607,030	3,441,995	3,441,995	3,441,995	8,014,529	

	As of December 31,					As of June 30,
	2008 (unaudited)	2009	2010	2011	2012	2013 (unaudited)
Balance sheet data:						
Cash and cash equivalents	$ 2,196,881	$ 9,236,395	$ 7,226,366	$ 5,829,886	$ 2,025,338	$ 92,685,912

Total assets	$ 10,856,476	$ 9,329,341	$ 7,502,912	$ 6,453,096	$ 5,029,611	$ 93,848,091
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)	$ (18,125,539)
Total stockholders' (deficit)/equity	$ (3,355,509)	$ (6,162,215)	$ 5,851,561	$ 3,205,340	$ (2,508,420)	$ 89,675,368

Table of Contents

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 the treatment of blastic plasmacytoid dendritic cell neoplasm, or BPDCN, third-line acute myeloid leukemia, or AML, and multiple other hematologic cancers. In February 2011, SL-401 received Orphan Drug designation from the FDA for the treatment of AML. In June 2013, SL-401 received Orphan Drug designation from the FDA for the treatment of BPDCN. SL-701 is a subcutaneously-administered therapeutic cancer vaccine comprised of synthetic peptides, and is currently being developed for the treatment of advanced adult and pediatric brain cancer. In completed investigator-sponsored Phase 1/2 clinical trials, both SL-401 and SL-701 have demonstrated single agent activity, including durable complete responses, or CRs, and longer median overall survival, or OS, in heavily pretreated patients compared with that achieved in the past with traditional therapies.

Based upon favorable clinical data observed to date with SL-401 and SL-701 across multiple liquid and solid tumor types, we plan to initiate multiple clinical trials in 2014 of SL-401 in a variety of hematological cancers and of SL-701 in adult and pediatric brain cancers. These include pivotal programs with SL-401 in patients with BPDCN, and in patients with AML who failed two previous treatments, i.e., third-line AML. In addition, given the breadth of SL-401 clinical activity seen to date, including clinical response in three indications: BPDCN, AML, and myelodysplastic syndrome, or MDS; the robust preclinical activity demonstrated in multiple leukemia and lymphoma indications; and the widespread expression of the target of SL-401, the interleukin-3 receptor, or IL-3R, across the majority of hematologic cancers, we plan to initiate Phase 2 trials in many of these indications. These may include multiple myeloma, earlier stages of AML, MDS, chronic myeloid leukemia, or CML, Hodgkin's and certain non-Hodgkin's lymphomas, and other hematologic indications, including several rare malignancies and proliferative disorders, such as hairy cell leukemia, mastocytosis, basophilic leukemia and eosinophilic leukemia. In addition to SL-401, we have developed next generation product candidates, SL-501 and SL-101, directed to the same clinically-validated IL-3R target. Each of these preclinical stage compounds has distinguishing characteristics SL-501 is a high affinity variant of SL-401 with elevated potency against IL-3R+ malignancies, and SL-101 is a monoclonal antibody-conjugate directed to the IL-3R alpha chain (CD123) also with high potency against IL-3R+ malignancies. We plan to advance SL-501, SL-101, and possibly other preclinical candidates, through investigational new drug, or IND, enabling studies and potentially into clinical trials. We plan to complete a Phase 2b trial of SL-701 in adult patients with glioblastoma multiforme, or GBM, who failed one previous treatment, i.e., second-line GBM. In addition, we plan to complete a Phase 2 trial of SL-701 in pediatric patients with brainstem and non-brainstem high-grade glioma.

The field of CSCs is an emerging area of cancer biology that we believe is fundamentally altering the approach to oncology drug development. CSCs have been identified in virtually all major tumor types, including leukemia and cancers of the brain, breast, colon, prostate and pancreas. CSCs are the highly malignant "seeds" of a tumor that self-renew and generate more mature cells that comprise the bulk of the tumor, or the "tumor bulk." As such, we believe that CSCs are responsible for tumor initiation, propagation, and metastasis. Moreover, many of the characteristics of CSCs, such as their slow growth, presence of multi-drug resistance proteins, anti-cell death mechanisms, and increased activity of cellular mechanisms that repair damaged DNA, may enable CSCs to resist therapeutic agents traditionally used to

Table of Contents

treat cancer. Further, we believe there is now a significant body of evidence indicating that while standard therapies may initially shrink tumors by targeting the tumor bulk, their failure to effectively eradicate CSCs contributes to treatment failure, tumor relapse and poor survival. Accordingly, we believe that targeting both CSCs and the tumor bulk, may represent a major advance in the fight against cancer. This premise has formed the basis of our drug development strategy, as illustrated below.

As a leader in the CSC field, we intend to develop and commercialize our lead product candidates, advance our preclinical pipeline, and utilize our discovery platform, StemScreen®, to identify new drug candidates for additional indications. Since our inception in 2003, we have leveraged our knowledge of CSCs to establish a leadership position in this new field of oncology. During this time, we have internally developed or strategically in-licensed key intellectual property, and built an innovative drug discovery platform and developed preclinically and 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, Eleventh Floor, New York, New York 10022 and our telephone number is (646) 502-2310.

Our website address is www.stemline.com. The information set forth on our website is not a part of this prospectus. We will make available free of charge through our website our annual reports on Form 10-K, quarterly reports on Form 10-Q and current reports on Form 8-K, and any amendments to these reports, as soon as reasonably practicable after we electronically file such material with, or furnish such material to, the SEC. We are not including the information on our website as a part of, nor incorporating it by reference into, this prospectus. 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.

Table of Contents

Management

We are led by a team with extensive experience in managing biopharmaceutical companies and in oncology drug development, including:

Ivan Bergstein, M.D. Chairman, Chief Executive Officer and President. Dr. Bergstein is Chief Executive Officer and Founder of Stemline Therapeutics. He led Stemline through multiple rounds of private financing and ultimately its successful IPO and subsequent follow-on offering, raising over $100 million as a public company. Prior to founding Stemline, Dr. Bergstein was Medical Director of Access Oncology, Inc., a clinical stage oncology-focused biotechnology company where he was a key member of a small team responsible for the acquisition and development of the company's clinical stage assets and ultimately the sale of the company. Previously, he was a senior biopharmaceuticals analyst at a Wall Street-based firm that advised mutual funds and hedge funds on investments in public companies with late clinical stage assets. He completed an internal medicine residency and hematology-oncology fellowship at the New York Presbyterian Hospital Weill Medical College of Cornell University.

Eric K. Rowinsky, M.D. Executive Vice President, Chief Medical Officer and Head of Research and Development. Dr. Rowinsky was previously the Chief Medical Officer for ImClone Systems, Inc. Dr. Rowinsky has more than 25 years of experience managing clinical trials and developing drugs in oncology, including leading the FDA approval of Erbitux® for head and neck and colorectal cancers and advancing eight other biological therapeutics through clinical development while at ImClone. He has also played integral roles in the development and registration of a wide range of cancer therapeutics, including paclitaxel, docetaxel, irinotecan, topotecan, erlotinib, gefitinib, panitumumab, lapatinib, and temsirolimus, among others. Dr. Rowinsky currently serves on the Board of Directors of Biogen Idec Inc., as well as several other public biopharmaceutical companies.

Kenneth Hoberman Chief Operating Officer. Mr. Hoberman was previously Vice President of Corporate and Business Development of Keryx Biopharmaceuticals, Inc., where he was instrumental in securing multiple sources of capital including over $200 million in equity investments through public and private offerings. He also initiated and executed a $100 million strategic alliance and originated, negotiated and closed dozens of licensing and operational contracts, and helped grow the company to a $900 million market capitalization.

Stephen P. Hall Vice President of Finance and Chief Accounting Officer. During the past 20 years, Mr. Hall has held senior positions, including Chief Financial Officer, Chief Accounting Officer, and Treasurer of multiple life science companies. These include Orthocon Inc, Helicos Biosciences and TriPath Imaging, a public oncology company that was subsequently acquired by Becton Dickinson and Company where Mr. Hall continued to serve as a Senior Advisor. He was also founder and managing director of Deimos Consulting LLC, a consulting firm specializing in life sciences He earned an A.B. degree from Harvard College and an MBA from the Stanford Graduate School of Business.

Strategy

Our goal is to maintain and fortify a leadership position in the discovery, acquisition and development of novel oncology therapies that target CSCs and to build a fully integrated pharmaceutical company with commercial infrastructure to support the marketing of our CSC targeted oncology drugs, if approved. The fundamental components of our business strategy to achieve this goal include the following:

Develop and commercialize SL-401 in multiple hematological cancers. We plan to initiate Company-sponsored clinical trials in 2014 with SL-401 in multiple hematologic cancers. We plan to initiate pivotal

Table of Contents

programs in BPDCN and in third-line AML. In particular, we plan to initiate a Phase 2b single-arm trial in patients with BPDCN with overall response rate, or ORR, as the primary endpoint, and a Phase 3 randomized trial as a third-line treatment for patients with AML, with OS as the primary endpoint. We intend to conduct these trials in North America and Europe, and we believe these trials will serve as pivotal trials in these territories. BPDCN and AML are orphan indications, which are rare diseases or conditions affecting fewer than 200,000 people in the United States, and each represents an unmet medical need. We also plan to initiate multiple Phase 2 trials of SL-401 in additional hematologic cancers, which may include multiple myeloma, earlier stages of AML (i.e., second- and first-line; either combined with standard treatment regimens or as a single agent as maintenance therapy for minimum residual disease, or MRD), MDS, CML, Hodgkin's and certain non-Hodgkin's lymphomas, and other hematologic indications, including several rare diseases such as hairy cell leukemia, mastocytosis, basophilic leukemia, and eosinophilic leukemia. We believe that some of these trials could be expanded to serve as platform trials for potential registration. Accordingly, we believe that SL-401 could be active in many hematologic cancer indications, thereby representing significant market opportunities.

Develop and commercialize SL-701 in brain cancer. We plan to initiate Company-sponsored trials in 2014 with SL-701 in adult and pediatric brain cancers. We plan to initiate a Phase 2b trial of SL-701 in adult patients with second-line GBM. The design of the Phase 2b study may enable SL-701 to obtain accelerated regulatory approval or serve as the foundation for a subsequent Phase 3 pivotal trial in this indication. We also plan to initiate a Phase 2 trial of SL-701 in pediatric patients with brainstem and non-brainstem high-grade glioma, which are areas of unmet medical need.

Continue to advance and build out our pipeline. We also plan to advance and build out our pipeline of product candidates. In particular, we plan to advance SL-501 and SL-101, our next generation IL-3R-targeting agents through IND-enabling studies and potentially into clinical trials.

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

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

Continue to both leverage and fortify our intellectual property portfolio. We believe that we have a strong intellectual property position relating to the development and commercialization of our product candidates and technology. 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.

Table of Contents

Clinical pipeline

The following table summarizes the status of our two most advanced product candidates:

SL-401 A targeted therapy directed to IL-3R on 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 investigator-sponsored 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 six CRs in patients: four 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 has not demonstrated toxicity 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

Table of Contents

that a major reason for the failures of traditional treatments to provide long-term benefit is that these traditional treatments target tumor bulk rather than both tumor bulk and CSCs, and are often toxic to the bone marrow. Accordingly, by pursuing hematologic cancer indications with SL-401, a therapeutic that uniquely targets both CSCs and tumor bulk and has not demonstrated toxicity to the bone marrow, we hope to provide benefit to patients who historically have been difficult to treat with traditional therapies.

We plan to initiate Company-sponsored trials in 2014 with SL-401 in multiple hematologic cancers. Among these studies, we plan to initiate pivotal programs in patients with BPDCN, with overall response rate, or ORR, as the primary endpoint, and in AML patients as a third-line treatment, with OS as the primary endpoint. We plan to conduct these trials in both North America and Europe. In addition, we plan to initiate Phase 2 trials of SL-401 in additional hematologic cancers, which may include multiple myeloma, earlier stages of AML (i.e. second- and first-line; either combined with standard treatment regimens or as a single agent as maintenance therapy for minimum residual disease, or MRD), MDS, CML, Hodgkin's and certain non-Hodgkin's lymphomas, as well as several rare IL-3R-expressing malignancies and proliferative disorders such as hairy cell leukemia and malignancies and proliferative disorders of mast cell and basophilic lineages (e.g., systemic mastocytosis and basophilic leukemia), as well as eosinophilic leukemia.

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

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. We estimate that there are approximately 2,000 new cases of BPDCN annually in North American and Europe. BPDCN derives from plasmacytoid dendritic cells, which are specialized immune cells that express 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, overall prognosis remains poor. There are currently no approved therapies for BPDCN, and an optimal therapeutic regimen for BPDCN has not yet been established.

Acute myeloid leukemia (AML)

AML is a hematologic cancer characterized by dysregulated maturation of myeloid cells and failure of the bone marrow to properly function. AML is the most common type of acute leukemia in adults. Approximately 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 in combination with an anthracycline such as daunorubicin. 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. Despite a moderate to high proportion of patients obtaining a CR with first- and second-line

Table of Contents

chemotherapy, the high relapse rate and poor OS indicate that most patients harbor drug-resistant CSCs following chemotherapy. In third-line AML, there are currently no approved treatments, and these 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 (MDS)

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.

Multiple myeloma (MM)

Multiple myeloma (MM) is a hematologic malignancy that is characterized by the dysfunction of plasma cells, which are white blood cells that produce antibodies. During MM, malignant plasma cells overproduce abnormal monoclonal antibodies and can interfere with normal blood cell function in the bone marrow leading to immunodeficiency. Other common clinical manifesations of advanced MM include osteolytic bone lesions and renal disease. The bone marrow (BM) microenvironment confers growth, survival, and drug resistance of MM cells, and it has recently been shown that plasmacytoid dendritic cells (pDCs), which express high levels of IL-3R, are significantly increased in the BM of patients with MM and promote MM proliferation. Approximately 22,000 new cases of MM are reported annually in the United States and approximately 33,000 new MM cases are reported annually in Europe. The median age at diagnosis is approximately 62 years for men and 61 years for women. The median overall survival after conventional treatments is 3-4 years, but high-dose treatment followed by autologous stem cell transplantation can extend the median survival to 5-7 years. Despite FDA approved therapies for MM, including thalidomide (Thalomid®), lenalidomide (Revlimid®), bortezomib (Velcade®), and dexamethasone (Decadron®), most patients invariably relapse from the disease.

Other rare IL-3R cancers

A number of other rare hematologic diseases express IL-3R at elevated levels, including hairy cell leukemia, and malignancies and proliferative disorders of mast cell and basophil lineages including mastocytosis and basophilic leukemias, as well as eosinophilic leukemias. Hairy cell leukemia (HCL)'s an uncommon hematological malignancy characterized by a clonal accumulation of abnormal B lymphocytes. Approximately 2,000 new cases of HCL occur annually in the United States. The median age at diagnosis is approximately 62 years with male predominance. Although the 6-year overall survival rate has been estimated to be approximately 80% and there are FDA approved therapies for HCL, including cladribine (Litak® and Movectro®) and pentostatin (Nipant®), there is no permanent cure for the disease.

Mastocytosis is a proliferative disorder characterized by an overabundance of mast cells in various organs and tissues. Mastocytosis can be systemic or localized to one or a few organs, and can be malignant or

Table of Contents

nonmalignant. The World Health Organization, or WHO, classifies mastocytosis into the following categories: cutaneous, indolent, systemic (with associated hematologic non-mast cell lineage disease), aggressive systemic, mast cell leukemia, mast cell sarcoma, and extracutaneous astrocytoma. There are approximately 3,000 cases of systemic mastocytosis diagnosed annually in the United States. Patients with indolent disease typically have a favorable prognosis, whereas aggressive systemic mastocytosis carries a median overall survival of 41 months. There are no currently approved drugs and no cure for mastocytois. Treatment for aggressive variants includes various chemotherapy agents, imatinib (Gleevec®), corticosteroids, and antihistamines.

Several other malignancies involve the proliferation of malignant basophils, which overexpress the IL-3R. Basophilic leukemia is a very rare hematologic disorder that is characterized by the presence of abnormal and undifferentiated basophils. It is classified as a sub-type of acute myeloid leukemia. In addition, patients with chronic myeloid leukemia, or CML, may have increased numbers of circulating neoplastic basophils, particularly when they develop an accelerated phase of their disease which is generally associated with resistance to available treatments.

Hypereosinophilic syndrome (HES), a rare myeloproliferative disorder characterized by a persistently elevated eosinophil count, results in damage to the heart, lungs, peripheral nervous system, and other organs. An acquired (non-familial) form of HES is particularly aggressive and debilitating. Acquired forms of HES are subclassified as secondary (reactive), idiopathic, and clonal HES, the latter often transitioning into chronic eosinophilic leukemia (CEL), or hypereosinophilic leukemia, which can result in myocardial fibrosis and congestive heart failure. Eosinophils are known to ubiquitously express the IL-3R. Current treatments for CEL include corticosteroids, mepolizumab, alemtuzumab (Campath®), and imatinib (Gleevec®), which is approved by the FDA for HES patients who express the FIP1L1-PDGFRA fusion protein. However, some of these agents can cause severe toxicity and may not induce durable responses. Therefore, newer and more effective therapies are needed.

Chronic myeloid leukemia (CML)

Chronic myeloid leukemia, or CML, is a hematopoietic stem cell disease resulting in impaired bone marrow function. Annually, approximately 5,000 new cases are reported in the United States each year and approximately 4,000 to 9,000 new cases are reported each year in Europe. The five-year OS rate for CML patients is 57%. When CML advances to an accelerated or blastic phase, the median OS is less than one year. In patients who have failed or are intolerant to tyrosine kinase inhibitors (or TKIs), a relapsed or refractory setting, the median OS is four to 11 months. Current first-line treatments for CML include three TKIs: imatinib (Gleevec®), nilotinib (Tasigna®) and dasatinib (Sprycel®). In cases of relapse, second- and third-line treatments include a TKI not previously used in that patient. In certain circumstances, interferon or bone marrow transplantation is also used.

Hodgkin's lymphoma (HL)

Hodgkin's lymphoma (HL) is a cancer of the lymphatic system that commonly affects lymph nodes in the neck or the area between the lungs and behind the breastbone. Approximately 9,000 new HL cases occur annually in the United States, and we estimate that approximately 12,000-17,000 cases occur annually in Europe. The disease has four subtypes, including nodular sclerosis, lymphocyte-rich, mixed cellularity, and lymphocyte-depleted HL, all of which produce increased numbers of a unique cell type called "Reed-Sternberg" cells. These cells are considered to be the clonal tumor cells of HL and are known to express the IL-3R. Although combination chemotherapy and/or radiation therapy are affective at combating this disease, 20-30% of patients relapse after initial treatment or have primary refractory disease. Of these

Table of Contents

patients, those who do not obtain a complete remission (CR) prior to transplantation, or who relapse after second line therapy, have few effective therapeutic options.

Design of SL-401 and mechanism of action

SL-401 is a biologic targeted therapy directed to the IL-3R. SL-401 consists of IL-3 recombinantly linked to a truncated diphtheria toxin payload. Mechanistically, the IL-3 domain of SL-401 directs the cytotoxic payload to IL-3R+ cells. SL-401 is then internalized by target cells, leading to intracellular release of the payload, inhibition of protein synthesis and cell death, or apoptosis. Accordingly, the targeting and mechanism by which SL-401 kills cells differs from therapeutics that are commonly used to treat hematologic malignancies 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, Hodgkin's and certain aggressive non-Hodgkin's lymphomas, hairy cell leukemia, and malignancies and proliferative disorders involving mast cell and basophilic lineages. 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 stem cells were relatively insensitive to SL-401. SL-401 also exhibited anti-CSC activity. In particular, SL-401 inhibited AML colony formation, an assay for stem cell activity, compared with normal human bone marrow. As further validation of an anti-CSC effect, SL-401 reduced the incorporation and growth (i.e., tumorigenicity) of AML cells, relative to normal human bone marrow, when treated *ex vivo* and reimplanted into immunodeficient mice indicating activity at the level of the CSC. In addition, SL-401 prolonged the survival of mice implanted with human leukemia xenografts compared with untreated mice.

Table of Contents

In addition, SL-401 demonstrated high potency against BPDCN cells from patients, with an IC_{50} in the femtomolar range. 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 been shown to possess a synergistic anti-CML effect when used in combination with certain tyrosine kinase inhibitors, or TKIs. SL-401 has also demonstrated potent in vitro anti-tumor activity against several lymphoid cancer types, including lymphoid leukemia (e.g. T cell acute lymphoid leukemia, or T-ALL), Hodgkin's and non-Hodgkin's lymphoma, and multiple myeloma, or MM. Interestingly, SL-401 has been shown to have both a direct as well as an indirect anti-MM effect, the latter caused by SL-401's ability to target IL-3R+ hyperproliferative dendritic cells that provide a microenvironmental growth stimulus to their neighboring MM cells. This is notable for several reasons including its novel mechanism of anti-MM action as well as linking BPDCN and MM via the IL-3R target.

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.

Completed Phase 1/2 clinical trial advanced hematologic cancers

Overview

SL-401 was evaluated in a completed multi-center investigator-sponsored 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 six CRs in patients: four 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 has not demonstrated toxicity to bone marrow, which we believe is a key differentiating feature relative to other hematologic cancer therapies.

The 401 AHC Study was undertaken in 83 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=6), with "n" representing the number of patients. The median patient age was 65 years, with a range of seven to 84 years of age. Patients received a single cycle of 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) and University of Texas Southwestern (Dallas, 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,

Table of Contents

Canada). The results from the 401 AHC Study, which are set forth below, were presented at the American Society of Clinical Oncology (ASCO) annual conference in June 2013.

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 tolerability 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 has not demonstrated toxicity 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, tumor shrinkages or disease stabilizations were seen in 46% of patients with relapsed or refractory AML, 55% of AML who were poor risk and thus not candidates for chemotherapy, 43% of high-risk MDS patients and 83% of relapsed or refractory BPDCN patients. There were also multiple additional cases of tumor shrinkages in response to a single cycle of SL-401 treatment. Durable CRs were induced in two patients with relapsed or refractory AML. Four additional CRs and one PR occurred after a single cycle of SL-401 in six patients with BPDCN.

Table of Contents

SL-401 clinical anti-tumor activity in patients with advanced hematological cancers after only a single cycle of SL-401 therapy

	BPDCN (n=6)	AML (relapsed refractory) (n=59)	AML (≥ 3rd line) (n=35*)	AML (not chemo candidate) (n=11)	MDS (high risk) (n=7)
Tumor shrinkages/disease stabilization	83%	46%	43%	55%	43%
Tumor shrinkages	83%	25%	23%	27%	29%
	4 CRs	2 CRs	1 CR		

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 was refractory to 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 major objective anti-tumor response (4 CRs, 1 PR) in five of six patients with BPDCN, a rare and aggressive hematologic malignancy that highly overexpresses IL-3R. This included a third-line BPDCN patient, having received two 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 also normalized. No serious side effects were noted. This CR, following only a single cycle of SL-401 therapy, lasted five months. Another notable CR was experienced by a patient who had fourth-line BPDCN, 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, no serious side effects were observed. This CR, following only a single cycle of SL-401 therapy, is ongoing and currently exceeds eleven 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

Table of Contents

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.

Table of Contents

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)

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)

* 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 all of our planned clinical trials of SL-401.

Planned pivotal programs in BPDCN and third-line AML and regulatory strategy

We plan to initiate Company-sponsored trials of SL-401 in multiple hematologic cancers throughout 2014. These include programs of SL-401 in patients with BPDCN, and in patients with AML who failed two previous regimens, i.e., third-line AML, which we believe can serve as pivotal

trials in these indications. In

Table of Contents

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 plan to complete a pivotal Phase 2b single-arm trial of patients with BPDCN, with overall response rate, or ORR, as the primary endpoint, which we believe can serve as a pivotal trial in this indication. We plan to conduct the study in both North America and Europe. 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 plan to complete a Phase 3 randomized trial of SL-401 in AML patients who failed two previous treatments, i.e., third line AML, with overall survival, or OS, as the primary endpoint, which we believe can serve as a pivotal trial in this indication. We plan to conduct this study in North America and potentially in Europe. Patients with 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 overall survival, or 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. 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.

SL-401 clinical trials in additional hematologic indications

In addition, we plan to initiate Phase 2 trials of SL-401 in additional hematologic cancers, which may include multiple myeloma, earlier stages of AML (i.e., second- and first-line; either combined with standard treatment regimens or as a single agent as maintenance therapy for minimum residual disease, or MRD), MDS, CML, Hodgkin's and certain non-Hodgkin's lymphomas, as well as several rare IL-3R-expressing malignancies such as hairy cell leukemia mastocytosis, basophilic leukemia, and eosinophilic leukemia. We believe that some of these trials could be expanded to serve as platform studies for potential registration. Accordingly, we believe that SL-401 could be active in many hematologic cancer indications thereby representing significant market opportunities for SL-401.

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

Overview

SL-701, a clinically active therapeutic cancer vaccine comprised of synthetic peptides, is designed to direct the immune system to attack targets present on the CSCs and tumor bulk of brain cancer. High-grade gliomas, or HGGs, are the most aggressive brain cancers and have a poor prognosis. Treatment options are limited, particularly for adult patients with recurrent or refractory HGG, including glioblastoma multiforme, or GBM, and pediatric patients with HGG, including brainstem glioma, or BSG, and non-brainstem glioma. In two completed investigator-sponsored Phase 1/2 clinical trials, SL-701 demonstrated single agent anti-tumor activity, uncommon for a cancer vaccine, in these indications, inducing tumor shrinkage or disease stabilization in 59% (13/22) of HLA-A2+ (as defined below) adult patients with recurrent or refractory HGG (the 701 Adult-RHGG Study), and 87% (26/30) of HLA-A2+ pediatric glioma patients (the 701 Ped-G Study).

Table of Contents

To date, there have been eight major objective tumor responses (i.e., tumor regressions) in these two studies, consisting of two CRs and six partial responses, or PRs. SL-701 has also induced an additional tumor shrinkage, in the form of a minor response, or MR, a prolonged disease free survival following complete surgical resection (i.e. a clinical complete response, or CCR), as well as four additional cases of radiological evidence of anti-tumor activity.

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 initiate Company-sponsored trials in 2014 with SL-701 in adult and pediatric brain cancers. In particular, we plan to complete a Phase 2b trial of SL-701 in adult patients with second-line GBM. The design of this trial may enable SL-701 to obtain accelerated regulatory approval or serve as the foundation for a subsequent Phase 3 pivotal trial in this indication. We also plan to complete a Phase 2 trial of SL-701 in pediatric patients with brainstem and non-brainstem high grade glioma, which are areas of unmet medical need.

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

Gliomas are histologically heterogeneous tumors that are derived from glial cells in the brain. Gliomas are graded from 1 to 4, based on WHO classifications, with grade 4 glioma (i.e., glioblastoma, or GBM) and grade 3 glioma (i.e., anaplastic 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® received accelerated, but not full, approval 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 brainstem glioma, or BSG, is a highly malignant disease with very poor outcomes. The annual incidence of pediatric HGG is approximately 1,600 to 2,000 in the United States and approximately 3,400 in Europe. No therapy has been shown to have a favorable outcome in this population and almost all patients relapse after receiving first-line treatment. Pediatric patients with newly diagnosed HGG are typically treated with surgery, chemotherapy and/or radiation and have an expected median OS from diagnosis of less than one year.

Design of SL-701 and mechanism of action

SL-701 is a therapeutic cancer vaccine comprised of short synthetic peptides that correspond to epitopes of the brain cancer targets IL-13Rα2 and EphA2. The IL-13Rα2 synthetic peptide is a novel mutant designed to be highly immunogenic to amplify the vaccine's clinical anti-tumor immune response.

Table of Contents

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.

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 adult, recurrent, high-grade glioma

In a completed investigator-sponsored 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 and 701 Adult-LGG Study, in which SL-701 was administered to patients and demonstrated activity as a direct subcutaneous injection. The 701 Adult-RHGG Study was a single-arm trial whose objectives were to determine the general safety, dosage and efficacy of SL-701.

Well-tolerated at clinically active doses

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

Clinical activity

In the 701 Adult-RHGG Study, 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

Table of Contents

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)

Completed Phase 1/2 clinical trial pediatric glioma

In a completed investigator-sponsored 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 30 HLA-A2+ pediatric patients with glioma. Twenty of these patients had newly diagnosed brainstem glioma, or BSG, four had newly diagnosed non-brainstem HGG, three had recurrent non-brainstem HGG and three had multiply recurrent low-grade glioma, or LGG. Patients received a direct subcutaneous injection of SL-701 in the right or left upper arms associated with intact draining auxiliary lymph nodes once every three weeks with a separate concurrent injection of an adjuvant. The 701 Ped-G Study was a single-arm trial whose objectives were to determine the general safety, dosage and efficacy of SL-701.

Well-tolerated at clinically active doses

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

Table of Contents

Clinical activity

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

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

Positive immunological assays (both ELISPOT and tetramer assays) were demonstrated in six of seven evaluable children, including the newly diagnosed BSG pediatric patient who sustained a durable PR that lasted 15 months. We believe that these data indicate that SL-701 treatment stimulated the immune system in a highly specific fashion.

Low-grade glioma trial in adult patients

There is currently an investigator-sponsored study of SL-701 open in adult patients with LGG (the 701 Adult-LGG Study). Twenty-three HLA-A2+ patients have been enrolled, including twelve with newly diagnosed high-risk LGG without prior radiotherapy, one with newly diagnosed high-risk LGG with prior radiotherapy and ten with recurrent LGG. Patients were treated with SL-701 via direct subcutaneous injection every three weeks. 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. A positive correlation between immune response and progression-free survival, or PFS, was noted. Although a thorough evaluation of PFS requires a longer observation period, among 17 patients who completed eight courses, 10 had stable disease. 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 2 clinical trials and regulatory strategy

Adult trial

We plan to initiate Company-sponsored trials of SL-701 in adult and pediatric brain cancers throughout 2014. In particular, we plan to complete a Phase 2b trial of SL-701 in up to 100 adult patients with second-line GBM. In this trial, we plan to administer SL-701 as a single agent. The design of this study may enable SL-701 to obtain accelerated approval or serve as the foundation for a subsequent pivotal Phase 3 trial in this indication.

Table of Contents

Pediatric trial

We plan to complete a Phase 2 trial in children with brainstem and non-brainstem high-grade glioma, both of which represent an unmet medical need. We are currently collaborating with the Pediatric Brain Tumor Consortium, or PBTC, to conduct this study. The PBTC was formed by the NCI and consists of leading 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.

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-targeted compounds. This platform is differentiated from 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 on a miniaturized scale of many compounds for potential anti-CSC activity. We believe that this approach represents a major technological advance in oncology drug discovery. We have utilized StemScreen® to discover several of our preclinical product candidates. We believe that this robust platform will be instrumental in the potential 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.

StemScreen®-2

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

StemScreen®-2 utilizes an assay that uses live cells to track and follow CSCs in their natural state during high throughput screening and permits the rapid testing of many compounds on a small scale for enhanced efficiency. In particular, StemScreen®-2 utilizes a CSC-specific promoter linked to a reporter as a method

Table of Contents

for identifying and following CSCs in their native environment of surrounding tumor bulk, as illustrated below. In this way, StemScreen®-2 enables the identification of compound "hits," in a high throughput manner, with anti-CSC activity.

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

An initial screen of a moderately sized chemical compound library led to the identification of several "hits," comprising 2.4% of the library which demonstrated activity against CSCs with greater than 50% growth inhibition. Several of these compounds were then further validated using secondary functional assays to confirm anti-CSC activity. We plan to further optimize and miniaturize StemScreen®-2 for larger scale screening as well as expand its applicability for use in a broad range of tumor types. We plan to conduct these studies via fee-for-service contract research organizations, or CROs, as well as potentially within an internal research laboratory infrastructure that we may choose to build out in the future.

Preclinical pipeline

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

Table of Contents

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

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

Table of Contents

Principal stockholders

The following table sets forth information with respect to the beneficial ownership of our common stock as of October 1, 2013, on an actual and as adjusted basis to reflect the sale of our common stock offered by this prospectus by:

- each of our directors;
- each of our named executive officers;
- all of our directors and executive officers as a group; and
- each person, or group of affiliated persons, who is known by us to beneficially own more than 5% of our common stock.

Beneficial ownership is determined in accordance with the rules and regulations of the SEC and includes voting or investment power with respect to our common stock. Shares of our common stock subject to options and warrants that are currently exercisable or exercisable within 60 days of October 1, 2013 are considered outstanding and beneficially owned by the person holding the options or warrants for the purpose of calculating the percentage ownership of that person but not for the purpose of calculating the percentage ownership of any other person. Except as otherwise indicated in the footnotes below, we believe the persons and entities in this table have sole voting and investing power with respect to all of the shares of our common stock beneficially owned by them, subject to community property laws, where applicable. Except as otherwise indicated in the footnotes below, the address of the beneficial owner is c/o Stemline Therapeutics, Inc., 750 Lexington Avenue, Eleventh Floor, New York, New York 10022.

Name and address of beneficial owner	Number of shares beneficially owned before offering	Percentage of shares beneficially owned Before offering	After offering
Directors and Named Executive Officers			
Ivan Bergstein, M.D.	2,193,533(1)	16.53%	
Ron Bentsur	50,879(2)	*	
J. Kevin Buchi	13,772(3)	*	
Eric L. Dobmeier	7,963(4)	*	
Kenneth Zuerblis	9,763(5)	*	
Eric K. Rowinsky, M.D.	207,852(6)	1.60%	
Kenneth Hoberman	282,436(7)	2.15%	
Stephen P. Hall	40,000(8)	*	
John T. Cavan			
All directors and executive officers as a group (9 persons)	2,806,198(9)	20.57%	
5% Stockholders			
Ivan Bergstein, M.D.	2,193,533(1)	16.53%	
Fidelity Investments	1,482,956(11)	11.50%	
T. Rowe Price Associates	930,200(12)	7.22%	
Baker Bros Advisors L.P.	900,000(10)	6.98%	

* Represents beneficial ownership of less than one percent of our outstanding common stock.

(1) Consists of (i) 1,815,183 shares of common stock and (ii) 378,350 shares of common stock underlying options that are exercisable as of October 1, 2013 or will become exercisable within 60 days after such date.

Table of Contents

(2) Consists of (i) 3,252 shares of common stock and (ii) 47,627 shares of common stock underlying options that are exercisable as of October 1, 2013, or will become exercisable within 60 days after such date.

(3) Consists of (i) 500 shares of common stock and (ii) 13,272 shares of restricted stock, 6,636 of which shares have vested and 6,636 of which shares will vest in two equal annual installments on March 19, 2014, and March 19, 2015.

(4) Consists of 7,963 shares of restricted stock, 1,991 of which shares have vested and 5,972 of which shares will vest in three equal annual installments beginning on April 30, 2013.

(5) Consists of (i) 1,800 shares of common stock and (ii) 7,963 shares of restricted stock, 3,317 of which shares have vested and 2,654 of which shares will vest in two equal annual installments beginning on March 19, 2014, and March 19, 2015, and 1,992 of which shares will vest in three equal annual installments beginning on April 30, 2013.

(6) Consists of 142,869 shares of restricted stock, 11,956 of which have vested and 12,476 will vest on April 24, 2014 and 99,746 shares will vest in four equal annual installments beginning on April 24, 2015, and 18,701 will vest when the Company achieves an average market capitalization during any 30-trading day periond of $500 million and 64,983 shares of common stock underlying options that are exercisable as of October 1, 2013 or will become exercisable within 60 days after such date.

(7) Consists of (i) 19,288 shares of common stock, (ii) 263,148 shares of common stock underlying options that are exercisable as of October 1, 2013, or will become exercisable within 60 days after such date

(8) Consists of 40,000 shares of restricted stock, none of which shares have vested and 10,000 of which shares will vest in four equal annual installments beginning on June 19, 2014.

(9) Consists of (i) 2,052,090 shares of common stock, (ii) 754,108 shares of common stock underlying options that are exercisable as of October 1, 2013 or will become exercisable within 60 days after such date and (iii) 212,067 shares of restricted stock.

(10) Consists of 900,000 shares of common stock held by Baker Bros Advisors L.P. Based solely on a schedule 13F filed with the Securities and Exchange Commission for the quarter ending June 30, 2013. All shares were acquired in 2013. The address of Baker Bros Advisors L.P. is 667 Madison Avenue, New York, NY 10065.

(11) Consists of 1,482,956 shares of common stock held by Fidelity Investments and affiliates. Based solely on a Schedule 13G filed with the Securities and Exchange Commission on July 10, 2013. All shares were acquired in 2013. The address of Fidelity Investments is 245 Summer Street, Boston, MA 02210.

(12) Based solely on a Schedule 13F filed with the Securities and Exchange Commission for the quarter ending June 30, 2013 consists of 930,200 shares of common stock. All shares were acquired in 2013. The address of T. Rowe Price Associates, Inc. is 100 East Pratt Street, Baltimore, MD 21202.

Table of Contents

Market for our common stock

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 29, 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 such period.

As of October , 2013, the last reported sale price of our common stock was $, as reported by The NASDAQ Capital Market. The following table sets forth the ranges of high and low sales prices per share of our common stock as reported on The NASDAQ Capital Market for the period indicated. Such quotations represent inter-dealer prices without retail markup, markdown or commission and may not necessarily represent actual transactions.

	High	Low
Year ended December 31, 2013		
First Quarter (beginning January 29, 2013)	13.69	10.47
Second Quarter	25.84	11.19
Third Quarter	45.30	23.10
Fourth Quarter (through October , 2013)		

Holders

The number of record holders of our shares of outstanding common stock as of October , 2013 was . 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.

Table of Contents

Description of capital stock

General

The following description of our capital stock and provisions of our certificate of incorporation and bylaws are summaries and are qualified by reference to the certificate of incorporation and the bylaws that are currently in effect.

Our authorized capital stock consists of 33,750,000 shares of our common stock, par value $0.0001 per share, and 5,000,000 shares of our preferred stock, par value $0.0001 per share, of which all preferred stock will be undesignated.

Common stock

Holders of our common stock are entitled to one vote for each share held on all matters submitted to a vote of stockholders and do not have cumulative voting rights. Holders of our common stock are not entitled to vote on any amendment to our certificate of incorporation that relates solely to the terms of one or more outstanding series of preferred stock if the holders of such affected series are entitled, either separately or together as a class with the holders of one or more other such series, to vote thereon pursuant to the certificate of incorporation. An election of directors by our stockholders shall be determined by a plurality of the votes cast by the stockholders entitled to vote on the election. Holders of common stock are entitled to receive proportionately any dividends as may be declared by our board of directors, subject to any preferential dividend rights or other rights of outstanding preferred stock.

In the event of our liquidation or dissolution, the holders of common stock are entitled to receive proportionately all assets available for distribution to stockholders after the payment of all debts and other liabilities and subject to the preferential or other rights of any outstanding preferred stock. Holders of common stock have no preemptive, subscription, redemption or conversion rights. The rights, preferences and privileges of holders of common stock are subject to, and may be adversely affected by, the rights of the holders of shares of any series of preferred stock that we may designate and issue in the future.

Preferred stock

Under the terms of our certificate of incorporation, our board of directors is authorized to issue shares of preferred stock in one or more series without stockholder approval. Our board of directors has the discretion to determine the rights, preferences, privileges and restrictions, including voting rights, dividend rights, conversion rights, redemption privileges and liquidation preferences, of each series of preferred stock.

The purpose of authorizing our board of directors to issue preferred stock and determine its rights and preferences is to eliminate delays associated with a stockholder vote on specific issuances. The issuance of preferred stock, while providing flexibility in connection with possible acquisitions, future financings and other corporate purposes, could have the effect of making it more difficult for a third party to acquire, or could discourage a third party from seeking to acquire, a majority of our outstanding voting stock. There are no shares of preferred stock presently outstanding, there will be no shares of preferred stock outstanding upon the closing of this offering, and we have no present plans to issue any shares of preferred stock.

Table of Contents

Stock options

As of September 30, 2013, options to purchase 1,484,315 shares of our common stock at a weighted average exercise price of $4.27 per share were outstanding under our 2004 Equity Plan and 2012 Equity Plan.

Warrants

As of September 30, 2013, warrants to purchase 99,529 shares of common stock were outstanding at an exercise price of $15.00 per share. The warrant agreement that governs such warrants contains certain piggyback and demand registration rights.

Anti-takeover provisions

We are subject to Section 203 of the Delaware General Corporation Law. Subject to certain exceptions, Section 203 prevents a publicly held Delaware corporation from engaging in a "business combination" with any "interested stockholder" for three years following the date that the person became an interested stockholder, unless the interested stockholder attained such status with the approval of our board of directors or unless the business combination is approved in a prescribed manner. A "business combination" includes, among other things, a merger or consolidation involving us and the "interested stockholder" and the sale of more than 10% of our assets. In general, an "interested stockholder" is any entity or person beneficially owning 15% or more of our outstanding voting stock and any entity or person affiliated with or controlling or controlled by such entity or person.

Staggered board; removal of directors

Our certificate of incorporation and our bylaws divide our board of directors into three classes with staggered three-year terms. In addition, a director may be removed only for cause and only by the affirmative vote of the holders of at least 75% of the votes that all our stockholders would be entitled to cast in an annual election of directors. Any vacancy on our board of directors, including a vacancy resulting from an enlargement of our board of directors, may be filled only by vote of a majority of our directors then in office.

The classification of our board of directors and the limitations on the removal of directors and filling of vacancies could make it more difficult for a third party to acquire, or discourage a third party from seeking to acquire, control of our Company.

Super-majority voting

The Delaware General Corporation Law provides generally that the affirmative vote of a majority of the shares entitled to vote on any matter is required to amend a corporation's certificate of incorporation or by-laws, unless a corporation's certificate of incorporation or by-laws, as the case may be, requires a greater percentage. Our by-laws may be amended or repealed by a majority vote of our board of directors or the affirmative vote of the holders of at least 75% of the votes that all our stockholders would be entitled to cast in an annual election of directors. In addition, the affirmative vote of the holders of at least 75% of the votes which all our stockholders would be entitled to cast in an election of directors is required to amend or repeal or to adopt any provisions inconsistent with any of the provisions of our certificate of incorporation described in the prior two paragraphs.

Table of Contents

Stockholder action; special meeting of stockholders

Our certificate of incorporation provides that any action required or permitted to be taken by our stockholders must be effected at a duly called annual or special meeting of such stockholders and may not be effected by any consent in writing by such stockholders. Our certificate of incorporation and our amended and restated bylaws also provide that, except as otherwise required by law, special meetings of our stockholders can only be called by our chairman of the board, our chief executive officer or our board of directors and business transacted at any special meeting is limited to the stated purposes of the meeting.

Authorized but unissued shares

The authorized but unissued shares of common stock and preferred stock are available for future issuance without stockholder approval, subject to any limitations imposed by the listing standards of The NASDAQ Capital Market. These additional shares may be used for a variety of corporate finance transactions, acquisitions and employee benefit plans. The existence of authorized but unissued and unreserved common stock and preferred stock could make more difficult or discourage an attempt to obtain control of us by means of a proxy contest, tender offer, merger or otherwise.

Transfer agent and registrar

The transfer agent and registrar for our common stock is Computershare Trust Company, N.A.

The NASDAQ Capital Market

Our common stock is listed on The NASDAQ Capital Market under the symbol "STML."

Table of Contents

Shares eligible for future sale

Our common stock trades on The NASDAQ Capital Market. Future sales of substantial amounts of our common stock in the public market, including shares issued upon exercise of outstanding options and warrants, or the anticipation of these sales, could materially and adversely affect market prices prevailing from time to time and could impair our ability to raise capital through sales of equity or equity-related securities.

Upon the closing of this offering, we will have outstanding an aggregate of shares of our common stock, assuming the representatives of the underwriters do not exercise the option to purchase additional shares. Of the outstanding shares of common stock, shares will be "restricted securities" under Rule 144, and of these restricted securities will be subject to the 90-day lock-up period under the lock-up agreements as described below. These restricted securities may be sold in the public market only if registered or pursuant to an exemption from registration, such as Rule 144 or Rule 701 under the Securities Act.

Rule 144

Affiliate resales of restricted securities

In general, subject to the lock-up restrictions described below, a person who is an affiliate of ours, or who was an affiliate at any time during the 90 days before a sale, who has beneficially owned shares of our common stock for at least six months would be entitled to sell in "broker's transactions" or certain "riskless principal transactions" or to market makers, a number of shares within any three-month period that does not exceed the greater of:

- 1% of the number of shares of our common stock then outstanding, which will equal approximately shares immediately after this offering; or
- the average weekly trading volume in our common stock on The NASDAQ Capital Market during the four calendar weeks preceding the filing of a notice on Form 144 with respect to such sale.

Affiliate resales under Rule 144 are also subject to the availability of current public information about us. In addition, if the number of shares being sold under Rule 144 by an affiliate during any three-month period exceeds 5,000 shares or has an aggregate sale price in excess of $50,000, the seller must file a notice on Form 144 with the SEC and The NASDAQ Capital Market concurrently with either the placing of a sale order with the broker or the execution directly with a market maker.

Non-affiliate resales of restricted securities

In general, subject to the lock-up restrictions described above, a person who is not an affiliate of ours at the time of sale, and has not been an affiliate at any time during the three months preceding a sale, and who has beneficially owned shares of our common stock for at least six months but less than a year, is entitled to sell such shares subject only to the availability of current public information about us.

If such person has held our shares for at least one year, such person can resell under Rule 144(b)(1) without regard to any Rule 144 restrictions, including the current public information requirement.

Non-affiliate resales are not subject to the manner of sale, volume limitation or notice filing provisions of Rule 144.

Upon expiration of the 90 day lock-up period described below, approximately shares of our common stock will be eligible for sale under Rule 144, including shares eligible for resale immediately

Table of Contents

upon the closing of this offering as described above. We cannot estimate the number of shares of our common stock that our existing stockholders will elect to sell under Rule 144.

Rule 701

Rule 701 generally allows a stockholder who purchased shares of our common stock pursuant to a written compensatory plan or contract and who is not deemed to have been an affiliate of ours during the immediately preceding 90 days to sell these shares in reliance upon Rule 144, but without being required to comply with the public information, holding period, volume limitation, or notice provisions of Rule 144. Rule 701 also permits affiliates of ours to sell their Rule 701 shares under Rule 144 without complying with the holding period requirements of Rule 144.

Lock-up agreements

We and each of our directors and executive officers have agreed that, without the prior written consent of the representatives on behalf of the underwriters, we and they will not, subject to limited exceptions, during the period ending 90 days after the date of this prospectus, subject to extension in specified circumstances:

- offer, pledge, sell or contract to sell, sell any option or contract to purchase, purchase any option or contract to sell, grant any option, right or warrant to purchase, lend, or otherwise transfer or dispose of, directly or indirectly, any shares of common stock or any securities convertible into or exercisable or exchangeable for common stock;

- enter into any swap or other arrangement that transfers to another, in whole or in part, any of the economic consequences of ownership of our common stock or any securities convertible into or exchangeable or exercisable for shares of our common stock, whether such transaction is to be settled by delivery of shares of our common stock or such other securities, in cash or otherwise;

- make any demand for or exercise any right with respect to the registration of any shares of our common stock or any securities convertible into or exchangeable or exercisable for shares of our common stock; or

- publicly announce an intention to do any of the foregoing.

The lock-up restrictions, specified exceptions and the circumstances under which the 90-day lock-up period may be extended are described in more detail under "Underwriting."

Stock options

As of September 30, 2013, we had outstanding options to purchase 1,484,315 shares of our common stock, of which options to purchase 956,174 shares were vested. We have filed a registration statement on Form S-8 under the Securities Act to register all of the shares of our common stock subject to outstanding options and options and other awards issued or issuable pursuant to our 2012 Equity Plan and shares of common stock subject to outstanding options issued pursuant to our 2004 Equity Plan. Accordingly, shares of our common stock registered under the registration statements will be available for sale in the open market, subject to Rule 144 volume limitations applicable to affiliates, and subject to any vesting restrictions and lock-up agreements applicable to these shares.

Warrants

As of September 30, 2013, warrants to purchase 99,529 shares of common stock were outstanding at an exercise price of $15.00 per share. The warrant agreement that governs such warrants contains certain piggyback and demand registration rights.

Table of Contents

Underwriting

We are offering the shares of common stock described in this prospectus through a number of underwriters. J.P. Morgan Securities LLC and Jefferies LLC are acting as joint book-running managers of the offering and as representatives of the underwriters. We have entered into an underwriting agreement with the underwriters. Subject to the terms and conditions of the underwriting agreement, we have agreed to sell to the underwriters, and each underwriter has severally agreed to purchase, at the public offering price less the underwriting discounts and commissions set forth on the cover page of this prospectus, the number of shares of common stock listed next to its name in the following table:

Name	Number of shares
J.P. Morgan Securities LLC	
Jefferies LLC	
Aegis Capital Corp.	
Roth Capital Partners, LLC	
Ladenburg Thalmann & Co. Inc.	
H.C. Wainwright & Co., LLC	
Total	

The underwriters are committed to purchase all the common shares offered by us if they purchase any shares. The underwriting agreement also provides that if an underwriter defaults, the purchase commitments of non-defaulting underwriters may also be increased or the offering may be terminated.

The underwriters propose to offer the common shares directly to the public at the public offering price set forth on the cover page of this prospectus and to certain dealers at that price less a concession not in excess of $ per share. Any such dealers may resell shares to certain other brokers or dealers at a discount of up to $ per share from the public offering price. After the public offering of the shares, the offering price and other selling terms may be changed by the underwriters. Sales of shares made outside of the United States may be made by affiliates of the underwriters.

The underwriters have an option to buy up to additional shares of common stock from us at the public offering price, less the underwriting discount, to cover sales of shares by the underwriters which exceed the number of shares specified in the table above. The underwriters have 30 days from the date of this prospectus to exercise this option to purchase additional shares. If any shares are purchased with this option, the underwriters will purchase shares in approximately the same proportion as shown in the table above. If any additional shares of common stock are purchased, the underwriters will offer the additional shares on the same terms as those on which the shares are being offered.

The underwriting fee is equal to the public offering price per share of common stock less the amount paid by the underwriters to us per share of common stock. The underwriting fee is $ per share. The following table shows the per share and total underwriting discounts and commissions to be paid to the

Table of Contents

underwriters assuming both no exercise and full exercise of the underwriters' option to purchase additional shares.

	Without exercise of option to purchase additional shares	With full exercise of option to purchase additional shares
Per Share	$	$
Total	$	$

We have agreed to pay Trout Capital LLC, a FINRA member, a fee for financial advisory services provided in connection with this offering.

We estimate that the total expenses of this offering, including registration, filing and listing fees, printing fees and legal and accounting expenses, but excluding the underwriting discounts and commissions, will be approximately $. We have agreed to pay the filing fees incidental to, and the fees and disbursements of counsel for the underwriter in connection with, any required review by FINRA in connection with this offering.

A prospectus in electronic format may be made available on the web sites maintained by one or more underwriters, or selling group members, if any, participating in the offering. The underwriters may agree to allocate a number of shares to underwriters and selling group members for sale to their online brokerage account holders. Internet distributions will be allocated by the representatives to underwriters and selling group members that may make Internet distributions on the same basis as other allocations.

We have agreed that we will not (i) offer, pledge, announce the intention to sell, sell, contract to sell, sell any option or contract to purchase, purchase any option or contract to sell, grant any option, right or warrant to purchase or otherwise transfer or dispose of, directly or indirectly, or file with the Securities and Exchange Commission, or SEC, a registration statement under the Securities Act of 1933, as amended, or the Securities Act, relating to, any shares of our common stock or securities convertible into or exchangeable or exercisable for any shares of our common stock, or publicly disclose the intention to make any offer, sale, pledge, disposition or filing, or (ii) enter into any swap or other arrangement that transfers all or a portion of the economic consequences associated with the ownership of any shares of common stock or any such other securities (regardless of whether any of these transactions are to be settled by the delivery of shares of common stock or such other securities, in cash or otherwise), in each case without the prior written consent of J.P. Morgan Securities LLC and Jefferies LLC on behalf of the underwriters for a period of 90 days after the date of this prospectus.

The restrictions described in the immediately preceding paragraph do not apply, subject to certain conditions, to the following:

- the sale of shares of common stock pursuant to the underwriting agreement;
- the issuance of shares of our common stock pursuant to our stock plans, so long as the recipients of such securities shall sign and deliver a lock-up agreement;
- the issuance of shares of our common stock upon the exercise of warrants outstanding on the date hereof;

Table of Contents

the filing by us of any Registration Statement on Form S-8 or a successor form thereto; or

the issuance of shares of common stock or securities convertible into or exercisable or exchangeable for shares of common stock representing in the aggregate no more than 5% of our outstanding shares of common stock immediately following the closing of this offering, which may be sold only in connection with a transaction that includes a commercial relationship (including joint ventures, marketing or distribution arrangements, collaboration agreements or intellectual property license agreements) or any acquisition of assets or not less than a majority or controlling portion of the equity of another entity, so long as the recipients of such securities shall sign and deliver a lock-up agreement.

Our directors and executive officers have entered into lock-up agreements with the underwriters prior to the commencement of this offering pursuant to which each of these persons or entities, with limited exceptions, for a period of 90 days after the date of this prospectus, may not, without the prior written consent of J.P. Morgan Securities LLC and Jefferies LLC on behalf of the underwriters, (1) offer, pledge, announce the intention to sell, sell, contract to sell, sell any option or contract to purchase, purchase any option or contract to sell, grant any option, right or warrant to purchase, or otherwise transfer or dispose of, directly or indirectly, any shares of our common stock or any securities convertible into or exercisable or exchangeable for our common stock (including, without limitation, common stock or such other securities which may be deemed to be beneficially owned by such directors, executive officers, managers and members in accordance with the rules and regulations of the SEC and securities which may be issued upon exercise of a stock option or warrant) or (2) enter into any swap or other agreement that transfers, in whole or in part, any of the economic consequences of ownership of the common stock or such other securities, whether any such transaction described in clause (1) or (2) above is to be settled by delivery of common stock or such other securities, in cash or otherwise, or (3) make any demand for or exercise any right with respect to the registration of any shares of our common stock or any security convertible into or exercisable or exchangeable for our common stock.

The restrictions described in the immediately preceding paragraph do not apply, subject to certain conditions, to the following:

the transfer of shares of our common stock as a bona fide gift or gifts;

the transfer to any trust for the direct or indirect benefit of the individual executing the lock-up agreement or a member of his or her immediate family in a transaction not involving a disposition for value;

the transfer by will, other testamentary document or intestate succession;

a distribution to partners, members or stockholders of the individual executing the lock-up agreement in a transaction not involving a disposition for value;

the exercise of options to purchase shares of common stock granted under our stock plans;

transfers of shares of common stock to us by the individual executing the lock-up agreement to satisfy tax withholding obligations upon the vesting of awards under our stock plans or upon the net or cashless exercise of stock options under our stock plans; or

the establishment of a trading plan pursuant to Rule 10b5-1 under the Securities Exchange Act of 1934, as amended, for the transfer of common stock.

Table of Contents

We have agreed to indemnify the underwriters against certain liabilities, including liabilities under the Securities Act.

Our common stock is listed on The NASDAQ Capital Market under the symbol "STML".

In connection with this offering, J.P. Morgan Securities LLC on behalf of the underwriters may engage in stabilizing transactions, which involve making bids for, purchasing and selling shares of common stock in the open market for the purpose of preventing or retarding a decline in the market price of the common stock while this offering is in progress. These stabilizing transactions may include making short sales of the common stock, which involves the sale by the underwriters of a greater number of shares of common stock than they are required to purchase in this offering, and purchasing shares of common stock on the open market to cover positions created by short sales. Short sales may be "covered" shorts, which are short positions in an amount not greater than the underwriters' option to purchase additional shares referred to above, or may be "naked" shorts, which are short positions in excess of that amount. The underwriters may close out any covered short position either by exercising their option to purchase additional shares, in whole or in part, or by purchasing shares in the open market. In making this determination, the underwriters will consider, among other things, the price of shares available for purchase in the open market compared to the price at which the underwriters may purchase shares through the option to purchase additional shares. A naked short position is more likely to be created if the underwriters are concerned that there may be downward pressure on the price of the common stock in the open market that could adversely affect investors who purchase in this offering. To the extent that the underwriters create a naked short position, they will purchase shares in the open market to cover the position.

The underwriters have advised us that, pursuant to Regulation M of the Securities Act, they may also engage in other activities that stabilize, maintain or otherwise affect the price of the common stock, including the imposition of penalty bids. This means that if the representatives of the underwriters purchase common stock in the open market in stabilizing transactions or to cover short sales, the representatives can require the underwriters that sold those shares as part of this offering to repay the underwriting discount received by them.

These activities may have the effect of raising or maintaining the market price of the common stock or preventing or retarding a decline in the market price of the common stock, and, as a result, the price of the common stock may be higher than the price that otherwise might exist in the open market. If the underwriters commence these activities, they may discontinue them at any time. The underwriters may carry out these transactions on The NASDAQ Capital Market, in the over-the-counter market or otherwise.

In addition, in connection with this offering certain of the underwriters (and selling group members) may engage in passive market making transactions in our common stock on The NASDAQ Capital Market prior to the pricing and completion of this offering. Passive market making consists of displaying bids on The NASDAQ Capital Market no higher than the bid prices of independent market makers and making purchases at prices no higher than these independent bids and effected in response to order flow. Net purchases by a passive market maker on each day are generally limited to a specified percentage of the passive market maker's average daily trading volume in the common stock during a specified period and must be discontinued when such limit is reached. Passive market making may cause the price of our common stock to be higher than the price that otherwise would exist in the open market in the absence of these transactions. If passive market making is commenced, it may be discontinued at any time.

Table of Contents

Other than in the United States, no action has been taken by us or the underwriters that would permit a public offering of the securities offered by this prospectus in any jurisdiction where action for that purpose is required. The securities offered by this prospectus may not be offered or sold, directly or indirectly, nor may this prospectus or any other offering material or advertisements in connection with the offer and sale of any such securities be distributed or published in any jurisdiction, except under circumstances that will result in compliance with the applicable rules and regulations of that jurisdiction. Persons into whose possession this prospectus comes are advised to inform themselves about and to observe any restrictions relating to the offering and the distribution of this prospectus. This prospectus does not constitute an offer to sell or a solicitation of an offer to buy any securities offered by this prospectus in any jurisdiction in which such an offer or a solicitation is unlawful.

This document is only being distributed to and is only directed at (i) persons who are outside the United Kingdom or (ii) to investment professionals falling within Article 19(5) of the Financial Services and Markets Act 2000 (Financial Promotion) Order 2005 (the "Order") or (iii) high net worth entities, and other persons to whom it may lawfully be communicated, falling with Article 49(2)(a) to (d) of the Order (all such persons together being referred to as "relevant persons"). The securities are only available to, and any invitation, offer or agreement to subscribe, purchase or otherwise acquire such securities will be engaged in only with, relevant persons. Any person who is not a relevant person should not act or rely on this document or any of its contents.

In relation to each Member State of the European Economic Area which has implemented the Prospectus Directive (each, a "Relevant Member State"), from and including the date on which the European Union Prospectus Directive (the "EU Prospectus Directive") was implemented in that Relevant Member State (the "Relevant Implementation Date") an offer of securities described in this prospectus may not be made to the public in that Relevant Member State prior to the publication of a prospectus in relation to the shares which has been approved by the competent authority in that Relevant Member State or, where appropriate, approved in another Relevant Member State and notified to the competent authority in that Relevant Member State, all in accordance with the EU Prospectus Directive, except that, with effect from and including the Relevant Implementation Date, an offer of securities described in this prospectus may be made to the public in that Relevant Member State at any time:

- to any legal entity which is a qualified investor as defined under the EU Prospectus Directive;

- to fewer than 100 or, if the Relevant Member State has implemented the relevant provision of the 2010 PD Amending Directive, 150 natural or legal persons (other than qualified investors as defined in the EU Prospectus Directive); or

- in any other circumstances falling within Article 3(2) of the EU Prospectus Directive, provided that no such offer of securities described in this prospectus shall result in a requirement for the publication by us of a prospectus pursuant to Article 3 of the EU Prospectus Directive.

For the purposes of this provision, the expression an "offer of securities to the public" in relation to any securities in any Relevant Member State means the communication in any form and by any means of sufficient information on the terms of the offer and the securities to be offered so as to enable an investor to decide to purchase or subscribe for the securities, as the same may be varied in that Member State by any measure implementing the EU Prospectus Directive in that Member State. The expression "EU Prospectus Directive" means Directive 2003/71/EC (and any amendments thereto, including the 2010 PD Amending Directive, to the extent implemented in the Relevant Member State) and includes any relevant implementing measure in each Relevant Member State, and the expression "2010 PD Amending Directive" means Directive 2010/73/EU.

Table of Contents

Other relationships

Certain of the underwriters and their affiliates have historically provided, and may provide from time to time in the future, various advisory, investment and commercial banking and other services to us in the ordinary course of business, for which they have received and may continue to receive fees and commissions. In addition, from time to time, certain of the underwriters and their affiliates may effect transactions for their own account or the account of customers, and hold on behalf of themselves or their customers, long or short positions in our debt or equity securities or loans, and may do so in the future.

On June 18, 2013, we entered into an advisory agreement with Aegis Capital Corp. under which Aegis Capital Corp. provides us with certain advisory services, including advice on business and financial planning, corporate organization and structure, and the Company's offer or sale of securities, through January 1, 2014. Aegis received advisory fees in connection with this advisory agreement. Aegis Capital Corp. is acting as the lead manager of this offering.

Previously, Aegis Capital Corp. acted as the sole book-running manager for our initial public offering completed on January 31, 2013. Additionally, for our public offering of common stock completed on May 22, 2013, Jefferies LLC and Aegis Capital Corp. acted as joint book-running managers, and Roth Capital Partners, LLC, acted as co-lead manager.

Legal matters

The validity of the shares of common stock offered hereby is being passed upon for us by Alston & Bird LLP. Mintz, Levin, Cohn, Ferris, Glovsky and Popeo, P.C. is acting as counsel for the underwriters in connection with this offering.

Experts

Our financial statements at December 31, 2012 and 2011, and 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, incorporated by reference in this prospectus from Stemline Therapeutics, Inc.'s Annual Report on Form 10-K for the year ended December 31, 2012, have been incorporated herein by reference in reliance on the report of Ernst & Young LLP, an independent registered public accounting firm, given on the authority of said firm as experts in auditing and accounting.

Where you can find more information

We have filed with the SEC a registration statement on Form S-1 under the Securities Act with respect to the shares of common stock we are offering to sell. This prospectus, which constitutes part of the registration statement, does not include all of the information contained in the registration statement and the exhibits, schedules and amendments to the registration statement. For further information with respect to us and our common stock, we refer you to the registration statement and to the exhibits and schedules to the registration statement. Statements contained in this prospectus about the contents of any contract, agreement or other document are not necessarily complete, and, in each instance, we refer you to the copy of the contract, agreement or other document filed as an exhibit to the registration statement. Each of these statements is qualified in all respects by this reference.

You may read and copy the registration statement of which this prospectus is a part at the SEC's Public Reference Room, which is located at 100 F Street, N.E., Room 1580, Washington, DC 20549. You can

Table of Contents

request copies of the registration statement by writing to the Securities and Exchange Commission and paying a fee for the copying cost. Please call the SEC at 1-800-SEC-0330 for more information about the operation of the SEC's Public Reference Room. In addition, the SEC maintains a website, which is located at www.sec.gov, that contains reports, proxy and information statements and other information regarding issuers that file electronically with the SEC. You may access the registration statement of which this prospectus is a part at the SEC's website.

We are subject to the information reporting requirements of the Securities Exchange Act of 1934, and we file reports, proxy statements and other information with the SEC. All documents filed with the SEC are available for inspection and copying at the public reference room and website of the SEC referred to above. We maintain a website at www.stemline.com. You may access our reports, proxy statements and other information free of charge at this website as soon as reasonably practicable after such material is electronically filed with, or furnished to, the SEC. The information on such website is not incorporated by reference and is not a part of this prospectus.

Table of Contents

Incorporation of documents by reference

The SEC allows us to "incorporate by reference" information from other documents that we file with it, which means that we can disclose important information to you by referring you to those documents. The information incorporated by reference is considered to be part of this prospectus. Information in this prospectus supersedes information incorporated by reference that we filed with the SEC prior to the date of this prospectus.

We incorporate by reference into this prospectus and the registration statement of which this prospectus is a part the information or documents listed below that we have filed with the SEC (Commission File No. 001-35619).

- our Annual Report on Form 10-K for the fiscal year ended December 31, 2012, filed on April 1, 2013;
- our Quarterly Report on Form 10-Q for the quarter ended March 31, 2013, filed on May 14, 2013, as amended on June 12, 2013;
- our Quarterly Report on Form 10-Q for the quarter ended June 30, 2013, filed on August 14, 2013;
- our Current Reports on Form 8-K filed on February 6, 2013, March 29, 2013 and June 25, 2013 (other than the portions of those reports not deemed to be "filed");
- the portions of our Definitive Proxy Statement on Schedule 14A filed on April 30, 2013 that are deemed "filed" with the SEC under the Exchange Act; and
- the description of our common stock contained in our Registration Statement on Form 8-A filed on July 30, 2012, including any amendment or report filed for the purpose of updating such description.

Any statement contained in a document incorporated or deemed to be incorporated by reference in this prospectus will be deemed modified, superseded or replaced for purposes of this prospectus to the extent that a statement contained in this prospectus modifies, supersedes or replaces such statement.

You may request, orally or in writing, a copy of any or all of the documents incorporated herein by reference. These documents will be provided to you at no cost, by contacting: Stemline Therapeutics, Inc., 750 Lexington Avenue, Eleventh Floor, New York, NY 10022, (646) 502-2311, email address: info@stemline.com. In addition, copies of any or all of the documents incorporated herein by reference may be accessed at our website at www.stemline.com.

You should rely only on information contained in, or incorporated by reference into, this prospectus and any prospectus supplement. We have not authorized anyone to provide you with information different from that contained in this prospectus or incorporated by reference in this prospectus.

shares

Common stock

Prospectus

J.P. Morgan **Jefferies**

Aegis Capital Corp

Roth Capital Partners **Ladenburg Thalmann & Co. Inc.** **H.C. Wainwright & Co., LLC**

, 2013

Table of Contents

Part II Information not required in prospectus

Item 13. Other expenses of issuance and distribution.

The following table sets forth the expenses to be incurred in connection with the offering described in this Registration Statement, other than underwriting discounts and commissions, all of which will be paid by us. All amounts are estimates except the Securities and Exchange Commission registration fee and the Financial Industry Regulatory Authority, Inc., filing fee.

Securities and Exchange Commission registration fee	
Financial Industry Regulatory Authority, Inc., filing fee	
Accountants' fees and expenses	
Legal fees and expenses	
Blue sky fees and expenses	
Transfer agent's fees and expenses	
Printing and engraving expenses	
Miscellaneous	
Total expenses	

Item 14. Indemnification of directors and officers.

Section 102 of the Delaware General Corporation Law permits a corporation to eliminate the personal liability of its directors or its stockholders for monetary damages for a breach of fiduciary duty as a director, except where the director breached his or her duty of loyalty, failed to act in good faith, engaged in intentional misconduct or knowingly violated a law, authorized the payment of a dividend or approved a stock repurchase in violation of Delaware corporate law or obtained an improper personal benefit. Our certificate of incorporation provides that no director shall be personally liable to us or our stockholders for monetary damages for any breach of fiduciary duty as a director, notwithstanding any provision of law imposing such liability, except to the extent that the Delaware General Corporation Law prohibits the elimination or limitation of liability of directors for breaches of fiduciary duty.

Section 145 of the Delaware General Corporation Law provides that a corporation has the power to indemnify a director, officer, employee or agent of the corporation and certain other persons serving at the request of the corporation in related capacities against expenses (including attorneys' fees), judgments, fines and amounts paid in settlements actually and reasonably incurred by the person in connection with an action, suit or proceeding to which he or she is party or is threatened to be made a party by reason of such position, if such person acted in good faith and in a manner he or she reasonably believed to be in or not opposed to the best interests of the corporation, and, in any criminal action or proceeding, had no reasonable cause to believe his or her conduct was unlawful, except that, in the case of actions brought by or in the right of the corporation, no indemnification shall be made with respect to any claim, issue or matter as to which such person shall have been adjudged to be liable to the corporation unless and only to the extent that the Court of Chancery or other adjudicating court determines that, despite the adjudication of liability but in view of all of the circumstances of the case, such person is fairly and reasonably entitled to indemnification for such expenses which the Court of Chancery or such other court shall deem proper.

Our certificate of incorporation provides that we will indemnify each person who was or is a party or threatened to be made a party to any threatened, pending or completed action, suit or proceeding, whether civil, criminal, administrative or investigative (other than an action by or in the right of us) by

Table of Contents

reason of the fact that he or she is or was, or has agreed to become, our director or officer, or is or was serving, or has agreed to serve, at our request as a director, officer, partner, employee or trustee of, or in a similar capacity with, another corporation, partnership, joint venture, trust or other enterprise (including any employee benefit plan) (all such persons being referred to as an "Indemnitee"), or by reason of any action alleged to have been taken or omitted in such capacity, against all expenses (including attorneys' fees), liabilities, losses, judgments, fines, excise taxes and penalities arising under the Employee Retirement Income Security Act of 1974, and amounts paid in settlement actually and reasonably incurred in connection with such action, suit or proceeding and any appeal therefrom, if such Indemnitee acted in good faith and in a manner he or she reasonably believed to be in, or not opposed to, our best interests, and, with respect to any criminal action or proceeding, he or she had no reasonable cause to believe his or her conduct was unlawful.

Our certificate of incorporation also provides that we will indemnify any Indemnitee who was or is a party to any threatened, pending or completed action or suit by or in the right of us to procure a judgment in our favor by reason of the fact that the Indemnitee is or was, or has agreed to become, our director or officer, or is or was serving, or has agreed to serve, at our request as a director, officer, partner, employee or trustee of, or in a similar capacity with, another corporation, partnership, joint venture, trust or other enterprise (including any employee benefit plan), or by reason of any action alleged to have been taken or omitted in such capacity, against all expenses (including attorneys' fees) and, to the extent permitted by law, amounts paid in settlement actually and reasonably incurred in connection with such action, suit or proceeding, and any appeal therefrom, if the Indemnitee acted in good faith and in a manner he or she reasonably believed to be in, or not opposed to, our best interests, except that no indemnification shall be made with respect to any claim, issue or matter as to which such person shall have been adjudged to be liable to us, unless a court determines that, despite such adjudication but in view of all of the circumstances, he or she is entitled to indemnification of such expenses. Notwithstanding the foregoing, to the extent that any Indemnitee has been successful, on the merits or otherwise, he or she will be indemnified by us against all expenses (including attorneys' fees) actually and reasonably incurred by him or her or on his or her behalf in connection therewith. If we don't assume the defense, expenses must be advanced to an Indemnitee under certain circumstances.

We have entered into indemnification agreements with our directors. In general, these agreements provide that we will indemnify the director to the fullest extent permitted by law for claims arising in his or her capacity as a director of our Company or in connection with his or her service at our request for another corporation or entity. The indemnification agreements also provide for procedures that will apply in the event that a director makes a claim for indemnification and establish certain presumptions that are favorable to the director.

We maintain a general liability insurance policy which covers certain liabilities of our directors and officers arising out of claims based on acts or omissions in their capacities as directors or officers.

Additionally, reference is made to the Underwriting Agreement we will enter into in connection with the offering of common stock being registered hereby, which provides for indemnification by the underwriters of the Company, or directors and officers who sign the registration statement and persons who control the Company, under certain circumstances.

Item 15. Recent sales of unregistered securities.

Set forth below is information regarding securities sold by us within the past three years that were not registered under the Securities Act of 1933, as amended, or the Securities Act. Also included is the

Table of Contents

consideration, if any, received by us for such 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.

(a) Issuances of common stock and convertible notes

In January 2012, we sold an aggregate of $0.9 million of convertible promissory notes in a private placement to certain investors. The notes accrued interest at a rate of 1.27% per annum and had a maturity date of January 2, 2017, unless converted prior thereto. The principal amount of the notes and accrued and unpaid interest thereon were automatically converted into shares of our common stock upon the closing of our initial public offering in January 2013 at a conversion price equal to 87.5% of our initial public offering price.

In March 2012, we issued a total of 23,890 shares of restricted common stock to our directors and a service provider. These shares of restricted stock vested as to 25% of the award upon the closing of our initial public offering in January 2013, with the remaining 75% vesting in equal annual installments, as long as the respective party continues as a director or service provider, as applicable, through the third anniversary of the date of grant.

In April 2012, we issued a total of 10,617 shares of restricted common stock to certain of our directors. These shares of restricted stock vested as to 25% of the award upon the closing of our intial public offering in January 2013, with the remaining 75% vesting in equal annual installments, as long as the respective party continues as director, through the third anniversary of the date of grant.

No underwriters were involved in the foregoing sales of securities. The securities described in this section (a) of Item 15 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 shares of our common stock and convertible notes described above represented to us in connection with their purchase that they were accredited investors and were acquiring the shares and convertible notes 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 written 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.

On January 31, 2013 the Company completed its initial public offering ("IPO"). Upon the closing of the IPO, certain transactions were triggered based on a successful completion of an IPO. Convertible debt of $1.4 million principal, plus accrued interest thereon, was converted into 166,769 shares of common stock. Finally, certain options (discussed below) and restricted stock (discussed above) began to vest and fully vest to directors, consultants and key employees.

In April 2013, certain convertible notes were converted into 67,198 shares of our common stock.

In April 2013, the Company granted an employee an aggregate of 149,614 shares of restricted common stock. Of the 149,614 shares of restricted stock, 112,212 shares vest ratably over 5 years and 37,402 shares vest based on meeting certain market capitalization thresholds.

In June 2013, the Company granted employees an aggregate of 82,000 shares of restricted common stock. The restricted stock vests ratably over 4 years.

Table of Contents

(b) Stock option grants

From January 1, 2009 through April 24, 2013, we issued to employees, directors and consultants options to purchase an aggregate of 1,996,402 shares of common stock, of which, 374,972 have been exercised and 137,115 options have been forfeited, as of September 30, 2013.

The stock options and the common stock issuable upon the exercise of such options as described in this section (b) of Item 15 were issued pursuant to written compensatory plans or arrangements with our employees, directors and consultants, in reliance on the exemption from the registration requirements of the Securities Act provided by Rule 701 promulgated under the Securities Act or the exemption set forth in Section 4(2) under the Securities Act and Regulation D promulgated thereunder relative to transactions by an issuer not involving any public offering. All recipients either received adequate information about us or had access, through employment or other relationships, to such information.

All of the foregoing securities are deemed restricted securities for purposes of the Securities Act. All certificates representing the issued shares of common stock and the convertible notes described in this Item 15 included appropriate legends setting forth that the securities had not been registered and the applicable restrictions on transfer.

Item 16. Exhibits and financial statement schedules.

The exhibits to the Registration Statement are listed in the Exhibit Index attached hereto and incorporated by reference herein.

Item 17. Undertakings.

(a) Insofar as indemnification for liabilities arising under the Securities Act may be permitted to directors, officers and controlling persons of the registrant pursuant to the foregoing provisions, or otherwise, the registrant has been advised that, in the opinion of the Securities and Exchange Commission, such indemnification is against public policy as expressed in the Act and is, therefore, unenforceable. In the event that a claim for indemnification against such liabilities (other than the payment by the registrant of expenses incurred or paid by a director, officer or controlling person of the registrant in the successful defense of any action, suit or proceeding) is asserted by such director, officer or controlling person in connection with the securities being registered, the registrant will, unless in the opinion of its counsel the matter has been settled by controlling precedent, submit to a court of appropriate jurisdiction the question whether such indemnification by it is against public policy as expressed in the Act and will be governed by the final adjudication of such issue.

(b) The undersigned registrant hereby undertakes that:

(1) For purposes of determining any liability under the Securities Act, the information omitted from the form of prospectus filed as part of this registration statement in reliance upon Rule 430A and contained in a form of prospectus filed by the registrant pursuant to Rule 424(b)(1) or (4) or 497(h) under the Securities Act shall be deemed to be part of this registration statement as of the time it was declared effective.

(2) For the purpose of determining any liability under the Securities Act, each post-effective amendment that contains a form of prospectus shall be deemed to be a new registration statement relating to the securities offered therein, and the offering of such securities at that time shall be deemed to be the initial bona fide offering thereof.

Table of Contents

Signatures

Pursuant to the requirements of the Securities Act of 1933, the registrant has duly caused this Registration Statement to be signed on its behalf by the undersigned, thereunto duly authorized, in the City of New York, State of New York, on this 17th day of October, 2013.

STEMLINE THERAPEUTICS, INC.

By: /s/ IVAN BERGSTEIN, M.D.

Ivan Bergstein, M.D.
Chairman, President and Chief Executive Officer

Signatures and power of attorney

We, the undersigned officers and directors of Stemline Therapeutics, Inc., hereby severally constitute and appoint Ivan Bergstein, M.D., our true and lawful attorney-in-fact and agent, with full power of substitution and resubstitution in him for him and in his name, place and stead, and in any and all capacities, to sign any and all amendments (including post-effective amendments) to this Registration Statement, and any other registration statement for the same offering pursuant to Rule 462(b) under the Securities Act of 1933, and to file the same, with all exhibits thereto and other documents in connection therewith, with the Securities and Exchange Commission, granting unto said attorney-in-fact and agent full power and authority to do and perform each and every act and thing requisite or necessary to be done in and about the premises, as full 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 his substitute or substitutes, may lawfully do or cause to be done by virtue hereof.

Pursuant to the requirements of the Securities Act of 1933, this Registration Statement has been signed by the following persons in the capacities held on the dates indicated.

Signature	Title	Date
/s/ IVAN BERGSTEIN, M.D. Ivan Bergstein, M.D.	Chairman, President, Chief Executive Officer and Director (Principal Executive Officer)	October 17, 2013
/s/ STEPHEN P. HALL Stephen P. Hall	Vice President of Finance and Chief Accounting Officer (Principal Financial and Accounting Officer)	October 17, 2013
/s/ RON BENTSUR Ron Bentsur	Director	October 17, 2013

Table of Contents

Signature	Title	Date
/s/ J. KEVIN BUCHI J. Kevin Buchi	Director	October 17, 2013
/s/ ERIC L. DOBMEIER Eric L. Dobmeier	Director	October 17, 2013
/s/ KENNETH ZUERBLIS Kenneth Zuerblis	Director	October 17, 2013

Table of Contents

Exhibit index

Exhibit No.	Description
1.1♦	Underwriting Agreement.
3.1	Restated Certificate of Incorporation of Stemline Therapeutics, Inc., filed as Exhibit 3.1 to Form 8-K filed on February 6, 2013 (File No. 001-35619) and incorporated herein by reference.
3.2	Amended and Restated Bylaws of Stemline Therapeutics, Inc., filed as Exhibit 3.2 to Form 8-K filed on February 6, 2013 (File No. 001-35619) and incorporated herein by reference.
3.3	Certificate of Amendment of Restated Certificate of Incorporation of Stemline Therapuetics, Inc., filed as Exhibit 3.3 to Form 10-Q filed on August 14, 2013 (File No. 001-35619) and incorporated herein by reference.
4.1	Specimen certificate evidencing shares of common stock, filed as Exhibit 4.1 to Form S-1/A 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.
5.1♦	Opinion of Alston & Bird LLP.
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 11, 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.

Table of Contents

Exhibit No.	Description
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.

Table of Contents

Exhibit No.	Description
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.
23.1	Consent of Ernst & Young LLP, Independent Registered Public Accounting Firm.
23.2	Consent of Alston & Bird LLP (to be included in Exhibit 5.1).
24.1**	Power of Attorney (included on signature page).

♦ To be filed by amendment.

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.