

**UNITED STATES
SECURITIES AND EXCHANGE COMMISSION**

Washington, D.C. 20549

FORM 10-K

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

For the fiscal year ended December 31, 2019

OR

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

For the transition period from _____ to _____

Commission File Number: 001-35619

STEMLINE THERAPEUTICS, INC.

(Exact name of registrant as specified in its charter)

Delaware

(State or other jurisdiction of incorporation or
organization)

45-0522567

(I.R.S. Employer Identification Number)

750 Lexington Avenue

Eleventh Floor

New York, New York 10022

(Address of principal executive offices, including zip code)

(646)-502-2311

(Registrant's telephone number, including area code)

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

<u>Title of each class:</u>	<u>Trading Symbol(s)</u>	<u>Name of each exchange on which registered:</u>
Common Stock	STML	Nasdaq Capital Market

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

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

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

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

Indicate by check mark whether the registrant has submitted electronically every Interactive Data File required to be submitted pursuant to Rule 405 of Regulation S-T (§232.405 of this chapter) during the preceding 12 months (or for such shorter period that the registrant was required to submit such files). Yes No

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

Large accelerated filer	<input type="checkbox"/>	Accelerated filer	<input checked="" type="checkbox"/>
Non-accelerated filer	<input type="checkbox"/>	Smaller reporting company	<input checked="" type="checkbox"/>
		Emerging growth company	<input type="checkbox"/>

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

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

The aggregate market value of voting common stock held by non-affiliates of the registrant (assuming, for purposes of this calculation, without conceding, that all executive officers and directors are "affiliates") was \$ 612,997,628 as of June 30, 2019, based on the closing sale price of such stock as reported on the NASDAQ Capital Market.

There were 50,375,079 shares of the registrant's common stock outstanding as of March 16, 2020.

DOCUMENTS INCORPORATED BY REFERENCE

Portions of the registrant's Proxy Statement for the 2020 Annual Meeting of Stockholders are incorporated by reference in Part III of this Annual Report on Form 10-K.

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

SPECIAL CAUTIONARY NOTICE REGARDING FORWARD-LOOKING STATEMENTS

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

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

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

- the success of our launch and commercialization of ELZONRIS® in the U.S. and abroad;
- the success of our commercial infrastructure buildout, launch, and commercialization in the U.S. and potentially in other regions of the world should ELZONRIS be approved for marketing by ex-U.S. regulatory authorities for any indication or by U.S. regulatory authorities for any as yet unapproved indication or should any of our other product candidates be approved for marketing in the U.S. or abroad;
- the success and timing of our clinical trials for ELZONRIS in unapproved indications and our other product candidates, including risks relating to regulatory authority approval, institutional review board approval, scientific review committee approval, site initiation, patient accrual, trial results including safety and efficacy, and the relevance of trial results to potentially viable regulatory pathways and commercialization efforts;
- the possibility that results of clinical trials are not predictive of safety and efficacy results of our products or product candidates in broader or additional patient populations;
- our ability to adhere to ongoing compliance requirements of all health authorities to which we are subject, in the U.S. and abroad;
- our ability to obtain and maintain adequate reimbursement for our products;
- product quality, efficacy or safety concerns resulting in complaints, adverse events, product recalls or regulatory action;
- the risk that estimates regarding the number of patients with the diseases that our products and product candidates are designed to treat are inaccurate, do not predict, or are not reflective of actual numbers;

- our products not gaining acceptance among patients, providers and/or third party payors, including governmental agencies, for certain approved indications, due to cost or otherwise;
- our ability to maintain or increase sales of ELZONRIS;
- the adequacy of our pharmacovigilance and drug safety reporting processes;
- the successful development and implementation of sales and marketing campaigns;
- the loss of key management, scientific, or other personnel;
- the success and timing of any regulatory filings for ELZONRIS, or any of our other product candidates, including for approval in the U.S., Europe, and other regions for any indication(s);
- changes in regulations in the U.S., Europe and any other regions where ELZONRIS may be approved, marketed and sold;
- new products, new product candidates or new uses for existing products or technologies introduced or announced, by our competitors, and the timing of these introductions or announcements;
- our available cash and investments;
- the accuracy of our estimates regarding expenses, future income or revenue, capital requirements and needs for additional financing;
- our ability to obtain additional funding;
- our ability to obtain and maintain intellectual property protection for our products and product candidates;
- delays, interruptions, or failures in the manufacture, supply and distribution of our products and/or our product candidates;
- our ability to maintain the license agreements for our products and product candidates;
- the ability of our third-party manufacturers to manufacture and supply our products, and the performance of, and our reliance on, our third-party manufacturers and suppliers;
- the performance of our third-party vendors, including clinical research organizations, clinical trial sponsors, and clinical trial investigators;
- the success of our preclinical, non-clinical, and pre-investigational new drug, or pre-IND, efforts;
- our ability to gain access to products we may plan to use in combination studies; and
- our ability to form corporate partnerships, should that be an avenue we choose to pursue.

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

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

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

Part I

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

Item 1. Business

Overview

We are a commercial-stage biopharmaceutical company focused on discovering, acquiring, developing and commercializing oncology therapeutics. ELZONRIS® (tagraxofusp-erzs) was approved by the U.S. Food and Drug Administration, or FDA, and is commercially available in the U.S., for the treatment of adult and pediatric patients, two years and older, with blastic plasmacytoid dendritic cell neoplasm, or BPDCN. ELZONRIS is the first treatment approved for BPDCN and the first approved CD123-directed therapy. In addition, we are also assessing ELZONRIS, as both single agent and in combination, in a variety of other indications including Phase 1 and 2 clinical trials in chronic myelomonocytic leukemia, or CMML, myelofibrosis, or MF, multiple myeloma, or MM, and acute myeloid leukemia, or AML, with additional trials planned including a CD123⁺ all-comers trial. We are also advancing a pipeline of additional novel therapeutic candidates, including felezonexor (SL-801), an oral small molecule XPO-1 inhibitor currently in an ongoing Phase 1 trial of patients with advanced solid tumors, and SL-1001, an oral small molecule RET (rearranged during transfection) kinase inhibitor currently in investigational new drug, or IND, directed studies intended for patients with a variety of genetically-defined malignancies.

ELZONRIS regulatory and commercial

ELZONRIS is a targeted therapy directed to CD123. ELZONRIS was approved by the FDA in December 2018 for the treatment of BPDCN in adults and in pediatric patients, two years and older. ELZONRIS was granted Breakthrough Therapy Designation, or BT, for the treatment of BPDCN in August 2016. ELZONRIS was granted Orphan Drug Designation, or ODD, in the U.S. for BPDCN in June 2013 and granted ODD in Europe for BPDCN in November 2015. ELZONRIS is the first drug ever approved for this indication, the first CD123-targeted agent approved, and is commercially available to patients with BPDCN in the U.S.

In January 2019, Stemline submitted a Marketing Authorization Application, or MAA, to the European Medicines Agency, or EMA, seeking approval of ELZONRIS for the treatment of adult patients with BPDCN. The application is being reviewed on a standard timeline under the centralized procedure. In June 2019, we received the Day 120 List of Outstanding Issues, which include matters relating to clinical, non-clinical, quality, chemistry, manufacturing and controls, or CMC, and all stages of the clinical trial, including stage 4, which largely utilized a new lyophilized drug product formulation. We continue to interact with the EMA regarding the application. A scientific advisory group (SAG) meeting to discuss clinical data of tagraxofusp in patients with BPDCN was held on March 5th, 2020. Aspects of benefit-risk (i.e. efficacy and safety) of tagraxofusp as a therapy for BPDCN as well as other elements pertaining to the MAA procedure were discussed. Minutes of the meeting will be reflected in the European Public Assessment Report, or EPAR. We expect an opinion or further questions from the Committee for Medicinal Products for Human Use, or CHMP, in mid-2020. In anticipation of potential regulatory approval, we have assessed European staffing and infrastructure needs, and have been hiring personnel to meet the needs of a possible second half of 2020 commercial launch in Europe.

In addition, Stemline has instituted a global Early Access Program, or EAP, whereby physicians may seek access to Stemline’s investigational medicine outside of a clinical trial and/or before it is commercially available.

ELZONRIS additional clinical activities

We are seeking to broaden the commercial potential of ELZONRIS, globally, through clinical trials in additional indications including CMML, MF, and AML, as maintenance therapy in BPDCN patients post-transplant, as well as planned trials in other indications including a CD123⁺ all-comers trial.

Additional pipeline candidates

Our other pipeline candidates include: felezonexor (SL-801), a novel oral small molecule reversible inhibitor of XPO1, which is currently in a Phase 1 trial of patients with advanced solid tumors and recent data were presented at the 2019 European Society of Medical Oncology, or ESMO, annual conference, and SL-1001, an oral, selective small molecule RET kinase inhibitor that has demonstrated potent, selective, preclinical anti-cancer activity in RET-driven tumor models and is currently in IND-enabling studies. Additional candidates include: SL-901, an oral, small molecule kinase inhibitor, assessed in an abbreviated Phase 1 trial of patients with solid tumors in Europe, currently in Investigational Medicinal Product Dossier, or IMPD-enabling studies, and SL-701, an immunotherapeutic, which has completed a Phase 2 trial in patients with second-line glioblastoma.

Our Company

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

Our website address is www.stemline.com. The information set forth on our website is not a part of this report. We will make available free of charge through our website our annual reports on Form 10-K, quarterly reports on Form 10-Q and current reports on Form 8-K, and any amendments to these reports, as soon as reasonably practicable after we electronically file such material with, or furnish such material to, the Securities and Exchange Commission, or SEC. We are not including the information on our website as a part of, nor incorporating it by reference into, this report. The SEC maintains a website that contains annual, quarterly, and current reports, proxy statements, and other information that issuers (including us) file electronically with the SEC. The SEC's website address is <http://www.sec.gov/>.

Management

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

- Ivan Bergstein, M.D. — Chairman, Chief Executive Officer and President. Dr. Bergstein is Chief Executive Officer and Founder of Stemline Therapeutics. Dr. Bergstein has managed the company's evolution from early-stage research and development to the FDA approval and commercial launch of its lead product, ELZONRIS. Prior to founding Stemline, Dr. Bergstein was Medical Director of Access Oncology, Inc., a clinical stage oncology-focused biotechnology company where he was a key member of a small team responsible for the acquisition and development of the company's clinical stage assets and ultimately the sale of the company to Keryx Biopharmaceuticals, Inc. He received a BA in mathematics from the University of Pennsylvania, was elected to the Pi Mu Epsilon National Mathematics Honor Society, and was captain of the varsity wrestling team at Penn. He then received an MD from the Mount Sinai School of Medicine where he was elected to the Alpha Omega Alpha Honor Medical Society, received the Merck Award for Clinical Excellence, and subsequently completed an internship in general surgery. He then became the Jerome A. Urban Post-Doctoral Research Fellow at the Cornell University Medical College where he studied and published work relating to Wnt genes in human breast cancer. He then completed an internal medicine residency and hematology-oncology fellowship at the New York Presbyterian Hospital — Weill Medical College of Cornell University where he studied and published work on gene therapy manipulations of the sonic hedgehog pathway.
- Kenneth Hoberman — Chief Operating Officer. Mr. Hoberman has extensive financial, accounting, investor relations, corporate governance and business development experience including M&A, strategic alliances and partnerships both domestic and international. His operational expertise includes regulatory oversight, human resources, manufacturing and clinical development. He was previously Vice President of Corporate and Business Development of Keryx Biopharmaceuticals, Inc., where he was instrumental in the success of the company. He also helped secure multiple sources of capital including over \$200 million in equity investments through public and private offerings. He also initiated and executed a \$100 million strategic alliance and originated, negotiated and closed dozens of licensing and operational contracts, helping to grow the company's market capitalization to over \$1 billion. He also led the team that originated, in-licensed, and developed Auryxia™ which was approved by the FDA in September 2014. He is on the Board of Directors of TG Therapeutics, Inc. (Nasdaq: TGTX). He received a B.S.B.A. in Finance from Boston University and completed post-baccalaureate studies at Columbia University.

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

Robert Francomano — Chief Commercial Officer. Mr. Francomano joined Stemline Therapeutics in 2016 and is responsible for leading the transformation of the organization from a clinical-stage entity to one with a full commercial capability and infrastructure. Mr. Francomano brings more than 25 years of biopharmaceutical experience to Stemline, with the majority of time spent in the hematology/oncology therapeutic area. Prior to Stemline, Robert held several key roles of increasing responsibility within the oncology divisions of Baxalta, Pfizer, GlaxoSmithKline and AstraZeneca with workstreams that spanned the life cycle continuum from asset discovery through patent expiry. Mr. Francomano earned a bachelor’s degree in marketing/management from Siena College (NY) and an MBA from the University of Albany with a concentration in pharmaceutical/chemical studies.

Strategy

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

Commercialize ELZONRIS in the U.S. On December 21, 2018, the FDA granted approval of ELZONRIS for the treatment of BPDCN in adults and pediatric patients two years and older, in both treatment-naïve and previously-treated populations. ELZONRIS is the first treatment approved for BPDCN and the first approved CD123-targeted therapy. In an effort to optimize commercial success, we have invested and continue to invest in disease awareness and an infrastructure that includes sales professionals, marketing, medical affairs, and reimbursement specialists.

Assess ELZONRIS in additional indications. We are also assessing ELZONRIS as monotherapy in additional indications including in Phase 1/2 trials of patients with CMML and MF. ELZONRIS is also being assessed in Phase 1/2 trials of other indications including AML and multiple myeloma, in combination with other therapies. A CD123⁺ all-comers trial is also being planned.

Obtain approval and commercialize ELZONRIS in the E.U. Stemline submitted a MAA in January 2019 to the EMA seeking approval of ELZONRIS for the treatment of adult patients with BPDCN. The MAA was accepted and is currently under review by the EMA. The application is being reviewed on a standard timeline under the centralized procedure. A SAG in the EU met in March 2020. We are in the process of building out a commercial infrastructure, including the hiring of commercial personnel, in Europe to meet the needs of a possible European commercial launch in the second half of 2020 should ELZONRIS obtain approval in the E.U.

- *Assess felezonexor (SL-801) in a variety of cancer types.* We are assessing felezonexor (SL-801) in a Phase 1 trial of adult patients with advanced solid tumors. Patients are currently enrolling in this dose escalation study and we are assessing safety, including attempting to identify a recommended dose and administration schedule, as well as potential efficacy.
- *Develop SL-1001 in certain genetically-defined tumors.* We are assessing SL-1001, a novel RET kinase inhibitor, which is currently in IND-enabling studies with anticipation of entering clinical trials in 2021.

ELZONRIS

Overview

ELZONRIS is a novel targeted therapy directed to the interleukin-3 receptor- α , or CD123, a target present on a wide range of malignancies as well as some autoimmune disorders. ELZONRIS has been approved by the FDA for the treatment of BPDCN in adults and pediatric patients two years and older, in both treatment-naïve and previously-treated populations. ELZONRIS is the first treatment approved for BPDCN and the first approved CD123-targeted therapy. The ELZONRIS label contains a boxed warning for capillary leak syndrome, or CLS, which may be life-threatening or fatal. Physicians are advised to monitor for signs and symptoms of CLS and take actions as recommended in the full prescribing information.

BPDCN, formerly known as blastic NK-cell lymphoma, is an aggressive, orphan hematologic malignancy with historically poor outcomes. BPDCN may present with features similar to, and can be mistaken for, certain diseases including AML, non-Hodgkin's lymphoma, or NHL, acute lymphocytic leukemia, or ALL, myelodysplastic syndrome, or MDS, and CMML, and other malignancies including those with skin manifestations as well as certain or non-malignant cutaneous conditions. BPDCN typically presents in the bone marrow and/or skin, and may also involve lymph nodes and viscera. The diagnosis of BPDCN is based on the immunophenotypic diagnostic triad of CD123, CD4, and CD56, as well as other markers including TCL-1.

ELZONRIS is designed to specifically target CD123. CD123 is expressed on BPDCN and is a key marker, as a part of a triad of markers, that enables proper diagnosis of BPDCN. Additionally, CD123 represents a potential target for therapeutic research in a variety of cancers beyond BPDCN, as well as certain autoimmune disorders. Expression of CD123 has also been associated with poor prognosis and clinical outcomes in additional diseases including AML and CMML.

In April 2019, the pivotal trial results of ELZONRIS in patients with BPDCN were published in the April 25 issue of the *New England Journal of Medicine*.

In June 2019, ELZONRIS clinical data were presented at the 2019 American Society of Clinical Oncology, or ASCO, annual meeting in Chicago, Illinois and at the 24th Congress of the European Hematology Association, or EHA, in Amsterdam, Netherlands. Presentations included results from ongoing Phase 2 clinical trials of ELZONRIS monotherapy in relapsed/refractory CMML and relapsed/refractory MF. In October 2019, ELZONRIS clinical data from the pivotal trial in BPDCN were presented at the 81st Annual Meeting of the Japanese Society of Hematology, or JSH, in Tokyo, Japan. In November 2019, ELZONRIS preclinical results in systemic sclerosis, an autoimmune disorder in which CD123⁺ plasmacytoid dendritic cells, or pDCs, play a role in disease pathogenesis, were presented at the 2019 American College of Rheumatology, or ACR, annual meeting in Atlanta, Georgia. In December 2019, ELZONRIS clinical and preclinical data were presented at the 2019 American Society of Hematology, or ASH, annual meeting in Orlando, Florida. Presentations included results from ongoing Phase 2 clinical trials of ELZONRIS as monotherapy in patients with relapsed/refractory MF and in combination with pomalidomide and dexamethasone in patients with relapsed/refractory multiple myeloma. Preclinical data of ELZONRIS in combination with other agents in myeloproliferative neoplasms was also presented.

The FDA granted ELZONRIS BTM for the treatment of BPDCN in August 2016, and ODD for AML in February 2011 and ODD for BPDCN in June 2013. The EMA granted ELZONRIS ODD for AML in September 2015 and ODD for BPDCN in November 2015. The FDA granted the ELZONRIS Biologics License Application, or BLA, Priority Review and subsequently approved the BLA on December 21, 2018, two months ahead of its February 21, 2019, Prescription Drug User Fee Act, or PDUFA, date. In January 2019, Stemline submitted a MAA to the EMA, seeking approval of ELZONRIS for the treatment of adult patients with BPDCN. The application is being reviewed on a standard timeline under the centralized procedure. A SAG met during March 2020.

In addition to ELZONRIS, our pipeline of product candidates includes: felezonexor (SL-801, an XPO1 inhibitor), SL-1001 (a RET kinase inhibitor), SL-901 (a kinase inhibitor) and SL-701 (an immunotherapy).

ELZONRIS for blastic plasmacytoid dendritic cell neoplasm (BPDCN)

ELZONRIS is the first treatment approved for BPDCN and the first approved CD123-targeted therapy.

BPDCN is an aggressive hematologic cancer that carries a poor prognosis. BPDCN had been previously classified as blastic NK cell lymphoma, agranular CD4+/CD56+ hematodermic neoplasm, and plasmacytoid dendritic cell cancer. In 2008, this disease was renamed BPDCN by the World Health Organization, or WHO, due to the realization of its derivation from plasmacytoid dendritic cells, or pDCs, which are specialized immune cells.

BPDCN is an aggressive malignancy that most commonly affects middle-aged and older patients and is approximately three times more common in men than women. This malignancy typically presents with skin lesions, as well as bone marrow involvement. BPDCN growth in the bone marrow results in decreased blood cell counts, which can lead to serious infections, fatigue, bleeding, and death. BPDCN has had historically poor outcomes and was considered an area of unmet medical need prior to the approval of ELZONRIS.

ELZONRIS mechanism of action

ELZONRIS is a novel targeted therapy directed to CD123. ELZONRIS is comprised of human IL-3 recombinantly fused to a truncated diphtheria toxin, or DT, payload engineered such that IL-3 replaces the native DT receptor-binding domain. In this way, the IL-3 domain of ELZONRIS directs the cytotoxic DT payload to cells expressing CD123. Upon internalization, ELZONRIS irreversibly inhibits protein synthesis and induces apoptosis of the target cell.

CD123

CD123 is normally expressed on certain maturing hematopoietic cells, including maturing myeloid cells, B cells, plasmacytoid dendritic cells, or pDCs, mast cells, basophils and eosinophils, and appears to be involved in cell maturation, differentiation, and survival. CD123 expression appears to be lower on normal hematopoietic stem cells.

CD123 is expressed on multiple malignancies, to varying extents, including BPDCN, AML, certain myeloproliferative neoplasms, or MPNs, myelodysplastic syndrome, or MDS, CMML, B-cell acute lymphoid leukemia, or B-ALL, hairy cell leukemia, Hodgkin's and certain non-Hodgkin's lymphomas. In addition to expression on tumor bulk, CD123 expression has been reported on the cancer stem cells, or CSCs, of certain hematologic cancers including AML, CMML, MDS, and potentially T-cell ALL. In addition, elevated CD123 expression has been correlated with poor prognosis in certain hematologic cancers.

CD123⁺ plasmacytoid dendritic cells (pDCs)

CD123⁺ pDCs are the cell of origin for BPDCN. Notably, CD123⁺ pDCs have also been reported in the microenvironment of several tumor types including multiple myeloma, CMML and other MPNs including myelofibrosis, or MF, and mastocytosis, as well as AML and some solid tumors where they may play a tumor-promoting role. In several cases, including CMML and AML, tumor microenvironmental pDCs have been shown to be neoplastic and part of the malignant clone, indicating a potential need for therapeutic targeting. Accordingly, the potential to target neoplastic pDCs with ELZONRIS in a variety of tumor types, both hematologic and possibly solid, could provide significant label and market expansion opportunities.

CD123⁺ pDCs have also been implicated in the pathogenesis of certain autoimmune diseases, including scleroderma and cutaneous lupus, and is a potential therapeutic target for these conditions. We are conducting preclinical research in this area and preclinical data presented at the Annual European Congress of Rheumatology, or EULAR, demonstrated the cytotoxic activity of ELZONRIS against potentially pathogenic pDCs. This data suggests depleting pDCs or attenuation of pDC function could represent a potentially novel approach for the treatment of systemic sclerosis and certain other autoimmune disorders. In November 2019, ELZONRIS preclinical results in systemic sclerosis, an autoimmune disorder in which CD123⁺ pDCs play a role in disease pathogenesis, were presented at the 2019 American College of Rheumatology, or ACR, annual meeting in Atlanta, Georgia.

ELZONRIS regulatory and commercial

On December 21, 2018, the FDA granted full approval of ELZONRIS for the treatment of BPDCN in adult and pediatric patients two years and older, in both treatment-naïve and previously-treated populations. ELZONRIS is commercially available in the U.S.

Stemline is committed to helping patients with BPDCN in the U.S. access ELZONRIS through the Stemline ARC® program. Stemline ARC is a comprehensive access program designed to provide support, information and assistance to patients prescribed ELZONRIS. Dedicated oncology nurse advocates are available to provide personalized education about BPDCN and ELZONRIS to patients and their caregivers and to connect patients and their caregivers with helpful tools and resources. Stemline ARC offers a copay assistance program for patients with commercial insurance who qualify. Stemline is also partnering with patient advocacy groups to support the needs of other patients with BPDCN in the U.S.

In January 2019, Stemline submitted a MAA to the EMA seeking approval of ELZONRIS for the treatment of adult patients with BPDCN. The application is being reviewed on a standard timeline under the centralized procedure. A SAG in the EU met in March 2020. We are in the process of building out a commercial infrastructure in Europe should ELZONRIS obtain approval.

Stemline's commercial group is primarily focused on effectively launching and commercializing ELZONRIS in the U.S. The commercial group is comprised of a wide range of functions including commercial operations, sales, marketing, and market access and reimbursement. Additionally, the organization has established a medical affairs presence in the field which is predominantly staffed by medical science liaisons. We aim to build on this field presence based on routine reviews of the business and market. For 2020, we anticipate adding diagnostic and telephonic sales teams to further enhance product uptake. We believe the aforementioned functions represent the necessary infrastructure to support a successful launch of ELZONRIS in BPDCN in the U.S.

Over the past several years, the organization has been focused on preparing the market, the product, and the organization for the successful launch of ELZONRIS. In December 2017, we initiated our BPDCN disease awareness campaign at the American Society of Hematology, or ASH, annual meeting. One of the campaign's primary goals has been to educate multidisciplinary healthcare professionals, including hematologist-oncologists, dermatologists, pathologists, and allied healthcare professionals about, among other things, the appropriate testing for CD123 in order to bring the diagnosis of BPDCN to the forefront, while limiting misdiagnoses and underdiagnoses. The campaign highlighted the importance of CD123 as a key diagnostic marker for correct patient identification. Beyond BPDCN, CD123 may have additional utility in identifying other hematologic cancer patient subsets, including those with poor prognosis AML or CMML.

Access to ELZONRIS remains a top priority within our managed care group, with key success criteria identified as removing hurdles to product access and reimbursement. The Centers for Medicare & Medicaid Services, or CMS, granted approval for a New Technology Add-On Payment, or NTAP, for ELZONRIS for the treatment of BPDCN, as well as a new ELZONRIS-specific J-code. Both of these milestones went into effect on October 1, 2019. NTAP is intended to support Medicare beneficiaries' access to ELZONRIS by providing an incremental payment to hospitals for qualifying Medicare inpatient cases. A J-code is a permanent code assigned by CMS and used by Medicare, Medicaid, and commercial payers for billing and claims processing specific to ELZONRIS. We have also set up a formal patient support program and commercial co-payment assistance program known as the Stemline ARC program and have donated to an independent 501(c)(3) foundation for patients with BPDCN that require assistance.

ELZONRIS Clinical Trials in Additional Indications

We are also conducting additional clinical development activities with ELZONRIS including in Phase 1/2 clinical trials of patients with CMML, MF, and AML, and other trials are planned.

Chronic Myelomonocytic Leukemia (CMML)

At the 2019 EHA and ASCO annual conferences, we reported Phase 1/2 data from 23 patients with relapsed/refractory CMML who received ELZONRIS as a monotherapy in Stage 1 (lead-in, dose escalation stage) and Stage 2 (expansion stage). A new Stage 3a cohort is now open for enrollment.

In Stage 1, ELZONRIS at 12 mcg/kg/day for 3 days every 3-6 weeks was the highest tested dose for CMML, and a maximum tolerated dose, or MTD, was not reached. In Stage 2, patients are receiving ELZONRIS at 12 mcg/kg/day for three (3) days every 3-6 weeks.

Median age was 69 years (range: 42-80); 83% were male. 52% (12/23) of patients had baseline splenomegaly by physical examination (measured in centimeters that spleen was palpable below the left costal margin). The most common treatment-related adverse events, or TRAEs, were hypoalbuminemia (35%), thrombocytopenia (30%), vomiting and nausea (each 26%), and anaemia (22%). There were 3 cases of capillary leak syndrome; all three cases were grade 2. The most common TRAEs, grade 3+, were thrombocytopenia (30%), anaemia (17%), and nausea (4%).

ELZONRIS monotherapy demonstrated improvements in splenomegaly and bone marrow complete responses, or CRs, in patients with relapsed/refractory CMML. 100% (12/12) of evaluable patients with baseline splenomegaly, by physical examination, had a spleen response: 67% (8/12) of these patients had splenomegaly reductions by at least 50%, and 50% (4/8) of these patients with baseline splenomegaly of 5 cm or more below the left costal margin had splenomegaly reductions of at least 50%. In addition, three patients had bone marrow CRs including one patient who was bridged to stem cell transplant, or SCT.

Myelofibrosis (MF)

At the 2019 ASH annual conference in December 2019, the ELZONRIS Phase 1/2 data was the subject of an oral presentation by our principal investigators. The presentation included data from 29 patients with relapsed/refractory MF who received ELZONRIS in Stage 1 (lead-in, dose escalation stage) and Stage 2 (expansion stage). Stage 1 has completed enrollment, and Stage 2 has been expanded and is ongoing, with patient enrollment and follow up continuing. In Stage 1, ELZONRIS at 12 mcg/kg/day was the highest tested dose for MF, and an MTD was not reached. In Stage 2, patients are receiving ELZONRIS at 12 mcg/kg/day for three (3) days every 3-6 weeks.

Median age was 69 years (range: 54-87); 52% were female. 79% (23/29) of patients had baseline splenomegaly by physical examination (measured by spleen that is palpable >5 cm below costal margin). In Stage 1, no dose limiting toxicities, or DLT, were identified and a maximum tolerated dose, or MTD, was not reached. The most common TRAEs were alanine aminotransferase levels increased, headache and hypoalbuminaemia (each 17%).

The most common TRAEs, grade 3+, were anemia (14%), and thrombocytopenia (6%). There was also one case of CLS which was grade 3.

ELZONRIS monotherapy demonstrated improvements in splenomegaly in patients with relapsed/refractory MF. 53% (8/15) of evaluable patients with baseline spleen size >5 cm palpable by physical exam below the left costal margin experienced a reduction in splenomegaly: 20% (3/15) of patients had splenomegaly reduction by at least 35% measured by palpation during physical exam below left costal margin. 44% (7/16) of patients had splenomegaly reduction by at least 29% and 25% (4/16) had splenomegaly reductions by at least 45%. Additionally, 45% (9/20) of evaluable patients had symptom burden reduction, including 3 with symptom response per IWG-MRT 2013 MF response criteria.

In patients with MF, monocytosis ($>1 \times 10^9/L$ monocytes) has been associated with rapid disease progression and short survival, and may indicate an accelerated phase of the disease. In the trial, 100% (5/5) of evaluable patients treated with ELZONRIS and who had baseline monocytosis and spleen size ≥ 5 cm, had reduction in baseline splenomegaly, of which 40% (2/5) had reduction by $\geq 35\%$.

An additional ASH presentation demonstrated that ELZONRIS, either alone or in combination with ruxolitinib, had preclinical activity against primary MF patient samples, including those in accelerated phase and with high molecular risk profiles, which have limited therapeutic options beyond ruxolitinib and CD123 expression is evident. Based on these data, we are planning additional trials of ELZONRIS in MF, not only as a single agent but potentially in combination with other agents, including JAK inhibitors.

We continue to enroll patients with relapsed/refractory MF, and have expanded the trial to increased enrollment and add additional sites, in order to further elucidate the safety and efficacy profile of ELZONRIS in this patient population, including in patient subsets with monocytosis, thrombocytopenia and CD123 expression. By the end of 2020, we expect to provide updates on potential registration pathways in MF.

Multiple Myeloma (MM)

ELZONRIS, in combination with pomalidomide and dexamethasone, was assessed in a Phase 1/2 clinical trial in relapsed/refractory multiple myeloma.

At the 2019 ASH annual conference in December 2019, we presented preliminary Phase 1/2 data from 9 patients with relapsed/refractory multiple myeloma who received ELZONRIS in combination with pomalidomide and dexamethasone. The median age was 65 (range: 57-70); 56% were male. The median number of prior systemic therapies was 3 (range: 2-6), with all patients having previously received dexamethasone, bortezomib, and lenalidomide. The most common grade 1/2 TRAE's included hypoalbuminemia (67%), chills, insomnia, nausea, and pyrexia (each 56%), dizziness, and headache (each 44%). The most common grade 3+ TRAE's were thrombocytopenia (44%), neutropenia (33%), and hypophosphatemia (22%). There was one case of capillary leak syndrome which was grade 2.

Notably, five patients achieved partial responses (PRs) along with decreases in plasmacytoid dendritic cell (pDC) levels while on treatment. The presence of pDCs, which is the cell of origin of BPDCN, has been linked with myeloma growth and aggressiveness. These patients also experienced decreased levels of myeloma-related laboratory assessed values after 1 cycle of treatment with ELZONRIS combined with pomalidomide and dexamethasone.

Given the promising early results, and the strong scientific rationale, potential avenues for further development in this indication are currently under consideration. These include new patient populations, combination with daratumumab, and/or novel agents such as XPO1 inhibitors.

Acute Myeloid Leukemia (AML)

An investigator-sponsored trial of ELZONRIS in combination with azacitidine and venetoclax is currently being evaluated in the dose escalation stage of a Phase 1/2 trial of patients with relapsed/refractory AML, first-line AML who are unfit for chemotherapy, or high-risk myelodysplastic syndrome, or MDS. CD123 expression will be assessed. The trial is enrolling and data updates are expected later this year and on into next year.

Additional trials and potential indications for ELZONRIS

A Phase 1/2 trial is currently open for ELZONRIS as a maintenance therapy, post-stem cell transplantation, in patients with BPDCN. We expect to provide further program updates later this year.

Additional planned trials include ELZONRIS as monotherapy or in combination with other agents in a basket of CD123⁺ malignancies which may include acute lymphoid leukemia, or ALL, hairy cell leukemia, Hodgkin's disease, and certain other lymphomas.

In addition to BPDCN, CMML, MF and AML, additional potential indications for ELZONRIS include other hematologic cancers, solid tumors, and certain autoimmune disorders which are under evaluation for further development.

SL-801

Felezonexor (SL-801) is a structurally novel, oral, small molecule, reversible inhibitor of Exportin-1, or XPO1, a nuclear transport protein implicated in a variety of malignancies. XPO1 is a clinically validated target in oncology, and the FDA recently approved an XPO1 inhibitor in patients with relapsed/refractory multiple myeloma. Felezonexor has demonstrated preclinical in vitro and in vivo antitumor activity against a wide array of solid and hematologic cancers. Felezonexor's potential ability to reversibly bind XPO1 may offer the possibility to mitigate side effects and help optimize the therapeutic index. We are currently enrolling patients with advanced solid tumors in a Phase 1 dose escalation trial of single agent felezonexor. The dosing regimen for felezonexor was revised in an effort to improve tolerability while maintaining dose intensity, and dosing resumed at 70 mg/day with a new schedule (Schedule B).

In September 2019, we provided an update at the European Society of Medical Oncology, or ESMO, Congress 2019 in Barcelona, Spain. Results from 52 heavily pre-treated solid tumor patients (~90% were third line or greater) with a wide spectrum of solid tumors, including gastrointestinal, breast, lung, neuroendocrine, ovarian, and others were presented. Schedule A was amended to Schedule B in an effort to improve tolerability while maintaining dose intensity. In Schedule B (n=7), grade 3 weakness was experienced. As such, the 75mg cohort was expanded and is being evaluated. The most common TRAEs were nausea (69%), vomiting (53%), fatigue (44%), decreased appetite (24%), and diarrhea (22%), and the most common TRAE, grade 3 or higher, was nausea (9%).

A partial response, or PR, of duration 18+ weeks, was achieved with single agent felezonexor in a fourth line patient with KRAS-positive, microsatellite stable, or MSS, colorectal cancer. The PR (based on RECIST 1.1 criteria) was reported after two cycles of felezonexor (70mg then 65mg due to elevated creatinine), with the patient demonstrating serial reductions in the two target lesions (liver and spleen).

Response and treatment with felezonexor were ongoing at the time of presentation. Stable disease, or SD, was achieved in 12 patients, with 11/12 of these patients third line or greater. Five patients had SD for 4 and 11 months, including 1 patient with basal cell carcinoma with SD for ~11 months, and 20% disease shrinkage was noted in one patient with a heavily pre-treated neuroendocrine tumor.

We believe the ideal therapeutic dose and regimen have yet to be determined. Dose escalation is ongoing, and we intend to provide further updates as the Phase 1 clinical trial continues to enroll.

SL-1001

SL-1001 is an oral, selective small molecule RET (rearranged during transfection) kinase inhibitor. Genetic alterations in the RET kinase have been found in a diverse range of cancers. We believe RET kinase represents a clinically validated target in multiple oncology indications. In March 2019, we licensed this preclinical drug candidate from the CRT Pioneer Fund. The molecule was rationally designed by scientists at Cancer Research UK Manchester Institute (United Kingdom), and has demonstrated potent, selective, preclinical anti-cancer activity, both in vitro and in vivo, in RET-driven tumor models. IND-enabling studies are ongoing, and we expect to initiate clinical studies of SL-1001 in 2021.

SL-901

SL-901 is an oral, small molecule kinase inhibitor. In December 2017, we in-licensed this drug candidate from UCB Biopharma SPRL. Prior to in-licensing, the agent had demonstrated preclinical activity in several tumor types, and was evaluated in an abbreviated Phase 1 clinical trial in Europe. A PR was reported in one patient with advanced lung cancer. Neither a dose-limiting toxicity nor a maximum tolerated dose was reached in the trial and we believe further dose escalation is possible and warranted. In an effort to continue clinical dose escalation, we are currently conducting pre-IMPd-enabling work in anticipation of a regulatory re-filing.

SL-701

SL-701 is an immunotherapy designed to direct the immune system to attack targets present on brain cancer and other malignancies. SL-701 is comprised of several short synthetic peptides that correspond to epitopes of targets including IL-13R α 2, EphA2, and survivin; two of these synthetic peptides (IL-13R α 2 and survivin) are mutant and believed to enhance immune activity. We completed a Phase 2 trial of SL-701 in adult patients with second-line glioblastoma, or GBM. Phase 2 data preliminarily suggest SL-701 is generating target specific CD8+ T-cell responses in patients, which may be translating into improved clinical outcomes, including improved overall survival, or OS, in a subset of patients, which could form the basis of studies. SL-701 was awarded ODD from the FDA for the treatment of glioma in January 2015.

SL-501

SL-501 is a novel CD123-targeted therapy in preclinical development that has demonstrated potency, in vitro and in vivo, against several hematologic tumor types, including AML, CMML, Hodgkin's lymphoma, and NHL.

Patents and Proprietary Rights

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

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

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

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

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

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

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

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

From time to time, we may find it necessary or prudent to obtain licenses from third-party intellectual property holders. Where licenses are readily available at reasonable cost, such licenses are considered a normal cost of doing business. In other instances, however, where a third-party holds relevant intellectual property and is a direct competitor, a license might not be available on commercially reasonable terms or available at all. Accordingly, we attempt to manage the risk that such third-party intellectual property may pose by conducting, among other measures, freedom-to-operate studies to guide our early-stage research away from areas where we are likely to encounter obstacles in the form of third-party intellectual property. As our programs advance, we continue to monitor the intellectual property landscape in an effort to assess the advisability of licensing third-party intellectual property or taking other appropriate steps to address such freedom-to-operate or development issues in the manner we deem in the best interests of the Company.

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

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

Patents and Proprietary Rights Covering Stemline's Drug Candidates

We have an exclusive worldwide license to ELZONRIS. These patent rights include issued U.S. Patent 7,763,242; U.S. Patent 8,470,307; U.S. Patent 9,181,317; U.S. Patent 9,631,006; and U.S. Patent 10,259,853 covering methods of treating AML, BPDCN, MDS, and CMML that expire in 2027, as well as ten issued foreign patents. We have applied for an extension of the U.S. patent term pursuant to the Hatch Waxman act for issued patents covering ELZONRIS and if granted we will need to choose a single patent to extend. There are additional pending U.S. applications directed to methods of using ELZONRIS to treat other diseases that, if issued, would also expire in 2027 or 2039. In addition, we have filed foreign patent applications for the method of using ELZONRIS to treat various diseases, although there can be no assurances that such patents will be issued. In addition to patent protection, we also have the exclusivity afforded by the FDA's orphan designation of ELZONRIS for the treatment of BPDCN and by the provisions of the Biologics Price Competition and Innovation Act of 2009. See "Government Regulation — Orphan Drug Designation" and "— U.S. Patent Term Restoration and Marketing Exclusivity—Biologics Price Competition and Innovation Act of 2009."

We have exclusive and non-exclusive worldwide licenses to patents covering SL-801. These patent rights include issued U.S. Patents 8,084,454 and 8,415,357 covering composition of matter and uses of SL-801 that expire in 2030 and 2028, respectively, as well as eleven issued foreign patents that expire in 2028. We also have additional pending foreign patent applications directed to SL-801 which if issued, for which there can be no guarantee, would provide additional protection in certain non-U.S. territories and would be expected to expire in or around 2028.

We have an exclusive worldwide license to patents covering the SL-701 component, IL-13R α 2 mutant, a non-exclusive worldwide license to patents covering the SL-701 component, EphA2, and have filed U.S. and foreign patent applications covering the SL-701 component, survivin mutant. This intellectual property consists of an issued U.S. composition of matter patent (U.S. Patent 7,612,162) directed to an immunogenic mutant IL-13R α 2 peptide expiring in 2026, an issued U.S. composition of matter patent (U.S. Patent 8,574,584) directed to an immunogenic EphA2 peptide expiring in 2024, issued U.S. method of use patents (U.S. Patent 8,114,407, U.S. Patent 9,359,402, and U.S. Patent 10,131,699) directed to the use of EphA2 peptide expiring in 2024 and 2025, issued U.S. method of use patent (U.S. Patent 8,859,488) directed to the combined use of IL-13R α 2 mutant and EphA2 peptides expiring in 2026, an issued U.S. method of use patent (U.S. Patent 10,485,858) directed to the combined use of an IL-13R α 2 peptide, an EphA2 peptide, a survivin mutant peptide, and a Tetanus toxoid peptide formulated as an emulsion expiring in 2033, an issued European composition of matter patent (EP 2608799) directed to the combination of IL-13R α 2 mutant, EphA2, and survivin mutant peptides expiring in 2031, an issued U.S. method of use patent (U.S. 10,485,858) directed to the combined use of an IL-13R α 2 mutant, EphA2, and a new immunogenic survivin mutant peptide expiring in 2033, and additional pending U.S. and foreign patent applications directed to the use of an immunogenic mutant survivin peptide which, to the extent it issues, would be expected to expire in 2033. We also have additional pending patent applications directed to methods of using SL-701 components to treat certain diseases which, if issued (for which there can be no guarantee), would provide additional protection in the United States and certain non-U.S. territories and would expire in 2025, 2031, or 2033. In addition to patent protection, we also have the exclusivity afforded by the FDA's orphan designation of SL-701 for the treatment of glioma and by the provisions of the Biologics Price Competition and Innovation Act of 2009. See "Government Regulation — Orphan Drug Designation" and "— U.S. Patent Term Restoration and Marketing Exclusivity—Biologics Price Competition and Innovation Act of 2009".

We have an exclusive worldwide license to patents covering SL-901. This intellectual property consists of the patent family derived from WO 2009/001089, issued in the US, Europe and other territories (expiring in 2028); the patent family derived from WO 2008/001076, issued in the US and Europe (expiring in 2027); and the patent family derived from WO 2006/114606, issued in the US, Europe and other territories, expiring in 2026. We also have additional pending patent applications directed to SL-901 which, if issued (for which there can be no guarantee), would provide additional protection in the United States and certain non-U.S. territories and would expire in 2038.

We have an exclusive worldwide license to pending patents covering the SL-1001 component. This intellectual property consists of the patent family derived from PCT/GB2017/051076 (application date April 18, 2017, pending in the US, Europe and other territories, which if issued would expire in 2037); the patent family derived from PCT/GB2017/051077 (application date April 18, 2017, pending in the US, Europe and other territories); and the patent family derived from PCT/GB2018/050986 (application date April 13, 2018, pending in the U.S. Europe and other territories, which if issued would expire in 2038).

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

Patents and Proprietary Rights Covering CSC Focused Intellectual Property

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

- two pending U.S. patent applications filed in 2007 directed to CSC-directed therapies and regimens, including CSC-directed therapies and regimens for use in combination with companion diagnostics. Patent protection, to the extent it issues, would be expected to extend to 2027;
- a pending patent application that covers oligonucleotide-based oncology therapies, including CSC-targeted therapeutics, which target microRNA. Patent protection, to the extent it issues, would be expected to extend to 2022;
- a family of intellectual property covering methods to treat cancer through use of antibody-based compounds directed to IL-3R α as well as composition of matter covering IL-3R α -targeted antibody conjugates, including U.S. Patent 6,733,743; U.S. Patent 7,651,678; U.S. Patent 8,163,279; U.S. Patent 8,852,551; U.S. Patent 8,992,910; U.S. Patent 9,518,119; U.S. Patent 9,873,743; U.S. Patent 10,316,098; U.S. Patent 10,508,150; and other pending applications. Patent protection, to the extent it has or may issue, would be expected to extend to 2021 or 2028, as applicable; and
- an issued (U.S. Patent 10,538,742) and one pending U.S. patent covering CSC-focused drug discovery, including a novel high throughput screen to discover compounds that target CSCs, expiring in 2027.

License and Research Agreements

Scott and White Memorial Hospital

Research and License Agreement (ELZONRIS)

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

We must pay Scott and White royalties based on adjusted gross sales, by us or our sublicensees, of products containing the licensed compound for a period of ten years following the first commercial sale of each product in each country. Through December 31, 2019, we have expensed approximately \$0.6 million relating to royalty to Scott and White. The royalty rates for each product range from the low- to mid-single digits and are tiered based on our annual sales. We have sublicensing rights under the agreement, subject to our paying to Scott and White a percentage of the up-front payments we receive from a sublicensee.

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

The agreement survives until the later of the expiration of the last to expire licensed patent or the date on which we owe no further payments to Scott and White, after which our license becomes fully paid, irrevocable, perpetual, non-exclusive and royalty-free. We may terminate the license in whole or on a country-by-country and product-by-product basis upon prior written notice to Scott and White. If either we or Scott and White breach a material obligation under the agreement, and such obligation is not cured within a specified period of time following written notice from the other party, then the non-breaching party may terminate the agreement upon an additional written notice.

In addition, the agreement provides for Scott and White to conduct a research program with ELZONRIS. In March 2010, the agreement was amended to further the regulatory advancement of ELZONRIS. We have made certain payments to Scott and White for such research services pursuant to the agreement, which to date total approximately \$1.4 million in the aggregate. Additionally, we have been granted the exclusive right of reference to its IND for our own regulatory filings. We may assign the agreement to an affiliate of ours, a purchaser of all or substantially all of our assets or in connection with a merger, change in control or similar transaction by us.

CanBas, Ltd

License for SL-801

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

We are responsible to pay the remaining technical advisory fees over the next 1.5 years totaling 165 million Japanese Yen (JPY), if the clinical development continues over this time period. Additionally, we must pay CanBas tiered royalties based on aggregate net sales, by us or our sublicensees, of products containing the licensed compound until the latest date of a period of ten years following the first commercial sale of each product in each country; the date upon which there are no more valid claims or the expiration or termination of the last regulatory exclusivity period. The royalty rates start in the low single digits and are tiered up based on annual net sales. In the future, we may also be responsible, based on the achievement of specific clinical-development, regulatory and sales-based commercial milestones, for certain payments to CanBas of up to \$86 million. We have sublicensing rights under the agreement, subject to our paying to CanBas a standard royalty percentage of the payments we receive from a sublicensee.

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

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

University of Pittsburgh

Exclusive License Agreement to IL-13R α 2 peptide (SL-701 component)

In September 2009, we entered into an exclusive license agreement with the University of Pittsburgh, or the University, for the composition of matter, and use with other components, of a proprietary immunogenic mutant analog peptide of IL-13R α 2, an active ingredient of SL-701, our brain cancer immunotherapy candidate. Under the agreement, the University grants us an exclusive worldwide license under certain patent rights to make, have made, use, sell and import brain cancer peptide antigen immunotherapies (including SL-701, which has been developed by the University under a separate immunotherapy name designated by the University). The patent rights exclusively licensed to us under the agreement are described in more detail above under “Business — Patents and Proprietary Rights.” The University retains the right to practice the licensed patent rights for non-commercial education and research purposes. The license is also subject to certain retained rights of the United States government. Our right to grant sublicenses to third parties is subject to the prior written approval of the University, which the University may not unreasonably withhold or delay.

We paid the University an initial license fee and will pay the University annual license maintenance fees until the first commercial sale of a licensed product. To date, we have paid an aggregate of approximately \$0.8 million in fees to the University under the agreement. We must also pay the University a low-single digit royalty as a percentage of net sales of licensed products by us or our sublicensees, with standard provisions for royalty offsets to the extent we need to obtain any rights from third-parties to commercialize the licensed products. We must also pay a minimum annual royalty following the first commercial sale of a licensed product, but only to the extent the minimum annual royalty amount is greater than the annual royalty otherwise due. We also must pay the University a percentage of non-royalty revenue we receive from our sublicensees, which decreases if we enter into the applicable sublicense agreement after a certain clinical milestone has been met. We also must make certain payments to the University of up to approximately \$4.2 million upon the achievement of specific regulatory and commercial milestone events.

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

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

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

In March 2012, we entered into a non-exclusive license agreement with the University for the use of EphA2 epitopes, another active ingredient of SL-701. Under the agreement, the University grants us a non-exclusive worldwide license under certain patent rights to use the EphA2 peptide in or packaged with the IL-13R α 2 peptide, as well as other immunotherapies we may develop and own or exclusively control, for the diagnosis, treatment or prevention of diseases and tumors of the brain in human patients. The patent rights licensed to us under the agreement are described in more detail above under “Business — Patents and Proprietary Rights.” The University retains the right to practice the licensed patent rights for non-commercial education and research purposes. The license grant is also subject to certain retained rights of the United States government. We may only grant sublicenses to third parties who are permitted sublicensees under the exclusive IL-13R α 2 peptide license agreement with the University.

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

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

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

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

In March 2012, we entered into a non-exclusive license agreement with the University. Pursuant to the agreement, we acquired a non-exclusive, worldwide license to use and reference certain know-how, information and data that is contained in the INDs covering the clinical trials of SL-701 that were conducted by the University for the development, manufacture, regulatory approval and commercialization of pharmaceutical products. We may grant sublicenses in conjunction with a sublicense to a permitted sublicensee under the exclusive IL-13R α 2 peptide license agreement with the University.

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

The term of the license agreement is 20 years, and the University may terminate the agreement earlier (i) if we default in the performance of any of our obligations and do not cure the default within a specified time period, (ii) upon the termination of the exclusive IL-13R α 2 peptide license agreement with the University, or (iii) if we cease to carry out our business or become bankrupt or insolvent. We may terminate the agreement at any time prior to incorporating or referencing the data or University INDs, after a specified number of days following written notice. We may assign the agreement to an affiliate of ours, a purchaser of all or substantially all of our assets or in connection with a merger, change in control or similar transaction by us.

Cambridge University Technical Services Limited

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

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

Competition

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

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

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

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

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

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

Competition for ELZONRIS

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

Competition for SL-801

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

Competition for SL-701

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

Government Regulation

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

United States Drug Development Process

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

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

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

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

Before testing any compounds with potential therapeutic value in humans, the drug or biological candidate undergoes preclinical testing. Preclinical testing include laboratory evaluations of product chemistry, toxicity and formulation, as well as animal studies to assess the potential safety and activity of the drug or biological candidate. The conduct of the preclinical tests must comply with federal regulations and requirements including Good Laboratory Practices. The sponsor must submit the results of the preclinical tests, together with manufacturing information, analytical data, any available clinical data or published literature and a proposed clinical study design protocol, to the FDA as part of the IND. The IND automatically becomes effective 30 days after receipt by the FDA, unless the FDA places the clinical trial on a clinical hold within that 30-day time period. In such a case, the IND sponsor and the FDA must resolve any outstanding concerns before the clinical trial can begin. The FDA may also impose clinical holds on a drug or biological candidate at any time before or during clinical trials due to safety or compliance concerns. Accordingly, we cannot assure that submission of an IND will result in the FDA allowing clinical trials to begin, or that, once begun, issues will not arise that cause such trial to be delayed, suspended or terminated.

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

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

- *Phase 1.* The drug or biologic is initially introduced into small groups of healthy human subjects and tested for safety, dosage tolerance, absorption, metabolism, distribution and excretion. In the case of some products for severe or life-threatening diseases, especially when the product may be too inherently toxic to ethically administer to healthy volunteers, the initial human testing is often conducted in patients having the specific disease.
- *Phase 2.* The drug or biologic is evaluated in a larger, but still limited patient population to identify possible adverse effects and safety risks, to preliminarily evaluate the efficacy of the product for specific targeted diseases and to determine dosage tolerance, optimal dosage and dosing schedule for patients having the specific disease.
- *Phase 3.* Clinical trials are undertaken to further evaluate the selected dose, clinical efficacy and safety in an expanded patient population at geographically dispersed clinical trial sites. These pivotal clinical trials, which usually involve more subjects than earlier trials, are intended to establish the overall risk/benefit ratio of the product and provide adequate data which serve as the basis to inform product labeling. Generally, at least two adequate and well-controlled Phase 3 clinical trials are required by the FDA for approval of an NDA or BLA.

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

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

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

U.S. Review and Approval Processes

The results of product development, preclinical studies and clinical trials, along with descriptions of the manufacturing process, analytical tests conducted on the drug or biologic, proposed packaging and labeling and other relevant information are submitted to the FDA as part of an NDA or BLA requesting approval to market the product. The submission of an NDA or BLA is subject to the payment of substantial user fees; a waiver of such fees may be obtained under certain limited circumstances. We believe that we will be required to submit BLAs or NDAs for our product candidates.

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

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

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

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

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

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

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

Orphan Drug Designation

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

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

The FDA granted ODD to ELZONRIS for the treatment of AML, in February 2011 and for BPDCN in June 2013. The European Medicines Agency, or EMA, granted ODD to ELZONRIS for the treatment of AML in October 2015 and for BPDCN in November 2015. In addition, we received ODD for SL-701 for the treatment of glioma in January 2015. ELZONRIS was granted BTM by the FDA, for the treatment of patients with BPDCN in August 2016. On December 21, 2018, the FDA granted approval of ELZONRIS for the treatment of BPDCN in adult and pediatric patients two years and older, in both treatment-naïve and previously-treated populations.

Expedited Development and Review Programs

The FDA has established Expedited Review Programs to review applications for therapies that treat serious or life-threatening diseases or have the potential to provide unusually large benefits to patients more efficiently and expeditiously.

A product may be eligible for the BTM program, allowing expedited development and review by FDA. A BTM may apply to a drug that is intended, alone or in combination with one or more other drugs, to treat a serious or life-threatening disease or condition, and preliminary clinical evidence indicates that the drug may demonstrate a substantial improvement over existing therapies on one or more clinically significant endpoints, such as substantial treatment effects observed early in clinical development. The benefits of BTM include more intensive FDA guidance on an efficient drug development program, an organizational commitment involving senior FDA managers, and eligibility for rolling review and priority review of marketing applications. The receipt of a BTM for a product candidate may not result in a faster development process, review or approval compared to drugs considered for approval under conventional FDA procedures and a BTM may be rescinded by FDA where subsequent data no longer support the BTM of the candidate.

In August 2016, we announced that the FDA granted BTM to ELZONRIS for the treatment of BPDCN. On December 21, 2018, the FDA granted approval of ELZONRIS for the treatment of BPDCN in adult and pediatric patients two years and older, in both treatment-naïve and previously treated populations.

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

Any product submitted to the FDA for marketing approval, including those submitted to a Fast Track program, may also be eligible for other types of FDA programs intended to expedite development and review, such as priority review and accelerated approval.

Any product is eligible for Priority Review if it has the potential to provide safe and effective therapy where no satisfactory alternative therapy exists or a significant improvement in the treatment, diagnosis or prevention of a disease compared with marketed products. The FDA will attempt to direct additional resources to the evaluation of an application for a new drug or biological product designated for priority review in an effort to facilitate the review with the goal of taking Agency action on a marketing application within six (6) months.

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

Post-Approval Requirements

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

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

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

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

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

U.S. Patent Term Restoration and Marketing Exclusivity

Drug Price Competition and Patent Term Restoration Act of 1984

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

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

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

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

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

Biologics Price Competition and Innovation Act of 2009

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

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

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

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

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

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

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

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

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

Other U.S. Healthcare Laws and Compliance Requirements

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

Participation under the VHCA requires submission of pricing data and calculation of discounts and rebates pursuant to complex statutory formulas, as well as the entry into government procurement contracts governed by the Federal Acquisition Regulations.

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

Europe and Worldwide Government Regulation

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

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

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

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

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

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

Pharmaceutical Coverage, Pricing and Reimbursement

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

In 2003, the United States government enacted legislation providing a partial prescription drug benefit for Medicare recipients, which became effective at the beginning of 2006. Government payment for some of the costs of prescription drugs and biologics may increase demand for any products for which we receive marketing approval. However, to obtain payments under this program, we would be required to sell products to Medicare recipients through prescription drug plans operating pursuant to this legislation. These plans will likely negotiate discounted prices for our products. Further, to obtain coverage under Medicare Part B or Medicaid, we would be required to enter into a national rebate agreement with the Secretary of HHS to provide substantial rebates and discounts to state Medicaid agencies, certain government purchasers, and certain non-profit health care entities. Federal, state and local governments in the United States continue to consider legislation to limit the growth of healthcare costs, including the cost of prescription drugs and biologics. Future legislation could limit payments for pharmaceuticals such as the drug or biological candidates that we are developing.

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

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

Manufacturing

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

ELZONRIS Manufacturing and Supply

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

SL-801 Manufacturing and Supply

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

SL-701 Manufacturing and Supply

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

SL-901

SL-901 is a small molecule that is prepared via synthetic organic chemistry. Process development and active pharmaceutical ingredient, or API, CGMP manufacturing at an adequate scale to enable an IND, or foreign equivalent, submission and supply our planned clinical studies have been completed. We have also developed a stable capsule formulation that will be produced using CGMP manufacturing equipment at our third-party CMO. We believe that the manufacturing scale and product quality procedures and oversight ensure an adequate supply for our planned clinical studies.

SL-1001

SL-1001 is a small molecule that is prepared via synthetic organic chemistry. API process development and CGMP manufacturing is ongoing at a third-party CMO. Development of an oral capsule formulation is also ongoing at a different third-party CMO. We believe that the manufacturing scale and product quality procedures and oversight will ensure an adequate supply for our planned corporate sponsored clinical studies.

Sales and Marketing

Stemline's commercial group is primarily focused on effectively launching and commercializing ELZONRIS in the U.S. The commercial group is comprised of a wide range of functions including commercial operations, sales, marketing, and market access and reimbursement. Additionally, the organization has established a medical affairs presence in the field, which is predominantly staffed by medical science liaisons. We aim to build on this field presence based on routine reviews of the business and market. For 2020, we anticipate adding diagnostic and telephonic sales teams to further enhance product uptake. We believe the aforementioned functions represent the necessary infrastructure to support a successful launch of ELZONRIS indicated for BPDCN in the U.S.

The commercial infrastructure of specialty oncology products typically consists of a targeted, specialty sales force that calls on a highly targeted group of health care providers, or HCPs, supported by sales management, internal sales support, an internal marketing group, and distribution. Additional capabilities important to the oncology marketplace include the management of key accounts, such as managed care organizations, group-purchasing organizations, specialty distributors, oncology group networks, and government accounts. As ELZONRIS is approved for an ultra-orphan indication with a relatively small number of treating physicians. This corresponds with a reduced infrastructure need consisting of a small, targeted sales force. The current staffing is thought to be sufficient to support our current sales and marketing objectives but remains scalable given market needs. We have allocated management resources and made significant financial investments to support the commercial launch of ELZONRIS, including preparation of marketing and sales training materials developed in compliance with medical, legal and regulatory requirements. We may elect in the future to utilize strategic partners, distributors, or contract sales forces to assist in the commercialization of our products.

In January 2019, Stemline submitted a MAA to the EMA, seeking approval of ELZONRIS for the treatment of adult patients with BPDCN. The application is being reviewed under the centralized procedure on a standard review timeline. A SAG in the EU met in March 2020. In anticipation of potential regulatory success, we have assessed European staffing and infrastructure needs, and have hired commercial personnel to meet the needs of a possible second half of 2020 commercial launch in Europe.

Research and Development

Company sponsored research and development expenses totaled \$50.7 million in 2019, \$47.7 million in 2018, and \$50.2 million in 2017. “Research and development expenses” consist of costs associated with the development of our product candidates and our platform technology, which include: clinical trial costs, CMC-related costs, nonclinical costs, employee related expenses, external research and development expenses, license fees and milestone payments related to in-licensed products and technology, and facilities, depreciation and other allocated expenses. See “Item 7. Management’s Discussion and Analysis of Financial Condition and Results of Operations—Overview.”

Employees

As of March 16, 2020, we had 100 full-time employees, 7 of whom hold Ph.D. or M.D. degrees. None of our employees is subject to a collective bargaining agreement or represented by a trade or labor union. We believe that we have a good relationship with our employees.

Item 1.A Risk Factors.

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

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

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

To date, we have invested a significant portion of our efforts and financial resources in the acquisition and development of our clinical product candidates, which we plan to advance through clinical development. Our future success depends heavily on our ability to successfully manufacture, develop, obtain regulatory approval for, and commercialize these products and product candidates, which may never occur.

Before we generate any income from sales of our product candidates, clinical product candidates or future product candidates in the United States or elsewhere, we must complete preclinical and clinical development, conduct human subject research, submit clinical and manufacturing data to the U.S. Food and Drug Administration, or FDA, or foreign equivalent, qualify a third-party contract manufacturing organization, or CMO, satisfy the FDA or foreign equivalent that our CMO is capable of manufacturing the product in compliance with the FDA’s current good manufacturing practices (CGMPs), submit a marketing application (e.g., Biologics License Application, or BLA, or foreign equivalent), or New Drug Application, or NDA, or foreign equivalent, receive regulatory approval from the FDA or a foreign regulatory authority, build a commercial organization, make substantial investments, and undertake significant marketing efforts ourselves or in partnership with others to ensure compliant marketing and market acceptance of any products we commercialize. We are not permitted to engage in unrestricted marketing or promotion of 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 our current or future product candidates.

We cannot be certain that any further BLAs, NDAs or MAAs will be filed within a specified period of time, or that any BLA or NDA or similar foreign marketing application will allow us to obtain or maintain marketing approval. In addition, any marketing approval we may obtain may be for indications and uses that are more limited than we expect or include contraindications or risk measures that limit market acceptance of the product subject to the marketing approval. We also cannot be certain that our product or product candidates will be successful in clinical trials or that the clinical trials or data will support filing any further BLAs or NDAs in the U.S., or similar foreign marketing applications elsewhere. We also cannot be certain that any of our product candidates will receive the appropriate regulatory approval required to commence clinical trials. Further, the FDA, an independent review committee, or IRC, or an oncologic drugs advisory committee, or ODAC, may not agree with the interpretation by our investigators or us of the clinical safety and efficacy of our product candidates, and our product candidates may not receive regulatory approval.

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

If the incidence and/or prevalence of diseases, or disease areas, we are targeting for development and/or commercialization, and future growth, are low, including lower than our estimates or estimates of third-parties, this could significantly delay patient enrollment in our additional ongoing or future clinical trials and/or could negatively impact commercial revenue. The true incidence and/or prevalence, as well as market potential, can be difficult to determine, ahead of commercial launch, for certain rare diseases, such as blastic plasmacytoid dendritic cell neoplasm, or BPDCN, where there had been limited epidemiologic and published data, and databases, and where we are the first company to receive approval for this indication, with no prior product revenue data. Additionally, due to the unfamiliarity and/or rarity of certain diseases, such as BPDCN, health care providers may not be aware of, and/or may misdiagnose or underdiagnose such diseases, leading to low trial enrollment and/or product revenue. Our disease awareness campaign, intended to raise awareness of the disease and increase patient identification, could fail to do so for many reasons. At this time, we have no way of assuring the accuracy of any incidence/prevalence or revenue numbers, or the chances for successful development in related areas, thus if these are low, despite expectations to the contrary, this will negatively impact our revenue and future prospects for the company.

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

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

Clinical trials can be delayed, suspended or terminated for many reasons, including but not limited to:

- delays or failures in reaching an agreement on acceptable terms with prospective CMOs, CROs, and clinical trial sites, the terms of which can be subject to extensive negotiation and may vary significantly depending upon the circumstances;
- failure of our third-party contractors, including CROs and CMOs, or our investigators, to comply with regulatory requirements or otherwise meet contractual obligations in a timely manner;

- delays or failure in obtaining the necessary approvals from regulators, institutional review boards, or IRBs, or scientific review committees, or SRCs, in order to commence or continue a clinical trial;
- our inability to manufacture, or obtain from third-parties, adequate supply of drug substance, drug product or adjuvant therapies sufficient to complete our preclinical studies and clinical trials;
- risk of loss of drug product, adjuvants and/or other components of the product, due to third-party storage and distribution of such supplies;
- the FDA, or other regulatory authority, issuing a clinical hold or requiring alterations to any of our study designs, including extending a study or requiring new studies, to our overall strategy or to our manufacturing plans;
- delays in patient enrollment, variability in the number and types of patients available for clinical trials, poor accrual, or high drop-out rates of patients in our clinical trials;
- clinical trial sites deviating from trial protocols or dropping out of a trial and our inability to add new clinical trial sites;
- difficulty in maintaining contact with patients after treatment, resulting in incomplete data;
- poor effectiveness of our product candidates during clinical trials;
- safety issues, including serious adverse events associated with our product candidates and patient exposure to unacceptable health risks;
- reports of adverse events or other safety concerns involving ELZONRIS and our clinical drug candidates;
- receipt by a competitor of marketing approval for a product targeting an indication that one of our product candidates targets;
- governmental or regulatory delays and changes in regulatory leadership, requirements, policy and guidelines; or
- the FDA, or similar regulatory body, may not agree with the endpoints we select or the interpretation of the results related to the endpoints in the evaluation of our product candidates, thereby refusing to approve our product candidates for marketing approval, or withdrawing approval.

We could also encounter delays if a clinical trial is suspended or terminated by us, by the IRB where such trial is being conducted, by a Data Safety Monitoring Board, or DSMB, if one is utilized for any such trial, or by the FDA or other regulatory authorities. Such authorities may suspend or terminate a clinical trial due to a number of factors, including, among other things, failure to conduct the clinical trial in accordance with regulatory requirements or our clinical protocols, regulatory violations identified during an inspection of the clinical trial operations or trial site, imposition of a clinical hold by the FDA or other regulatory authorities, study subject safety concerns, adverse events or severe adverse events, including deaths, failure to demonstrate a benefit from using a drug, changes in governmental regulations or administrative actions, or lack of adequate funding to continue the clinical trial.

There are unknown risks for our clinical product candidates, including with respect to dosing, administration, pharmacokinetics, bioavailability, safety and efficacy, that we expect we will learn about during clinical development, which could halt or delay this development program and/or alter our current strategy for the development of these product candidates.

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

In any clinical trial of a product candidate, the results of such trial may not be adequate to support submission of a marketing application or marketing approval. Because our product candidates are intended for use in life-threatening diseases, in many cases we ultimately intend to seek marketing approval for each product candidate based on the results of a single clinical trial, which may be open-label and single-group in nature. As a result, these trials may receive enhanced scrutiny from the FDA. For any such trial, if the FDA disagrees with our choice or definition of primary endpoint, or the results for the primary endpoint are not robust or significant or clinically beneficial enough, including relative to a control or historical data, the FDA may refuse to approve a BLA or NDA based on such intended pivotal trial, despite meeting a primary endpoint of the study. In addition, the results of any such intended pivotal trial may be subject to confounding factors, or may not be adequately supported by other study endpoints, possibly including overall survival, or OS, overall response rate, or ORR, rate of complete response, or CR, rate of clinical complete response, or cCR, rate of partial response, or PR, rate and definition of bone marrow complete response with partial or incomplete hematologic recovery, rate and definition of other responses including spleen response, including measurement modality and timing of events, rate and definition of total symptom score response, and/or response duration including definition of response duration, in which case the FDA may refuse to approve a BLA or NDA based on such intended pivotal trial, despite meeting a primary endpoint of the study. The FDA may not accept the design of or our future Stage 3a results of ELZONRIS in CMML, or our conclusions related to our design of or future Stage 3a results, as a basis to design and implement the pivotal Stage 3b portion of the program. The FDA may also require the completion of additional clinical trials before or as a condition for approving our product candidates.

If we experience delays in the completion of, or a termination of, any clinical trial of our product candidates, the commercial prospects of our product candidates will be harmed, which will have a negative impact on our ability to commence product sales and generate product income from any of our product candidates. In addition, any delays in completing our clinical trials will increase our costs and slow down our product candidate development and approval process, and may negatively impact our ability to raise additional capital to support these increased costs. Delays in completing our clinical trials could also allow our competitors to obtain marketing approval before we do, or could 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 or subsequent clinical trials and results in the commercial setting may be different than clinical trial experience.

The results of preclinical studies and clinical trials, including early stage, late stage, and investigator-sponsored or corporate-sponsored clinical trials of any investigational or approved products, may not be predictive of the results of subsequent and/or later stage clinical trials such as Stage 3a or Stage 3b versus Stage 1 and 2 of the ELZONRIS Phase 1/2 trial in CMML or Stage 4 versus Stages 1, 2, and 3 of the ELZONRIS Phase 1/2 trial in BPDCN. Investigational or approved products in later stage, including subsequent stages of a given trial, or larger clinical trials may fail to show the same safety and efficacy results demonstrated in earlier studies, including having differing patients populations and/or endpoints and methods and schedules to assess such endpoints, 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, including canceling clinical development programs, due to adverse safety profiles or lack of efficacy observed in the commercial setting, notwithstanding promising results in earlier studies. Similarly, our clinical trials results, including with inclusion of subsequent stages of any given trial or experience with products in the commercial setting, may not be successful, including from a regulatory standpoint, for these or other reasons.

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

- As we optimize and scale-up production of our clinical product candidates, there may be manufacturing, formulation, fill-finish and other process and analytical changes that are part of the optimization and scale-up necessary for producing drug substance and drug product of a quality, quantity and stability sufficient for later stage clinical development and commercialization. Delays, including failures, in any of these steps, may delay initiation and completion of clinical trials, regulatory submissions, or commercial launch. We may also need to demonstrate comparability between newly manufactured drug substances and/or drug products relative to previously manufactured drug substances and/or drug products. Demonstrating comparability may cause us to incur additional costs or delay initiation or completion of our clinical trials, including the need or choice to initiate a dose escalation study, and, if unsuccessful, could require us to complete additional preclinical or clinical studies of our product candidates. Failure to demonstrate comparability could also result in delays in regulatory submissions or commercial launch. We are also developing a new lyophilized formulation of ELZONRIS. In the event that this formulation does not demonstrate comparability with the current liquid/frozen formulation, including from a pharmacokinetic, safety and/or efficacy perspective, the commercial success of ELZONRIS could be negatively impacted.
- We are, or may in the future be, treating patients with certain diseases or conditions that have not been previously treated with our product candidates. In these instances, we may choose to treat patients at several different doses and use multi-cycle dosing regimens to determine the optimal doses and schedules for both near-term and long-term safety and disease control in each indication. Use of our product candidates in new disease populations and at new dosing regimens could produce unforeseen adverse reactions and events that could impact the development and ability to obtain or maintain marketing approval for our product candidates.
- We may determine, based on safety and efficacy, that certain doses and regimens of our product candidates for particular indications are optimal for initial near-term therapy whereas the same, or other, doses and regimens are optimal for longer-term maintenance therapy.
- We are developing SL-701 as an injection administered under the skin, or subcutaneously, in our trials. Two previous investigator-sponsored trials of an earlier version of SL-701 used this method of delivery. Another previous investigator-sponsored trial of an earlier version of SL-701 used a different method of delivery, in which dendritic cells, which are a type of immune cell, were removed from the patient, exposed to immunogenic peptides, and then re-injected into or near a lymph node of the patient (intra/peri-nodally). Our plan continues with the subcutaneous injection method used in two of the previous studies and represents a change from one of the other previous studies.
- We manufactured and formulated SL-701 as a mixture of IL-13R2 mutant peptide, EphA2 peptide, a new survivin mutant peptide, and a tetanus toxoid peptide. An earlier version of this immunotherapy, which included IL-13R2 mutant and EphA2 peptides, was mixed with additional peptides in previous studies, including a different survivin peptide in some studies.

- In the initial stage of our SL-701 corporate-sponsored trial, we used granulocyte-macrophage-colony-stimulating factor, or GM-CSF, and imiquimod as the immunostimulants. In the second stage of our SL-701 trial, we used poly-ICLC as the immunostimulant, which was the immunostimulant used, along with an earlier version of SL-701, in the previous investigator-sponsored study but is not currently commercially available. If the poly-ICLC regimen is found to be superior, it would require successful approval and commercialization of poly-ICLC in addition to SL-701 to support product launch, which would entail a more complicated regulatory and commercialization strategy than required for a single product launch.
- In clinical trials, we have or are currently combining ELZONRIS with pomalidomide and dexamethasone in myeloma, ELZONRIS with a hypomethylating agent and potentially a Bcl-2 inhibitor and potentially certain chemotherapeutic agents in various indications including AML and BPDCN.
- In some of our current or future trials, we are, or may, combine our product candidates with each other or with other therapies such as chemotherapy, radiation, targeted therapy, or anti-angiogenic therapy which could result in unforeseen toxicities.

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

Adverse events or other safety concerns involving ELZONRIS or our clinical drug candidates could delay clinical development, delay or prevent us from obtaining or maintaining regulatory approvals, or negatively impact sales or the commercial prospects for our product candidates.

Adverse events or other safety concerns involving ELZONRIS or our clinical drug candidates in the development or commercial setting could interrupt, delay or halt our clinical trials and/or commercial sales of our products. For example, CLS is a known, sometimes fatal, and well-documented side effect of ELZONRIS. Reports of CLS cases, or other adverse events or other safety concerns involving ELZONRIS or our product candidates, could result in clinical trial delays including regulatory authorities placing trials on clinical hold or denying or withdrawing approval for trials of any or all indications, or adversely affect our ability to maintain or increase commercial sales of our product or products. Further, patients receiving ELZONRIS or our product candidates with co-morbid diseases and/or indications not previously well-studied, and/or in combination with other agents, may experience new or different serious adverse events in the future. Likewise, reports of adverse events or other safety concerns involving ELZONRIS or our product candidates could interrupt, delay or halt ongoing or planned clinical trials of such product candidates, could require redesign of study protocols and conduct of additional trials, could result in our inability to file for or obtain regulatory approvals for any of our product candidates, or could negatively impact commercial prospects for our product or product candidates.

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

We may not be able to continue clinical trials for our product candidates if we are unable to enroll a sufficient number of eligible patients to participate in these trials, including as required by the FDA or other regulatory authorities. Patient enrollment, a significant factor in the timing of clinical trials, is affected by many factors including the size and nature of the patient population, the proximity of patients to clinical sites, the eligibility criteria for the trial, the design of the clinical trial, competing clinical trials and clinicians' and patients' perceptions as to the potential advantages of the drug being studied in relation to other available therapies, including any new drugs that may be approved or may commence competing clinical trials for the indications we are investigating.

Some of our product candidates are being developed in rare indications with small available study populations. There are very limited independently reported data on annual incidences of these rare diseases. If the prevalence of these diseases is very low, including lower than our estimates or estimates of our third-party contractors, this could significantly delay patient enrollment in any one or more of our ongoing or future clinical trials.

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

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

The time required to obtain approval by the FDA and comparable foreign authorities can be unpredictable and depends upon numerous factors, including the substantial discretionary review afforded to the regulatory authorities, which could include the prerequisite of an advisory panel, e.g. ODAC review. In addition, regulations, policies or guidance documents, or the type and amount of preclinical, CMC, clinical pharmacology, and clinical data necessary to gain approval may change during the course of a product candidate's clinical development and may vary among jurisdictions. We may be required to undertake and complete certain additional preclinical, CMC, clinical pharmacology, bioanalytical, immunogenicity, or clinical studies to generate additional data required to support the submission of an IND, a BLA, or an NDA to the FDA or equivalent applications to comparable foreign authorities. An inadequacy in any of these areas, or a lack of personnel, financial resources or performance, including by third parties, could result in a delayed or unsuccessful regulatory filing. The FDA, or other non-U.S. regulatory authority, may provide feedback or make requests that are difficult to implement or not implementable at all, and/or our understanding of regulatory feedback, including regulatory authority minutes, may be incorrect. Also, the FDA, or any other non-U.S. regulatory authority, may require additional studies to support regulatory approval, including either full or accelerated or conditional, which could result in a delay in our clinical programs and/or a delayed or unsuccessful regulatory filing, or no filing at all.

To date, we have only obtained FDA regulatory approval for one drug product, ELZONRIS, and it is possible that none of our other existing product candidates, additional indications for ELZONRIS (including, but not limited to, CMML), 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 MAA is currently under review by the European Medicines Agency, or EMA, under a standard timeline. In June 2019, we received the Day 120 List of Questions which include matters relating to clinical, non-clinical, quality, and CMC, and all stages of the clinical trials, including stage 4, which largely utilized a new lyophilized drug product. We continue to interact with the EMA regarding the application and a SAG meeting was recently held during March 2020. While we remain confident in our ability to successfully address these matters, we are still early in the process of evaluating our data and options and we acknowledge that there is no guarantee we will be successful attaining full approval for previously-untreated and previously-treated BPDCN, full approval for one or the other indications, conditional approval for both indications, conditional approval for one or the other indications, or approval at all.

Our product candidates, alone or in combination with any adjuvant, immunostimulant including GM-CSF or imiquimod or poly-ICLC, or other agents with which we may combine our drug candidates, could fail to receive regulatory approval for many reasons, including, but not limited to:

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

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

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

Although we expect to focus a substantial amount of our efforts on the continued clinical testing and regulatory interactions for our clinical stage drug candidates, another key element of our strategy is to identify and test additional compounds. A portion of the preclinical research that we are conducting involves new and unproven drug discovery methods, as well as the preclinical testing of new compounds and potential new uses of existing compounds. The drug discovery that we are conducting using our StemScreen® platform technology may not be successful in identifying compounds that are useful in treating humans with cancer. Research programs designed to identify product candidates require substantial technical, financial and human resources, whether or not any product candidates suitable for approval and commercial marketing are ultimately identified. Even if our research programs may initially show promise in identifying potential product candidates, they may 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 is subject to the ongoing collection of safety and efficacy data and may, on further study, be shown to have harmful side effects, be prone to serious adverse events, fail to continue to exhibit that characteristics that support the initial findings of safety and efficacy or to otherwise fail to 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 additional compounds for preclinical and clinical development, we may not have sufficient or any product income, which could result in significant harm to our financial position and adversely impact our stock price.

If we obtain approval to market any products outside of the U.S., a variety of risks associated with international operations and expansion could materially adversely affect our business.

If ELZONRIS is approved for marketing outside of the U.S., which may not occur, we may enter into agreements with third parties to market ELZONRIS in certain jurisdictions. We have no prior experience in these countries, and many biopharmaceutical companies have found the process of marketing their products in foreign countries to be very challenging. We expect that we will be subject to additional risks related to international operations or entering into international business relationships, including:

- different regulatory requirements for drug development and approvals and rules governing drug commercialization in foreign countries, including postmarket surveillance, monitoring and reporting;
- possible failure by us or our distributors to obtain appropriate licenses or regulatory approvals for the sale or use of our product candidates, if approved, in various countries;
- reduced or no protection over intellectual property rights;
- unexpected changes in tariffs, export and import restrictions, trade barriers, and regulatory requirements;
- economic weakness, including inflation, or political instability in particular foreign economies and markets;
- complexities associated with managing multiple payor-reimbursement regimes or self-pay systems;
- financial risks, such as longer payment cycles, difficulty enforcing contracts and collecting accounts receivable;
- compliance with tax, employment, immigration and labor laws for employees living or traveling abroad;
- foreign reimbursement, pricing, and insurance regimes;
- foreign taxes;
- foreign currency fluctuations, which could result in increased operating expenses and reduced revenue, and other obligations incident to doing business in another country;
- workforce uncertainty in countries where labor unrest is more common than in the U.S.;
- difficulties in managing foreign operations;
- compliance with the U.S. Foreign Corrupt Practices Act, the United Kingdom Bribery Act 2010, or similar antibribery and anticorruption laws in other jurisdictions as well as various regulations pertaining to data privacy, such as the European Union General Data Privacy Regulation;
- production shortages resulting from any events affecting raw material supply or manufacturing capabilities abroad; and
- business interruptions resulting from geopolitical actions, including war and terrorism, or natural disasters including earthquakes, typhoons, floods, and fires.

We are subject to ongoing FDA regulatory requirements related to ELZONRIS and our product candidates, both before and after regulatory approval, which require significant resources. Additionally, our product and product candidates, if approved, could be subject to labeling and other restrictions and we may be subject to regulatory and enforcement actions or penalties if we fail to comply with regulatory requirements or experience unanticipated problems with our product.

Any additional regulatory approvals that we or our potential strategic partners may receive for our product candidates may also be subject to limitations on the approved indication for use for which the product may be marketed or to the conditions of approval, may contain product contraindications, warnings, or precautions that limit the use of our product candidates or may contain requirements for potentially costly post-marketing testing, including Phase 4 clinical trials, and surveillance to monitor the safety and efficacy of our product candidates. In addition, with regard to ELZONRIS or any product candidates, should they become approved by the FDA, the manufacturing processes, testing, packaging, labeling, storage, distribution, post-market reporting, advertising, promotion and recordkeeping for the product will be subject to extensive and ongoing regulatory compliance requirements. These requirements include submissions of safety and other post-marketing information and reports, as well as continued compliance with CGMPs for commercial manufacturing and good clinical practices, or GCPs, for any clinical trials that we conduct post-approval. For example, we have several post-marketing commitments related to the FDA approval of ELZONRIS. In addition, there are now and may be in the future manufacturing, formulation, fill-finish and other process and analytical changes required by the FDA related to producing drug substance and drug product of a quality, quantity and stability sufficient for commercial supply. Changes, delays or failures in any of these steps may negatively affect disposition of manufactured batches of drug substance and/or drug product, and as a result, may require production of additional batches of drug substance and/or drug product. Issues that may arise 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 product recalls;
- warning letters, untitled letters, or holds on clinical trials;
- refusal by the FDA to approve pending applications or supplements to approved applications filed by us or our strategic partners, or suspension or revocation of product license approvals;
- product seizure or detention, or refusal to permit the import and export of products;
- investigations or inspections by government entities, including, but not limited to, FDA or foreign health authorities; and
- injunctions, fines, consent decrees, corporate integrity agreements, or the imposition of other civil penalties or criminal penalties.

We cannot predict the likelihood, nature or extent of government regulation that may arise from future legislation or administrative action, either in the United States or abroad. If we are slow or are 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 Commercialization of ELZONRIS and the Development and Commercialization of Our Product Candidates

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

We continue to develop our infrastructure to commercialize ELZONRIS, and potentially our product candidates, if any are approved. We may potentially enter into contract research, contract sales, licensing or collaboration agreements to assist in the future development and commercialization of such product candidates.

To develop internal sales, distribution and marketing capabilities for our product candidates that might be approved, we would have to invest significant amounts of financial and management resources, some of which would be committed prior to knowing that our clinical drug candidates were approved. For ELZONRIS, as well as for our product candidates for which we decide to perform sales, marketing, and distribution functions ourselves, we face a number of additional risks, including:

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

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

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

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

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

In addition, we must be able to successfully identify patient populations with sufficient numbers in order to successfully commercialize our products. There can be no guarantee that any of our programs will be effective at identifying target patient populations, and the number of patients in the markets for which we may receive marketing approval (e.g., in the United States, Europe and elsewhere) may turn out to be lower than expected, or patients may not be otherwise amenable to treatment with our products, all of which would adversely affect the results of our operations and our business.

If ELZONRIS, or any product candidates for which we were to receive approval, failed to achieve market acceptance, we would not be able to generate significant income. In addition, there are no guarantees that any approved product will be effective, or gain market acceptance, if we were to obtain approval for additional indications.

Our product or product candidates may become subject to unfavorable pricing regulations, third-party reimbursement practices or healthcare reform initiatives, which would harm our business.

The regulations that govern marketing approvals, pricing and reimbursement for new drug products vary widely from country to country and are subject to changes in interpretation, application and new legislative proposals at any time. Some countries require the 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 income 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 the capital needed to commercialize products and our overall financial condition.

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

Our business may be affected by the efforts of government and third-party payors to contain or reduce the cost of healthcare through various means. For example, the Patient Protection and Affordable Care Act and the Health Care and Education Affordability Reconciliation Act of 2010 (collectively, the ACA), enacted in March 2010, substantially changed the way healthcare is financed by both governmental and private insurers, and significantly impacted the pharmaceutical industry. With regard to pharmaceutical products, among other things, the ACA expanded and increased industry rebates for drugs covered under federal health care programs. Legislative proposals such as expanding the Medicaid drug rebate program to the Medicare Part D program, providing authority for the government to negotiate drug prices under the Medicare Part D program and lowering reimbursement for drugs covered under the Medicare Part B program have been raised in Congress but have not been enacted so far. In the 116th U.S. Congress, there has been a renewed and bipartisan effort to address the cost of prescription drugs, including legislation intended to increase competition by speeding the approval of generic drugs and their entry to the marketplace, international reference pricing and direct government drug price negotiation, and increasing transparency on patents and price increases. The administration can rely on its existing statutory authority to make policy changes that could have an impact on the drug industry. For example, the Medicare program has in the past proposed to test alternative payment methodologies for drugs covered under the Part B program and finalized a proposal to pay hospitals less for Part B-covered drugs purchased through the 340B Drug Pricing Program effective January 1, 2018. At the end of 2019, the HHS released a draft rule related to importation of prescription drugs. It is unclear how this might impact the pharmaceutical market, but signals a continued effort by the administration to lower drug costs through regulatory action.

Modifications to or repeal of all or certain provisions of the ACA have been attempted in Congress as a result of the outcome of the recent presidential and congressional elections, consistent with statements made by the incoming administration and members of Congress during the presidential and congressional campaigns and following the election. In January 2017, Congress voted to adopt a budget resolution for the fiscal year of 2017, or the Budget Resolution, that authorizes the implementation of legislation that would repeal portions of the ACA.

The Budget Resolution is not a law. However, it is widely viewed as the first step toward the passage of legislation that would repeal certain aspects of the ACA. Further, on January 20, 2017, President Trump signed an Executive Order directing federal agencies with authorities and responsibilities under the ACA to waive, defer, grant exemptions from, or delay the implementation of any provision of the ACA that would impose a fiscal or regulatory burden on states, individuals, healthcare providers, health insurers, or manufacturers of pharmaceuticals or medical devices. In March 2017, following the passage of the budget resolution for the fiscal year of 2017, the U.S. House of Representatives passed legislation known as the American Health Care Act of 2017, which, if enacted, would amend or repeal significant portions of the ACA. Attempts in the Senate in 2017 to pass similar ACA repeal legislation, including the Better Care Reconciliation Act of 2017, were unsuccessful. However, in December 2017, the Tax Cuts and Jobs Act was enacted, which includes a provision that effectively repeals the ACA's individual mandate by reducing the tax penalty for failing to maintain minimum essential coverage to zero. Following this legislation, Texas and 19 other states filed a lawsuit alleging that the ACA is unconstitutional as the individual mandate was repealed, undermining the legal basis for the Supreme Court's prior decision. On December 14, Texas federal district court judge Reed O'Connor issued a ruling declaring that the ACA in its entirety is unconstitutional. Upon appeal, the Fifth Circuit upheld the district court's ruling that the individual mandate is unconstitutional. However, the Fifth Circuit remanded the case back to the district court to conduct a more thorough assessment of the constitutionality of the entire ACA despite the individual mandate being unconstitutional. While this decision has no immediate legal effect on the ACA and its provisions, this lawsuit is ongoing. The Bipartisan Budget Act of 2018, or BBA, passed in February 2018, set government spending levels for Fiscal Years 2018 and 2019 and revised certain provisions of the ACA. Specifically, beginning in 2019, the BBA increased manufacturer point-of-sale discounts off negotiated prices of applicable brand drugs in the Medicare Part D coverage gap from 50% to 70%, ultimately increasing the liability for brand drug manufacturers. This mandatory manufacturer discount also applied to biosimilars beginning in 2019. Regardless of whether or not the ACA is changed or modified by Congress or the Supreme Court, we expect both government and private health plans to continue to require healthcare providers, including healthcare providers that may one day purchase our products, to contain costs and demonstrate the value of the therapies they provide.

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

With the enactment of the Biologics Price Competition and Innovation Act of 2009, or BPCIA, as part of the ACA, an abbreviated pathway for the approval of biosimilar and interchangeable biological products was created. Under the BPCIA, an application for a biosimilar product cannot be submitted to the FDA until four years after, and approval by the FDA cannot be made effective until 12 years after, the date of the first licensure of the reference product. We believe that ELZONRIS, and or any of our product candidates that may receive marketing approval by the FDA as a biological product under a BLA, should qualify for the 12-year period of exclusivity. However, there is a risk that the U.S. Congress could amend the BPCIA to significantly shorten this exclusivity period, potentially creating the opportunity for generic competition sooner than anticipated. Moreover, the extent to which a biosimilar, once approved, will be substituted for any one of our reference products in a way that is similar to traditional generic substitution for non-biological products is not yet clear, and will depend on a number of marketplace and regulatory factors that are still developing. In addition, a competitor could decide to forego the biosimilar route and submit a full BLA after completing its own preclinical studies and clinical trials. In such cases, any exclusivity to which we may be eligible under the BPCIA would not prevent the competitor from marketing its product as soon as it is approved.

Risks Related to Our Financial Position and Capital Requirements

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

We have incurred net losses from operations from our inception through December 31, 2019 of approximately \$377.6 million. We do not know whether or when we will become profitable. Our losses have resulted principally from costs incurred in development and discovery activities. We anticipate that our operating losses will decrease over the next several years as we execute our strategy to commercialize ELZONRIS coupled with our plan to expand our discovery, research and development activities. We believe that our existing cash, cash equivalents, and investments, including the cash proceeds received from our follow-on public offering during the third quarter of 2019, will be sufficient to fund our operations and our capital expenditures for at least the next two years. If our cash is insufficient to meet future operating requirements, we will have to raise additional funds. If we are unable to obtain additional funds on terms favorable to us or at all, we may be required to cease or reduce our operating activities or sell or license to third-parties some or all of our intellectual property. If we raise additional funds by selling additional shares of our capital stock, the ownership interests of our stockholders will be diluted. If we need to raise additional funds through the sale or license of our intellectual property, we may be unable to do so on terms favorable to us, if at all. In addition, if we do not continue to meet our diligence obligations under our license agreements for our clinical drug candidates that we have in-licensed, we will lose our rights to develop and commercialize those clinical drug candidates.

If we do not successfully develop and obtain regulatory approval for our existing and future product candidates and effectively manufacture, market and sell our product candidates that are approved, we may never generate sales of those product candidates, and even if we do generate 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, 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 clinical product candidates and may expend additional resources on other product candidates and drug acquisition or discovery efforts. These expenditures will include costs associated with general administration, facilities, research and development, acquiring new technologies, manufacturing product and product candidates, conducting preclinical experiments, conducting clinical trials, preparing for and having regulatory interactions including applying for regulatory approvals, and commercializing ELZONRIS, as well as any product candidates that might receive approval for sale, as well as costs associated with operating as a public company.

As the outcome of our ongoing and future clinical trials is highly uncertain, our estimates of clinical trial costs necessary to successfully complete the development and commercialization of our product candidates may differ significantly from our actual costs. In addition, other unanticipated costs may arise, which could cause us to seek additional funds sooner than planned.

Our future capital requirements depend on many factors, including:

- the number of product candidates we pursue and the specific capital requirements to develop each product candidate;
- the scope, progress, results and costs of researching and developing our product candidates, and conducting preclinical studies and clinical trials;
- the ability of our product candidates (including ELZONRIS for use in other indications) to progress through clinical development successfully;
- the timing of, and the costs involved in, seeking regulatory approvals for our product candidates;
- the cost of commercialization activities for ELZONRIS, along with any of our other product candidates that may be approved for sale, including marketing, sales and distribution costs;
- the cost associated with securing and establishing commercialization and manufacturing capabilities for our product and product candidates for which we might receive regulatory approval;
- our ability to establish and maintain strategic partnerships, licensing or other arrangements and the economic and other terms of such agreements;
- the costs involved in preparing, filing, prosecuting, maintaining, defending and enforcing patent claims, including litigation costs and the outcome of such litigation;
- the timing, receipt and amount of sales of, or royalties on, our future products, if any;
- our need and ability to hire additional management and scientific, medical, sales, and marketing personnel;
- the effect of competing technological and market developments; and

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

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

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

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

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

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

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

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

Although we received FDA approval for ELZONRIS, and even if we receive regulatory approval for any of our product candidates, sales of our product depend on reimbursement by government health administration authorities, private health insurers, and other organizations. If we are unable to obtain or maintain anticipated levels, reimbursement for our product or coverage may be reduced, our pricing may be affected or our product sales, results of operations or financial condition could be harmed.

We may not be able to sell our products on a profitable basis or our profitability may be reduced if we are required to sell our products at lower than anticipated prices or reimbursement is unavailable or limited in scope or amount. Our products may be priced significantly more expensive than traditional drug treatments and almost all patients require some form of third-party coverage to afford their cost. We anticipate that we will depend, to a significant extent, on governmental payers, such as Medicare and Medicaid in the U.S. or country specific governmental organizations in foreign countries, and private third-party payers to defray the cost of our products to patients. These entities may refuse to provide coverage and reimbursement, determine to provide a lower level of coverage and reimbursement than anticipated, or reduce previously approved levels of coverage and reimbursement, including in the form of higher mandatory rebates or modified pricing terms.

In certain countries where we sell or are seeking or may seek to commercialize our products, pricing, coverage and level of reimbursement or funding of prescription drugs are subject to governmental control. We may be unable to timely or successfully negotiate coverage, pricing, and reimbursement on terms that are favorable to us, or such coverage, pricing, and reimbursement may differ in separate regions in the same country. In some foreign countries, the proposed pricing for a drug must be approved before it may be lawfully marketed. The requirements governing drug pricing vary widely from country to country, which may include a combination of distinct potential payers, including private insurance and governmental payers as well as health technology assessment of medicinal products for pricing and reimbursement methodologies. Therefore, we may not successfully conclude the necessary processes and commercialize our products in every, or even most countries in which we seek to sell our products.

A significant reduction in the amount of reimbursement or pricing for our products in one or more countries may reduce our profitability and adversely affect our financial condition. Certain countries establish pricing and reimbursement amounts by reference to the price of the same or similar products in other countries. Therefore, if coverage or the level of reimbursement is limited in one or more countries, we may be unable to obtain or maintain anticipated pricing or reimbursement in current or new territories. In the U.S., the European Union member states, and elsewhere, there have been, and we expect there will continue to be, efforts to control and reduce healthcare costs. In the U.S. for example, the price of drugs has come under intense scrutiny by the U.S. Congress. Third party payers decide which drugs they will pay for and establish reimbursement and co-payment levels. Government and other third-party payers are increasingly challenging the prices charged for healthcare products, examining the cost effectiveness of drugs in addition to their safety and efficacy, and limiting or attempting to limit both coverage and the level of reimbursement for prescription drugs.

Health insurance programs may restrict coverage of some products by using payer formularies under which only selected drugs are covered, variable co-payments that make drugs that are not preferred by the payer more expensive for patients, and by using utilization management controls, such as requirements for prior authorization or failure first on another type of treatment. Payers may especially impose these obstacles to coverage for higher-priced drugs, and consequently our products may be subject to payer-driven restrictions. Additionally, U.S. payers are increasingly considering new metrics as the basis for reimbursement rates.

In countries where patients have access to insurance, their insurance co-payment amount or other benefit limits may represent a barrier to obtaining or continuing use of our potential products. We anticipate providing support for non-profit organizations that assist patients in accessing treatment for certain diseases. Such organizations assist patients whose insurance coverage imposes prohibitive co-payment amounts or other expensive financial obligations. Such organizations' ability to provide assistance to patients is dependent on funding from external sources, and we cannot guarantee that such funding will be provided at adequate levels, if at all. We also may provide our products without charge to patients who have no insurance coverage for drugs through related charitable purposes. We are not able to predict the financial impact of the support we may provide for these and other charitable purposes; however, substantial support could have a material adverse effect on our profitability in the future.

Our commercial success depends on obtaining and maintaining reimbursement at anticipated levels for our products. It may be difficult to project the impact of evolving reimbursement mechanics on the willingness of payers to cover our products. If we are unable to obtain or maintain coverage, or coverage is reduced in one or more countries, our pricing may be affected or our product sales, results of operations or financial condition could be harmed.

Risks Related to Our Business and Industry

We are a commercial-stage biopharmaceutical company with one FDA approved product in a single indication, which makes it difficult to assess our future viability.

We are a commercial-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 and sustain successful product candidate development activities;
- obtain required regulatory approvals for the development and commercialization of our product candidates, and any new or expanded indications for these product candidates;
- maintain, defend, leverage and expand our intellectual property portfolio;
- build, deploy, and maintain sales, distribution and marketing capabilities, either on our own or in collaboration with strategic partners should our product candidates obtain market approval;
- gain market and third-party payor acceptance for ELZONRIS, our approved product, and our other product candidates, should they obtain market approval, or ELZONRIS should it obtain market approval in additional indications;
- develop and maintain CGMP compliant manufacturing and distribution capabilities sufficient to support the intended scope of our preclinical and clinical development plans and the commercial demand for our product;
- complete required process characterization and validation activities to support any planned regulatory submission, which historically has included the manufacture of at least three consecutive successful process validation batches for drug substance and at least three consecutive successful process validation batches for drug product;
- implement a successful post-market surveillance program to monitor the safety of any approved product that are being commercially marketed and sold;
- develop and maintain any strategic relationships we elect to enter into;
- satisfy our obligations under our licensing agreement and other agreements; and

- manage our spending as costs and expenses increase due to drug acquisition, discovery, preclinical development, clinical trials, regulatory interactions, agreements, and approvals, manufacturing, and commercialization.

If we are unsuccessful in accomplishing these objectives, we may not be able to adequately commercialize our product(s) or product candidates, generate meaningful revenue, develop our 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 or gaining acceptance for products or for the same markets that we plan to target. If we are not “first to market” with our product candidates, our competitive position could be compromised because it may be more difficult for us to obtain marketing approval for that product candidate and successfully market that product candidate as a competitor.

Even if we are “first to market” with one or more of our product candidates, a competitor could develop an alternative therapy for our approved indication(s) that demonstrates a superior efficacy and/or safety profile relative to our approved product.

We expect any product candidate that we are able to 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 with which we may potentially compete including AbbVie Inc., Agenus Inc., Agios Pharmaceuticals, Inc., Ambit Biosciences Corporation (now a Daiichi Sankyo company), Amgen Inc., Astellas Pharma U.S., Inc., Astex Pharmaceuticals (now an Otsuka Pharmaceutical company), Bayer AG, Bionomics Limited, Blueprint Medicines Corp., Boehringer Ingelheim GmbH, Bristol-Myers Squibb, Inc., Celator Pharmaceuticals (now a Jazz Pharmaceuticals company), Celgene Corporation, Celldex Therapeutics, Inc. Cellectis, Cortice Biosciences, Inc., CTI BioPharma Corp., Cyclacel Pharmaceuticals, Inc., CytRx Corporation, Eli Lilly and Company, Eisai Co., Ltd., Genmab, Sanofi Genzyme Corporation, Geron Corp., GlaxoSmithKline plc, Humanigen, Inc., Ignyta, Inc. (a Roche company), ImmunoCellular Therapeutics, Ltd., ImmunoGen, Inc., Impact Biomedicines, Inc. (now a Celgene company), Incyte Corporation, Inspyr Therapeutics, Inc., Janssen Pharmaceutical Companies of Johnson & Johnson, Karyopharm Therapeutics Inc., Kura Oncology, Inc., MacroGenics, Inc., Merck & Co., Inc., Micromet, Inc. (an Amgen Inc. company), Mustang Bio, Inc., Northwest Biotherapeutics, Inc., Novartis International AG, OncoMed Pharmaceuticals, Inc., Pfizer Inc., Roche Holding AG, Sanofi U.S. LLC, Seattle Genetics, Inc., Stemcentrx, Inc. (an AbbVie company), Sumitomo Dainippon Pharma Co., Ltd., Sunesis Pharmaceuticals, Inc., Verastem, Inc., Xencor, Inc. and others.

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

- design and develop products that address an unmet medical need or demonstrate a superior benefit/risk profile to other products in the market;
- conduct successful preclinical studies and clinical trials;
- attract qualified scientific, medical, sales, marketing and commercial personnel;

- obtain patent and/or other intellectual property protections 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 competitors, our business will not grow and our financial condition and operations will suffer.

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

Our success depends in part on our continued ability to attract, retain and motivate highly qualified management, clinical and scientific personnel. We are highly dependent upon our senior management as well as other employees, consultants and scientific and medical collaborators. As of March 16, 2020, we had 100 full-time employees, which may make us more reliant on our individual employees than companies with a greater number of employees. The loss of services of any of these individuals or one or more of our other members of senior management could delay or prevent the successful development of our product pipeline, completion of our ongoing and future clinical trials or the commercialization and successful marketing launch of our product candidates.

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

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

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

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

We expect to expand our development, regulatory, and sales and marketing capabilities, and as a result, we may encounter difficulties in managing our growth, which could disrupt our operations.

In connection with our commercial launch of ELZONRIS and possible international expansion, we expect to experience significant growth in the number of our employees and the scope of our operations, particularly in the areas of drug development, regulatory affairs, quality, commercial compliance, medical affairs, and sales and marketing. For example, we plan to hire additional personnel in connection with our commercial launch of ELZONRIS in the United States and Europe and prepare for potential regulatory filings for ELZONRIS in other markets. To manage our anticipated future growth, we must continue to implement and improve our managerial, operational and financial systems, expand our facilities and continue to recruit and train additional qualified personnel. 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. Our future financial performance, our ability to successfully commercialize ELZONRIS and our product candidates, and our ability to compete effectively will depend, in part, on our ability to manage any future growth effectively. Our management team has limited experience in managing a company with this anticipated growth, and we may not be able to do so effectively. The physical expansion of our operations may lead to significant costs and may divert our management and business development resources. We may not be able to effectively manage the expansion of our operations, which could delay the execution of our business plans or disrupt our operations.

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 and commercial sale of our product candidates and products. For example, we may be sued if any product we develop allegedly causes or contributes to an injury or is found to be otherwise defective during product testing, clinical study, clinical use, manufacturing, marketing or sale. Any such product liability claims may include allegations of defects in manufacturing, defects in design, failure to warn of dangers inherent in the product, negligence, strict liability and a breach of warranties. Fraud-based claims, as well as claims made pursuant to state consumer protection acts, are also a possibility. If we cannot successfully defend ourselves against product liability claims, we may incur substantial liabilities or be required to limit commercialization of our product candidates. Even a 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 and product candidates that we may develop;
- injury to our reputation;
- withdrawal of clinical trial participants and inability to enroll future clinical trial participants;
- costs to defend the related litigation;
- a diversion of management's time and our resources;

- substantial monetary awards to trial participants or patients;
- product recalls, withdrawals or labeling, marketing or promotional restrictions;
- loss of income;
- 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 healthcare providers, physicians, and third-party payors in the United States and in foreign jurisdictions are subject to applicable anti-kickback, fraud and abuse and other healthcare laws and regulations, which could expose us to criminal, civil or administrative sanctions, contractual damages, potential exclusion from government-funded healthcare programs, reputational harm, and diminished profits and future earnings.

Healthcare providers, physicians and third-party payors in the United States and in foreign jurisdictions 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 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 civil penalties, against individuals or entities for knowingly presenting, or causing to be presented, to the federal government, claims for payment that are false or fraudulent or making a false statement to avoid, decrease or conceal an obligation to pay money to the federal government and also includes provisions allowing for private, civil whistleblower or “qui tam” actions;
- the federal Health Insurance Portability and Accountability Act of 1996, or HIPAA, as amended by the Health Information Technology for Economic and Clinical Health Act, or 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 attorney generals;

- 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 ACA, commonly referred to as the Sunshine Act requires manufacturers of drugs, devices, biologics and medical supplies to report to the HHS information related to U.S.-licensed physician and teaching hospital payments and other transfers of value including research payments and ownership and investment interests;
- analogous state laws and regulations, such as state anti-kickback and false claims laws, may apply to sales or marketing arrangements and claims involving healthcare items or services reimbursed by non-governmental third-party payors, including private insurers, and some state laws require pharmaceutical companies to comply with the pharmaceutical industry's voluntary compliance guidelines and the relevant compliance guidance promulgated by the federal government in addition to requiring drug manufacturers to report information related to payments to physicians and other healthcare providers or marketing expenditures;
- the U.S. Foreign Corrupt Practices Act, or FCPA, and other anti-corruption laws and regulations pertain to interactions with foreign government officials. The FCPA prohibits U.S. companies and their employees, officers, and representatives from paying, offering to pay, promising, or authorizing the payment of anything of value, directly or indirectly, to any foreign government official, which includes any officer, employee, political candidate or any person acting in an official capacity for or on behalf of any agency, instrumentality, department, subdivision, or other body of any national, state, or local government, for the purpose of influencing the foreign official in his or her official capacity, inducing the foreign official to do or omit to do an act in violation of his or her lawful duty, or to secure any improper advantage in order to obtain or retain business or to otherwise seek favorable treatment. In many countries in which we operate or sell our products, the healthcare professionals with whom we interact may be considered to be foreign government officials for purposes of the FCPA. The FCPA's Accounting Provisions separately require that publicly traded companies make and keep books, records and accounts, which in reasonable detail, accurately and fairly reflect the transactions and dispositions of the company's assets and devise and maintain a system of internal controls sufficient to assure management's control, authority, and responsibility over the company's assets;
- the European Union's General Data Protection Regulation and implementing laws in its member states govern the collection and processing of residents' personal data and, among other requirements, imposes certain consent and data access rights. Such laws may impact our ability to conduct clinical trials that involve personal data and engage in other activities that require the processing of personal data. Outside of the U.S. and the European Union, there are numerous other jurisdictions that have their own privacy and information security laws, and new laws and regulations are being considered and/or enacted globally, which may affect our ability to collect, process, and store their residents' personal data; and
- analogous anti-kickback, fraud and abuse and healthcare laws and regulations in foreign countries.

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

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

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

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

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

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

Our internal computer systems, or those of our third-party CMOs, CROs or other contractors or consultants, may fail or suffer security breaches, which could result in a material disruption of our product candidates' development programs.

Despite the implementation of security measures, our internal computer systems and those of our third-party CMOs, CROs and other contractors and consultants are vulnerable to damage from computer viruses, unauthorized access, theft, natural disasters, terrorism, war and telecommunication and electrical failures. While we have not experienced any such system failure, accident or security breach to date, if such an event were to occur and cause interruptions in our operations, it could result in a material disruption of our programs. For example, the loss of clinical trial data for our product candidates could result in delays in our regulatory approval efforts and significantly increase our costs due to recovering or reproducing the data. To the extent that any disruption or security breach results in a loss of or damage to our data or applications, loss of or damage to other data or applications relating to our technology or product candidates, or inappropriate disclosure or theft of confidential or proprietary information, we could incur liabilities and the further development of our product candidates could be delayed.

Europe has enacted a new data privacy regulation, the General Data Protection Regulation, a violation of which could subject us to significant fines.

In May 2018, a new privacy regime, the General Data Protection Regulation, or GDPR, took effect across all member states of the European Economic Area. The new regime increases our obligations with respect to clinical trials conducted in the member states by expanding the definition of personal data to include coded data, and requiring changes to informed consent practices and more detailed notices for clinical trial subjects and investigators. In addition, it increases the scrutiny that clinical trial sites located in the member states should apply to transfers of personal data from such sites to countries that are considered to lack an adequate level of data protection, such as the United States. The regime imposes substantial fines for breaches of data protection requirements, which can be up to four percent of global turnover or 20 million Euros, whichever is greater, and it also confers a private right of action on data subjects for breaches of data protection requirements. Compliance with these directives is a rigorous and time-intensive process that may increase our cost of doing business, and the failure to comply with these laws could subject us to significant fines.

Risks Related to Our Dependence on Third Parties in the U.S. and Abroad

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

We are currently advancing our clinical-stage product candidates through multiple corporate-sponsored clinical trials under corporate-sponsored INDs and through investigator sponsored trials. Prior to sponsoring our INDs for our product candidates, faculty members at academic institutions and other companies may have conducted and sponsored the INDs and clinical trials relating to our drug candidates. As such, we did not control the design or conduct of any trials conducted prior to the initiation of our corporate-sponsored INDs, and it is possible that the FDA will not view these previous trials as providing adequate support for future clinical trials or regulatory filings.

In addition, we have relied on contractual arrangements with academic institutions and investigators that provide us certain information rights with respect to the completed investigator-sponsored trials, including access to, and the ability to use and reference the data, including for our own regulatory filings, resulting from the completed trials. If these obligations are breached by the investigators or institutions, or if the data prove to be inadequate, then our ability to conduct our planned corporate-sponsored clinical trials may be adversely affected. Additionally, the FDA may disagree with our interpretation of the adequacy of the preclinical, manufacturing, and/or clinical data from these studies. If so, the FDA may require us to obtain and submit additional preclinical, manufacturing, and/or clinical data relating to our planned trials and/or may not accept such additional data as adequate for our regulatory filings.

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

To conduct our preclinical and clinical studies, we rely on academic institutions, CROs, hospitals, clinics and other third-party collaborators who are outside our direct control, which limits our control over the overall conduct of these studies and the ability to successfully complete them. In our corporate-sponsored trials and investigator sponsored trials of ELZONRIS and our clinical drug candidates, we have continued to engage various third parties. If we are unable to maintain or enter into agreements with these third parties on acceptable terms, or if any such engagement is terminated, we may be unable to enroll patients on a timely basis or otherwise conduct our trials in the manner we anticipate. In addition, there is no guarantee that these third parties will devote adequate time and resources to our studies or perform as required by contract or in accordance with regulatory requirements. If these third-parties fail to meet expected deadlines, fail to adhere to protocols or fail to act in accordance with regulatory requirements or our agreements with them, or if they otherwise perform in a substandard manner or in a way that compromises the quality or accuracy of their activities or the data they obtain, then trials of our product candidates may be extended, delayed, compromised 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 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 may bear all of the risk related to the development of any such product candidates;
- the competitiveness of any product candidate that is commercialized could be reduced; and
- with respect to our platform technology, StemScreen®, we may not realize its potential as a means of identifying and validating new cancer therapies.

We rely on third-party manufacturers to produce and supply our commercial products, as well as our clinical and preclinical 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 products, or continue to sell our approved products.

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

We also expect to rely upon third parties to produce and test drug substance and drug product required for the clinical trials and commercial supply of our product and product candidates. If we are unable to arrange for third-party manufacturing sources, or to do so on commercially reasonable terms or on a timely basis, we may not be able to complete the development of such other product candidates or market those that are approved. 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, our supply needs for ELZONRIS, and future commercialization of any product candidates for which we may receive regulatory approval. In addition, the FDA and other regulatory authorities require that our products and product candidates be manufactured according to CGMPs, and similar standards for products manufactured for markets outside the U.S. Any failure by our third-party manufacturers to comply with CGMPs or failure to scale up manufacturing processes, including any failure to deliver sufficient quantities of product or product candidates of acceptable quality in a timely manner, could lead to a delay in, or failure to obtain, regulatory approval for trial initiation or marketing of any of our product candidates or commercial products. In addition, such failures could be the basis for action by the FDA to withdraw product approvals previously granted to us and for other regulatory action, which could result in or lead to recall, seizure, import alerts, fines, imposition of operating restrictions, total or partial suspension of production, injunctions, consent decrees, or civil or criminal sanctions.

Any adverse developments affecting our manufacturing operations or the operations of our third-party providers could result in a product shortage of clinical or commercial requirements, withdrawal of our product candidates or any approved products, shipment delays, lot failures or recalls. We may also have to write-off inventory and incur other charges and expenses for products that fail to meet specifications, undertake costly remediation efforts or seek more costly manufacturing alternatives. Each of these could have an adverse material impact on our business individually or in the aggregate.

We rely on our third-party manufacturers to purchase from third-party suppliers the materials necessary to produce our products, and product candidates for our clinical studies. There are a small number of suppliers for certain capital equipment and materials that we use to manufacture and test our drugs. Such suppliers may not sell these materials to our third-party manufacturers at the times we need them or on commercially reasonable terms. We do not have control over the process or timing of the acquisition of these materials by our third-party 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 third-party manufacturers or we are unable to purchase these materials after regulatory approval has been obtained for our product candidates, the commercial launch of our product candidates would be delayed or there would be a shortage in supply, which would impair our ability to generate revenues from the sale of our products or product candidates.

We are working with our third-party manufacturers to optimize the manufacturing processes for our products and product candidates, including related drug substances, so that these products and product candidates may be routinely produced in adequate quantities of adequate quality, and at an acceptable cost, to support our clinical trials and commercialization of products that might be approved. Our third-party manufacturers may not be able to control batch-to-batch variability below an acceptable threshold, increasing the risk of batch failures, which could cause significant delays and increased costs to our programs. Our third-party manufacturers may not be able to manufacture our products or 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 product candidates, we may be required to establish or access large-scale commercial manufacturing capabilities, and may require different technologies to manufacture these products. In addition, assuming that our drug development pipeline increases and matures, we will have a greater need for clinical trial and commercial manufacturing capacity. To meet our projected needs for commercial manufacturing, third parties with whom we currently work may need to increase their scale of production and/or we may need to secure additional suppliers with appropriate technologies to support our new product candidates. If our projected needs for commercial manufacturing are not accurate, or we do not obtain regulatory approval of new products or additional indications for existing products or additional delivery systems, or are significantly delayed or limited in doing so, our revenue may be adversely affected, we may experience surplus inventory and we may be required to write down certain assets.

Business or economic disruptions or global health concerns could seriously harm our development efforts and increase our costs and expenses.

Broad-based business or economic disruptions could adversely affect our ongoing or planned research and development, clinical, or commercial activities. For example, in December 2019 an outbreak of a novel strain of coronavirus originated in Wuhan, China, and has since spread to a number of other countries, including the United States. To date, this outbreak has already resulted in extended shutdowns of certain businesses in the Wuhan region and has had ripple effects to businesses around the world. The outbreak may result in additional or more extensive travel restrictions, closures, disruptions of businesses or facilities in China or other affected regions around the world or lead to social, economic, political or labor instability in the affected areas may impact our suppliers' or our customers' operations.

Global epidemics, such as the coronavirus, could also negatively affect the hospitals and clinical sites in which we conduct any of our clinical trials, which could have a material adverse effect on our business and our results of operation and financial condition. We cannot presently predict the scope and severity of any potential business shutdowns or disruptions, but if we or any of the third parties with whom we engage, including the suppliers, clinical trial sites, regulators and other third parties with whom we conduct business, were to experience shutdowns or other business disruptions, our ability to conduct our business in the manner and on the timelines presently planned could be materially and negatively impacted.

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

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

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

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

Our global commercialization strategy for certain of our product or product candidates may depend on our ability to enter into agreements with collaborators to obtain assistance and funding for the development and commercialization of these product or product candidates. Supporting diligence activities conducted by potential collaborators and negotiating the financial and other terms of a collaboration agreement are long and complex processes with uncertain results. Even if we are successful in entering into one or more collaboration agreements, collaborations may involve greater uncertainty for us, as we have less control over certain aspects of our collaborative programs than we do over our proprietary development and commercialization programs. We may determine that continuing to collaborate under the terms provided is not in our best interest, and we may terminate such collaboration. Our collaborators could delay or terminate their agreements, and our product or product candidates 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, so 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 income. In addition, we could have disputes with our future collaborators, on issues 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 might not be resolved in our favor.

Even with respect to certain other products 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 might 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 products or product candidates.

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

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

Recent patent reform legislation could increase the uncertainties and costs surrounding the prosecution of our patent applications and the enforcement or defense of our issued patents. The Supreme Court has issued several decisions in patent cases in recent years, which either narrow the scope of patent protection or weaken the rights of patent owners in certain situations. In addition to increasing uncertainty in regards to our ability to obtain patents in the future, this combination of events has created uncertainty with respect to the value of patents, once obtained. Depending on decisions by the U.S. Congress, the federal courts, and the USPTO, the laws and regulations governing patents could change in unpredictable ways that could hinder our ability to obtain new patents or to enforce our existing patents and patents that we might obtain in the future. On September 16, 2011, the Leahy-Smith America Invents Act, or the Leahy-Smith Act, was signed into law. The Leahy-Smith Act includes a number of significant changes to the United States patent law. These include changes to transition from a "first-to-invent" system to a "first-to-file" system and to the way issued patents are challenged.

The formation of the Patent Trial and Appeal Board (PTAB) now provides a quicker and less expensive process for challenging issued patents. Our patents, even after they are issued by the USPTO, may be challenged by competitors in the PTAB, in addition to challenges we could have faced previously in district court. The USPTO recently developed new regulations and procedures to govern the administration of the Leahy-Smith Act, and many of the substantive changes to patent law associated with the Leahy-Smith Act, and in particular, the first-inventor-to-file provisions, only became effective on March 16, 2013. Accordingly, it is not clear what, if any, impact the Leahy-Smith Act will have on the operation of our business.

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

Our patents and patent applications may not be sufficient to protect our product and product candidates from commercial competition. For example, we cannot obtain a composition of matter patent and are limited in the types of claims that we can obtain for ELZONRIS due to earlier published prior art. We have however obtained U.S. and foreign patents for certain methods of using ELZONRIS to treat AML, BPDCN, CMML, and myelodysplastic syndrome, or MDS. In addition, we have filed additional U.S. and foreign patent applications for the method of using ELZONRIS to treat AML, MDS, BPDCN, CMML, and other diseases although there can be no assurances that such patents will issue.

Failure to obtain patents directed to all approved uses of ELZONRIS may enable a competitor to market ELZONRIS for such approved but unpatented indication(s), which could lead to price erosion for sales of ELZONRIS. With respect to SL-701, although we have licensed an issued U.S. patent directed to the composition of matter for the mutant immunogenic IL-13R α 2 peptide, as well as issued patents in Europe, Japan, Australia, and Mexico directed to uses of the SL-701 composition, we currently do not have any composition of matter patent protection for SL-701. We have filed U.S. and foreign patent applications directed to methods of use of a new survivin mutant peptide for use in SL-701. The U.S. patent application directed to certain uses of a new survivin mutant peptide for use in SL-701 for treating brain cancer has been issued by the USPTO as U.S. 10,485,858 and patents have been issued for certain uses of the SL-701 composition in Europe and Japan. While we have a non-exclusive license to issued U.S. patents directed to methods of use for the EphA2 peptide, we currently do not have any composition of matter patent protection, although we do have rights to foreign pending patent applications that seek to cover certain uses of this peptide. With respect to SL-801 and SL-901, we have licensed composition of matter patents issued in the U.S. and abroad directed to the SL-801 compound and SL-901. With respect to SL-1001, we have filed U.S. and foreign patent applications directed to the SL-1001 composition, and these applications are pending. 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 or 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 products or product candidates, the defendant could counterclaim that our patent is invalid and/or unenforceable. In patent litigation in the United States, defendant counterclaims alleging invalidity and/or unenforceability are commonplace. Grounds for a validity challenge could be an alleged failure to meet any of several statutory requirements, for example, lack of patentable subject matter, novelty, obviousness, written description or non-enablement.

Grounds for an unenforceability assertion could be an allegation that someone connected with the prosecution of the patent withheld relevant information from the USPTO, or made a misleading statement, during prosecution. The outcome following legal assertions of invalidity and unenforceability during patent litigation is unpredictable. Furthermore, any claims asserted against accused infringers could provoke those parties to petition the USPTO to institute inter partes review against the asserted patents, which may lead to a finding that all or some of the claims of the patent are invalid. With respect to the validity question, for example, we cannot be certain that there is no invalidating prior art, of which we and the patent examiner and we were unaware during prosecution. If a defendant were to prevail on a legal assertion of invalidity and/or unenforceability, we would lose at least part, and perhaps all, of the patent protection on one or more of our products or product candidates or certain aspects of our platform technology, StemScreen®. Such a loss of patent protection could have a material adverse impact on our business. Furthermore, adverse results on U.S. patents may affect related patents in our global portfolio.

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

We cannot guarantee that our product or product candidates, the use of our product or product candidates, or our platform technology, StemScreen®, do not infringe third-party patents or other intellectual property. Third parties might allege that we are infringing their patent rights or that we have misappropriated their trade secrets. Such third parties might resort to litigation against us. The basis of such claims that our product candidates or platform technology infringe the rights of third parties could also adversely affect our business. Regardless of the outcome of any litigation, defending the litigation may be expensive, time-consuming and distracting to management. For example, we are aware of third-party patents with certain claims that may be directed to some of our product candidates, including one of the peptides used in SL-701 and structures which may be related to SL-1001. 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 reasonable or commercially acceptable terms or at all. Failure to obtain any required licenses could restrict our ability to commercialize our products in certain territories or subject us to patent infringement litigation, could result in us having to cease commercialization of our products and/or subject us to money damages in such territories.

It is also possible that we have failed to identify relevant patents or applications. Patent applications covering our product, product candidates, 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 product, product candidates 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 expected, reasonable, or acceptable terms, or at all. Even if we or any future strategic partners were able to obtain a license, the rights may be non-exclusive, which could result in our competitors gaining access to the same intellectual property.

Ultimately, we could be prevented from commercializing one or more of our products or 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. 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 a patent owner and us. 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. It is possible that the necessary licenses will not be available to us on commercially acceptable terms, or at all. This could limit our research and development activities, our ability to commercialize certain products, or both.

Most of our competitors are larger than we are and have substantially greater resources. They are, therefore, likely to be able to sustain the costs of complex patent litigation longer than we could. In addition, the uncertainties associated with litigation could have a material adverse effect on our ability to raise the funds necessary to continue our clinical trials, continue our internal research programs, in-license needed technology, or enter into strategic partnerships that would help us bring our product candidates to market. Such litigation or proceedings could substantially increase our operating losses and reduce the resources available for development activities or any future sales, marketing or distribution activities. We may not have sufficient financial or other resources to conduct such litigation or proceedings adequately.

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.

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

We are a party to several license agreements relating to certain patents and patent applications owned by third parties, upon which certain aspects of our business depend. In particular, we hold an exclusive license from Scott and White Memorial Hospital, or Scott and White, for ELZONRIS and SL-501, and we hold three licenses, including an exclusive license and two non-exclusive licenses, from the University of Pittsburgh relating to SL-701. Our license agreement with Scott and White survives, unless earlier terminated, until the later of the expiration of the last to expire licensed patent or the date on which we owe no further payments to Scott and White. Our exclusive and our non-exclusive patent license agreements with the University of Pittsburgh survive, unless earlier terminated, until the expiration of the last to expire licensed patent, and our non-exclusive license with the University of Pittsburgh to use and reference certain clinical trial data and information survives for a term of twenty years unless earlier terminated. We hold an exclusive worldwide license from CanBas Co., Ltd. for SL-801. The agreement with CanBas Co., Ltd. survives until the later of ten years following the first commercial sale of each product in each country; the date upon which there are no more valid claims; or the expiration or termination of the last regulatory exclusivity period, after which our license becomes fully paid, irrevocable, perpetual, non-exclusive and royalty-free. In March 2019, we acquired an exclusive worldwide license to pending patents covering the SL-1001 component from CRT Pioneer Fund. We also hold an exclusive worldwide license from UCB Biopharma SPRL for the patents covering SL-901. In addition, we hold licenses from academic institutions relating to intellectual property underlying ELZONRIS and our product candidates. We also hold an exclusive license from CRT Pioneer Fund LP for SL-1001, which survives until Stemline's obligation to pay royalties ends, which the agreement defines as the later of the date when the licensed product is no longer within the scope of a valid claim of a licensed patent in the country of sale or manufacture or the expiry of any extended exclusivity period in the relevant country, unless earlier terminated. We expect to enter into additional license agreements as part of the development of our business.

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

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

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 ELZONRIS and 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.

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

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

Trade secrets are difficult to protect. Courts outside the United States sometimes are less willing than U.S. courts to protect trade secrets. Moreover, if any of our trade secrets were to be lawfully obtained or independently developed by a competitor, we would have no right to prevent them, or those to whom they communicate it, from using that technology or information to compete with us. If any of our trade secrets were to be disclosed to or independently developed by a competitor, our competitive position would be harmed.

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

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

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

- others may be able to make compounds that are the same as or similar to our product or 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;
- it is possible that our applications for patent term extension for ELZONRIS pursuant to the Hatch Waxman Act will not result in added patent term, or may result in a shorter patent term extension than we applied for or that is available under the Hatch Waxman Act;
- the scope of our issued patents may not extend to competitive products developed or produced by others;
- issued patents that we own or have exclusively licensed may not provide us with any competitive advantages, or may be held invalid or unenforceable, as a result of legal challenges by our competitors;
- our competitors might conduct research and development activities in countries where we do not have patent rights or where the patent rights that we do not have are not comparable to those afforded in the United States, or where the applicable laws provide a safe harbor exemption from infringement liability for certain research purposes, and then use the information learned from such activities to develop competitive products for sale in our major commercial markets;
- we may not develop additional proprietary technologies that are patentable; and
- the intellectual property rights of others may have an adverse effect on our business.

Risks Related to Our Common Stock

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

The market price of our common stock may be highly volatile, and could be subject to wide fluctuations in response to various factors, some of which are beyond our control. Since our initial public offering which occurred in January 2013, the price of our common stock has ranged from \$3.43 per share to \$47.25 per share. The stock market in general and the market for biopharmaceutical companies in particular have experienced extreme volatility that has often been unrelated to the operating performance of particular companies. The market price for our common stock may be influenced by many factors, including:

- our ability to commercialize our approved product candidates;
- results from or delays of clinical trials of our product or product candidates, as well as results of regulatory reviews relating to the approval of our product candidates;
- our decision to initiate a clinical trial, not to initiate a clinical trial or to terminate an existing clinical trial;
- our dependence on third parties, including clinical research organizations and contract manufacturing organizations, trial sites, clinical trial sponsors and clinical investigators;
- the results of our efforts to discover, develop, acquire or in-license additional product candidates or products;
- new products, product candidates or new uses for existing products or technologies introduced or announced by our competitors and the timing of these introductions or announcements;
- regulatory or legal developments in the United States and other countries;
- our ability to maintain the license agreements for our product or 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 or product candidates or clinical development programs;
- actual or anticipated changes in estimates as to financial results, development timelines or recommendations by securities analysts;
- variations in our financial results or those of companies that are perceived to be similar to us;
- sales of common stock by us or our stockholders in the future, as well as the overall trading volume of our common stock;
- changes in the structure of healthcare payment systems and product pricing restrictions;
- market conditions in the pharmaceutical and biotechnology sectors;
- general economic, industry and market conditions and other factors that may be unrelated to our operating performance or the operating performance of our competitors, including changes in market valuations of similar companies; and
- the other factors described in this “Risk Factors” section.

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

Our executive officers, directors and principal stockholders beneficially own shares representing approximately 17.7% of our outstanding capital stock. As a result, if these stockholders were to choose to act together, they would be able to exert substantial influence over all matters submitted to our stockholders for approval, as well as our management and affairs. For example, these persons, if they choose to act together, would exert substantial influence over the election of directors and approval of any merger, consolidation or sale of all or substantially all of our assets. This concentration of voting power could delay or prevent an acquisition of our company on terms that other stockholders may desire.

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

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

- establish a classified board of directors such that not all members of the board are elected at one time
- allow the authorized number of our directors to be changed only by resolution of our board of directors;
- limit the manner in which stockholders can remove directors from the board;
- establish advance notice requirements for stockholder proposals that can be acted on at stockholder meetings and nominations to our board of directors;
- require that stockholder actions must be affected 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.

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

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

Item 1B. Unresolved Staff Comments

None.

Item 2. Properties

Our corporate and executive office is located in New York, New York, where we sublease space at 750 Lexington Avenue, Eleventh Floor, pursuant a lease agreement which expires in 2020. We are in the process of finalizing a new lease on the fourth floor at the same address to replace our expiring lease. Internationally, we lease additional office space in Zug, Switzerland. In the event we need additional space in the future, we believe it will be available at commercially reasonable terms.

Item 3. Legal Proceedings

From time to time, we are involved in legal proceedings in the ordinary course of our business. Refer to Note 15: Commitments and Contingencies for more information on legal proceedings.

Item 4. Mine Safety Disclosures

Not applicable.

Part II

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

Market Information

Our common stock is listed on the NASDAQ Capital Market and trades under the symbol "STML" and has been publicly traded since January 29, 2013. Prior to that time, there was no public market for our common stock. The following table sets forth the high and low sales prices for our common stock as reported on the NASDAQ Capital Market for the periods indicated.

	High	Low
2018		
First Quarter	\$ 18.75	\$ 13.05
Second Quarter	20.55	14.15
Third Quarter	17.85	13.90
Fourth Quarter	17.38	7.82
2019		
First Quarter	\$ 13.81	\$ 8.72
Second Quarter	16.35	12.58
Third Quarter	18.22	10.33
Fourth Quarter	11.38	8.45

Holdings

The number of record holders of our common stock as of March 16, 2020, was 188. This number does not include beneficial owners whose shares are held by nominees in street name.

Dividends

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

Securities Authorized for Issuance under Equity Compensation Plans

The following table contains information about our equity compensation plans as of December 31, 2019.

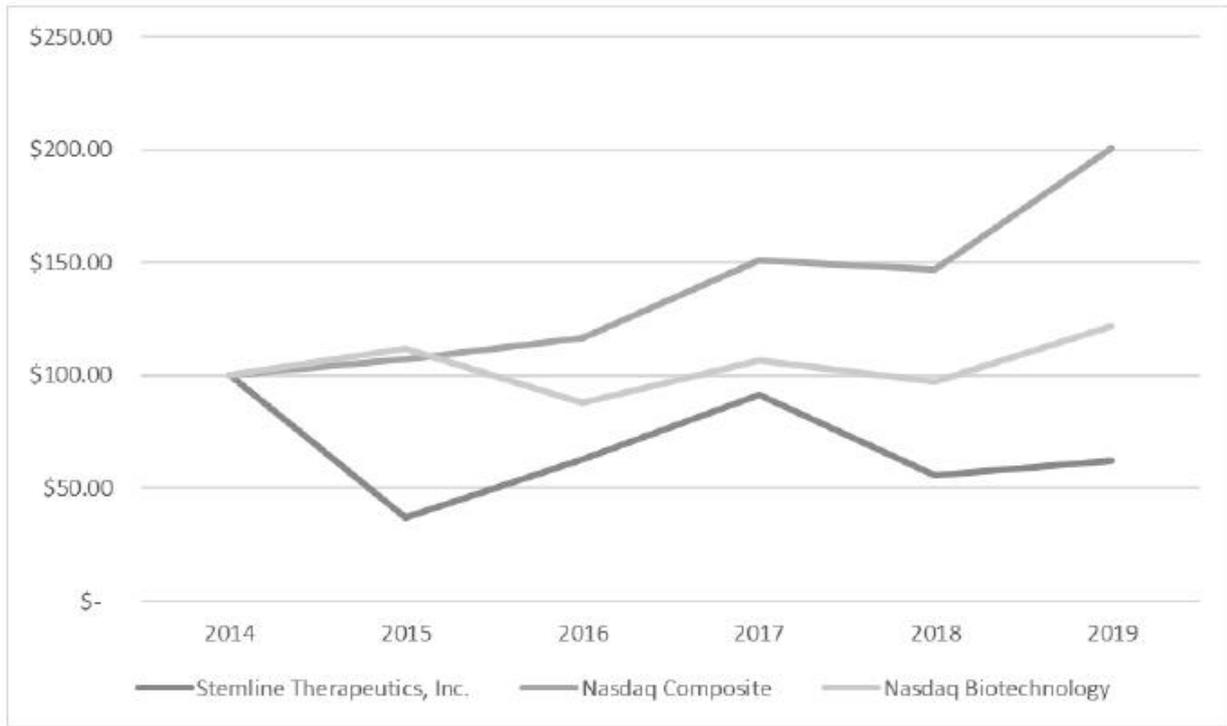
Equity Compensation Plan Information

Plan Category	Number of securities to be issued upon exercise of outstanding options and restricted stock	Weighted-average exercise price of outstanding options	Number of securities remaining available for future issuance under equity compensation plans (excluding securities reflected in column (a))
	(a)	(b)	(c)
Equity compensation plans approved by security holders			
Options	3,026,401	\$ 10.39	2,271,695
Restricted stock	93,000	N/A	—
Equity compensation plans not approved by security holders	—	—	—
Total	3,119,401	—	2,271,695

Common Stock Performance Graph

The following graph compares the cumulative total stockholder return on our common stock for the period from December 31, 2014 through December 31, 2019, with the cumulative total return over such period on (i) the U.S. Index of The NASDAQ Stock Market and (ii) the Biotechnology Index of The NASDAQ Stock Market. The graph assumes an investment of \$100 on December 31, 2014, in our common stock (at the closing market price) and in each of the indices listed above, and assumes the reinvestment of dividends.

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



* \$100 invested on December 31, 2014 in stock or index, including reinvestment of dividends.
 Fiscal year ending December 31.

Item 6. Selected Financial Data

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

	Year Ended December 31,				
	2019	2018	2017	2016	2015
Statement of consolidated operations data:					
Product revenue, net	\$ 43,216,862	\$ —	\$ —	\$ —	\$ —
Grant Income	—	500,000	898,199	1,041,354	654,160
Operating expenses:					
Cost of goods sold	4,893,339	—	—	—	—
Research and development	50,724,411	47,725,019	50,242,386	27,869,921	29,458,676
Selling, general and administrative	66,846,633	39,061,667	19,214,207	12,056,890	8,828,843
Total operating expenses	122,464,383	86,786,686	69,456,593	39,926,811	38,287,519
Loss from operations	(79,247,521)	(86,286,686)	(68,558,394)	(38,885,457)	(37,633,359)
Other (expense) income	(47,697)	(7,505)	(6,330)	11,438	1,609
Interest expense	(34,706)	(794)	—	—	—
Interest income	2,538,273	1,270,620	736,330	545,718	387,889
Net loss before income taxes	\$ (76,791,651)	\$ (85,024,365)	\$ (67,828,394)	\$ (38,328,301)	\$ (37,243,861)
Income tax (expense) benefit	(25,574)	—	—	25,296	—
Net loss	\$ (76,817,225)	\$ (85,024,365)	\$ (67,828,394)	\$ (38,303,005)	\$ (37,243,861)
Net loss attributable to common stockholders per common share:					
Basic and Diluted	\$ (1.83)	\$ (2.99)	\$ (2.94)	\$ (2.15)	\$ (2.15)
Weighted average number of common shares:					
Basic and Diluted	42,091,027	28,388,901	23,056,928	17,804,681	17,289,021
	As of December 31,				
	2019	2018	2017	2016	2015
Balance sheet data:					
Cash and cash equivalents	\$ 13,561,712	\$ 9,443,667	\$ 4,795,098	\$ 10,316,064	\$ 13,376,196
Total assets	\$ 186,979,004	\$ 63,493,570	\$ 67,006,168	\$ 68,119,098	\$ 98,215,623
Total long-term liabilities(1)	\$ 230,676	\$ 72,591	\$ 96,826	\$ 142,200	\$ 648,190
Accumulated deficit	\$ (365,905,289)	\$ (289,088,064)	\$ (204,275,690)	\$ (135,742,607)	\$ (97,439,602)
Total stockholders’ equity	\$ 163,616,391	\$ 42,202,055	\$ 47,070,429	\$ 57,723,085	\$ 88,111,956

(1) Amount for 2019 primarily reflects the adoption of ASC 842, Leases, on January 1, 2019. Refer to Note 2, “Summary of Significant Accounting Policies,” of the Notes to Consolidated Financial Statements for additional information regarding adoption.

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

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

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

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

Overview

We are a commercial-stage biopharmaceutical company focused on discovering, acquiring, developing and commercializing oncology therapeutics. In December 2018, the U.S. Food and Drug Administration, or FDA, approved our first product, ELZONRIS® (tagraxofusp-erzs), a targeted therapy directed to CD123, for the treatment of adult and pediatric patients, two years and older, with blastic plasmacytoid dendritic cell neoplasm, or BPDCN. ELZONRIS is the first treatment approved for BPDCN and the first approved CD123-directed therapy. ELZONRIS is commercially available for patients with BPDCN in the U.S.

Our pipeline of product candidates includes: Felezonexer (SL-801), SL-1001, SL-901, SL-701 and SL-501.

FDA approved product

ELZONRIS

ELZONRIS, a targeted therapy directed to CD123, was approved by the FDA on December 21, 2018 for the treatment of BPDCN in adult and pediatric patients two years and older, and is commercially available in the United States. The ELZONRIS label contains a boxed warning for capillary leak syndrome, or CLS, which may be life-threatening or fatal and physicians are advised to monitor for signs and symptoms of CLS and take actions as recommended in the full prescribing information.

We are also seeking to broaden the commercial potential of ELZONRIS through ongoing clinical trials in additional indications including chronic myelomonocytic leukemia, or CMML, myelofibrosis, or MF, and acute myeloid leukemia, or AML, and potentially others.

Clinical pipeline product candidates

SL-801

Felezonexor (SL-801) is a structurally novel, oral, small molecule, reversible inhibitor of Exportin-1, or XPO1, a nuclear transport protein implicated in a variety of malignancies. XPO1 is a clinically validated target in oncology, and the FDA recently approved an XPO1 inhibitor in patients with relapsed/refractory multiple myeloma. Felezonexor has demonstrated preclinical in vitro and in vivo antitumor activity against a wide array of solid and hematologic cancers. Felezonexor’s potential ability to reversibly bind XPO1 may offer the possibility to mitigate side effects and help optimize the therapeutic index. We are currently enrolling patients with advanced solid tumors in a Phase 1 dose escalation trial of single agent felezonexor. The dosing regimen for felezonexor was revised in an effort to improve tolerability while maintaining dose intensity, and dosing resumed at 70 mg/day with a new schedule (Schedule B).

In September 2019, we provided an update at the European Society of Medical Oncology, or ESMO, Congress 2019 in Barcelona, Spain. Results from 52 heavily pre-treated solid tumor patients (~90% were third line or greater) with a wide spectrum of solid tumors, including gastrointestinal, breast, lung, neuroendocrine, ovarian, and others were presented. Schedule A was amended to Schedule B in an effort to improve tolerability while maintaining dose intensity. In Schedule B (n=7), grade 3 weakness was experienced. As such, the 75mg cohort was expanded and is being evaluated. The most common TRAEs were nausea (69%), vomiting (53%), fatigue (44%), decreased appetite (24%), and diarrhea (22%), and the most common TRAE, grade 3 or higher, was nausea (9%).

A partial response, or PR, of duration 18+ weeks, was achieved with single agent felezonexor in a fourth line patient with KRAS-positive, microsatellite stable, or MSS, colorectal cancer. The PR (based on RECIST 1.1 criteria) was reported after two cycles of felezonexor (70mg then 65mg due to elevated creatinine), with the patient demonstrating serial reductions in the two target lesions (liver and spleen).

Response and treatment with felezonexor were ongoing at the time of presentation. Stable disease, or SD, was achieved in 12 patients, with 11/12 of these patients third line or greater. Five patients had SD for 4 and 11 months, including 1 patient with basal cell carcinoma with SD for ~11 months, and 20% disease shrinkage was noted in one patient with a heavily pre-treated neuroendocrine tumor.

We believe the ideal therapeutic dose and regimen have yet to be determined. Dose escalation is ongoing, and we intend to provide further updates as the Phase 1 clinical trial continues to enroll.

SL-1001

SL-1001 is an oral, selective small molecule RET (rearranged during transfection) kinase inhibitor. Genetic alterations in the RET kinase have been found in a diverse range of cancers. We believe RET kinase represents a clinically validated target in multiple oncology indications. In March 2019, we in-licensed this preclinical drug candidate from the CRT Pioneer Fund. The molecule was rationally designed by scientists at Cancer Research UK Manchester Institute (United Kingdom), and has demonstrated potent, selective, preclinical anti-cancer activity, both in vitro and in vivo, in RET-driven tumor models. Investigational New Drug, or IND, enabling studies, are ongoing, and we expect to initiate clinical studies of SL-1001 in 2021.

SL-901

SL-901 is an oral, small molecule kinase inhibitor. In December 2017, we in-licensed this drug candidate from UCB Biopharma SPRL. Prior to in-licensing, the agent had demonstrated preclinical activity in several tumor types, and was evaluated in an abbreviated Phase 1 clinical trial in Europe. A PR was reported in one patient with advanced lung cancer. Neither a dose-limiting toxicity nor a maximum tolerated dose was reached in the trial and we believe further dose escalation is possible and warranted. We are currently conducting IMPD enabling work in anticipation of a regulatory re-filing in order to restart clinical trials.

SL-701

SL-701 is an immunotherapy designed to direct the immune system to attack targets present on brain cancer and other malignancies. SL-701 is comprised of several short synthetic peptides that correspond to epitopes of targets including IL-13R α 2, EphA2, and survivin; two of these synthetic peptides (IL-13R α 2 and survivin) are mutant and believed to enhance immune activity. We completed a Phase 2 trial of SL-701 in adult patients with second-line glioblastoma, or GBM. Phase 2 data preliminarily suggest SL-701 is generating target specific CD8+ T-cell responses in patients, which may be translating into improved clinical outcomes, including improved overall survival, or OS, in a subset of patients, which could form the basis of studies. SL-701 was awarded ODD from the FDA for the treatment of glioma in January 2015. We expect to provide further updates relating to this program later this year.

SL-501

SL-501 is a novel CD123-targeted therapy in preclinical development that has demonstrated potency, in vitro and in vivo, against several hematologic tumor types, including AML, CMML, Hodgkin's lymphoma, and non-Hodgkin's lymphoma, or NHL.

Financings

We have devoted substantially all of our resources to the preclinical and clinical development of our products and product candidates, the design and implementation of our regulatory strategy for our Biologics License Application, or BLA, and Marketing Authorization Application, or MAA, filings, preparation for launch and commercialization of our approved product, launching and commercializing our approved product, manufacturing our product and product candidates, strengthening and building our intellectual property portfolio, conducting investor relations, raising capital, providing general and administrative support for these operations, and the execution of our business plan. We have funded our operations primarily through public sales of common stock to our investors. With the U.S. commercial launch of ELZONRIS currently underway, we have recently begun partially funding our operations through net product revenues.

From inception through December 31, 2019, we have received net proceeds of \$446.6 million from the sale of common stock.

On January 18, 2019, we completed an underwritten follow-on public offering of 8,888,889 shares of our common stock, which included the underwriters' exercise in full of the option to purchase 1,333,333 additional shares, at a price of \$9.00 per share. Aggregate gross proceeds from the follow-on public offering, including the exercise of the over-allotment option, were \$92 million, and net proceeds received after underwriting fees and offering expenses were approximately \$86.2 million.

On August 8, 2019, the Company completed an underwritten follow-on public offering, selling 5,000,000 shares at an offering price of \$15.25 per share. Additionally, the underwriters exercised in full their over-allotment option to purchase an additional 750,000 shares at an offering price of \$15.25 per share. Aggregate gross proceeds from the follow-on public offering, including the exercise of the over-allotment option, were \$87.7 million, and net proceeds received after underwriting fees and offering expenses were approximately \$82.2 million.

We have never been profitable and our net loss from operations was \$76.8 million for the year ended December 31, 2019, \$85.0 million for the year ended December 31, 2018, and \$67.8 million for the year ended December 31, 2017. We expect to incur significant expenses and increasing operating losses for the foreseeable future. We expect our expenses to trend higher in connection with our ongoing activities, particularly as we advance our product candidates through preclinical activities and clinical trials to seek regulatory approval and, if approved, commercialize such product candidates. Accordingly, we may need additional financing to support our continuing operations. We will seek to fund our operations through public or private equity or debt financings or other sources. Adequate additional financing may not be available to us on acceptable terms, or at all. Our failure to raise capital as and when needed would have a negative impact on our financial condition and our ability to pursue our business strategy. We will need to generate significant revenues to achieve profitability, and we may never do so.

Litigation

From time to time, we are involved in legal proceedings in the ordinary course of our business. Refer to Note 15: Commitments and Contingencies for more information on legal proceedings.

Financial Operations Overview

Product Revenues

Total revenue consists of net sales of ELZONRIS, which was commercially launched in January 2019. Net product revenue represents the expected consideration to be received from our customers for the sale of ELZONRIS. The expected consideration includes provisions for trade allowances, chargebacks, distribution service fees, product returns and government rebates. Although we expect net sales to increase over time, the provisions for product sales discounts and allowances may fluctuate based on the mix of sales to different customer segments including government customers, rates of product returns, and/or changes in our accrual estimates. Actual amounts of consideration ultimately received may differ from the Company's estimates. If actual results in the future vary from the Company's estimates, the Company will adjust these estimates, which would affect net product revenue and earnings in the period such variances become known.

Cost of Goods Sold

Cost of goods sold consists of all inventory costs related to manufacturing ELZONRIS finished drug product for sale to our customer and royalty fees paid based on product shipments. These costs include expenses incurred by our contract manufacturing organizations, or CMOs, to manufacture drug substance and drug product. Cost of goods sold also includes expenses related to labeling and packaging of drug product, indirect manufacturing overhead and stability testing and provisions for inventory obsolescence. Royalty costs due to the licensor of ELZONRIS are also recorded in cost of goods sold as a period cost.

Research and Development Expenses

The following table shows our research and development expenses for the years ended December 31, 2019, 2018 and 2017:

	Year Ended December 31,		
	2019	2018	2017
ELZONRIS	\$ 23,311,691	\$ 26,126,934	\$ 33,855,807
Other product candidates	5,864,576	3,538,773	3,975,935
Personnel expenses	17,867,830	15,308,877	10,276,948
Other expenses	3,680,314	2,750,435	2,133,696
Total research and development expenses	<u>\$ 50,724,411</u>	<u>\$ 47,725,019</u>	<u>\$ 50,242,386</u>

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

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

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

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

We anticipate that our future research and development expense levels will trend higher in future periods as we continue the preclinical and clinical development of our other product candidates, including ELZONRIS in additional indications and territories.

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

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

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

Selling, General and Administrative Expenses

Selling, general and administrative expenses consist primarily of selling and marketing expenses in support of the commercial launch and personnel cost, including stock-based compensation expense. In addition, our selling, general and administrative expenses are related to legal, finance, human resources, investor relations, and business development. Other general and administrative expenses include facility costs, insurance expense and professional fees for consulting and accounting services.

We anticipate that our selling, general and administrative expenses will be higher in future periods due to continued spend to support the commercialization of ELZONRIS in the United States and a potential commercial launch of ELZONRIS in Europe and additional territories, if marketing approval is obtained outside the U.S.

Interest Income

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

Critical Accounting Policies and Estimates

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

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

Revenue Recognition

Effective January 1, 2018, we adopted Accounting Standards Codification ("ASC") 606. This standard applies to all contracts with customers, except for contracts that are within the scope of other standards, such as leases, insurance, collaboration arrangements and financial instruments. Under ASC 606, an entity recognizes revenue when its customer obtains control of promised goods or services, in an amount that reflects the consideration which the entity expects to receive in exchange for those goods or services. To determine revenue recognition for arrangements that an entity determines are within the scope of ASC 606, the entity performs the following five steps: (i) identify the contract(s) with a customer; (ii) identify the performance obligations in the contract; (iii) determine the transaction price; (iv) allocate the transaction price to the performance obligations in the contract; and (v) recognize revenue when (or as) the entity satisfies a performance obligation. We only apply the five-step model to contracts when it is probable that we will collect the consideration we are entitled to in exchange for the goods or services it transfers to the customer. At contract inception, once the contract is determined to be within the scope of ASC 606, we assess the goods or services promised within each contract and determine those that are performance obligations; and assess whether each promised good or service is distinct. We then recognize as revenue the amount of the transaction price that is allocated to the respective performance obligation when (or as) the performance obligation is satisfied. We have determined that the delivery of our product to our customer constitutes a single performance obligation as there are no other promises to deliver goods or services. Shipping and handling activities are considered to be fulfillment activities and are not considered to be a separate performance obligation. We have assessed the existence of a significant financing component in the agreements with our customers. The trade payment terms with our customers do not exceed one year and therefore, no amount of consideration has been allocated as a financing component. Taxes collected related to product sales are remitted to governmental authorities and are excluded from revenue.

Product Revenue, Net

We have obtained marketing approval from FDA to sell ELZONRIS in the United States market. We sell ELZONRIS in the U.S. to our customer through its title distribution channel. The customer subsequently resells ELZONRIS to a limited number of specialty distributors who, in turn, distributes ELZONRIS to specialty hospitals. We recognize revenue on sales of ELZONRIS when our customer obtains control of the product, which occurs at a point in time (typically upon delivery). Product revenues are recorded at the wholesale acquisition costs, net of applicable reserves for variable consideration. Components of variable consideration include government rebates, product returns, commercial co-payment assistance programs, and distribution service fees. These reserves, as detailed below, are based on the amounts earned, or to be claimed on the related sales, and are classified as reductions of accounts receivable (if the amount is payable to the Customer and right of offset exists) or a current liability (if the amount is payable to a party other than a Customer). Overall, these reserves reflect our best estimates of the amount of consideration against product revenue that has been recognized. The amount of variable consideration which is included in the transaction price may be constrained and is included in the net sales price only to the extent that it is probable that a significant reversal in the amount of the cumulative revenue recognized under contracts will not occur in a future period. Our analyses contemplate the application of the constraint in accordance with ASC 606. Actual amounts of consideration ultimately received may differ from our estimates. If actual results in the future vary from our estimates, we will adjust these estimates, which will affect net product revenue and earnings in the period such variances become known.

Government Contracts

We entered into contracts (i) to participate in the Medicaid Drug Rebate Program and the Medicare Part D program, and (ii) to sell to the U.S. Department of Veterans Affairs, 340b entities, and other government agencies ("Government Payors") so that ELZONRIS will be eligible for purchase by, in partial or full reimbursement from, such Government Payors. These reserves are recorded in the same period the revenue is recognized, resulting in a reduction of product revenue and accounts receivable (if the amount is payable to the Customer) or a current liability (if the amount is payable to a party other than a Customer), which is included in accounts payable and accrued expenses in our consolidated balance sheet. For Medicare Part D, we estimate the number of patients in the prescription drug coverage gap for whom we will owe an additional liability under the Medicare Part D program. We estimate the rebates that will be provided to Government Payors for these programs. These rebate estimates are based upon (i) the government-mandated discounts applicable to government-funded programs, (ii) information obtained from customers and (iii) information obtained from other third parties regarding the payor mix for ELZONRIS. The liability for these rebates consists of estimates of claims for the current year and estimated future claims that will be made for product shipments that have been recognized as revenue but remain in the distribution channel inventories at the end of each reporting period.

Product Returns

Consistent with industry practice, we offer a limited right of return for our product sold. To estimate sales with a right of return, we assess, on a quarterly basis, the number of vials that are held in inventory throughout the distribution channel. Amounts for estimated product returns are established in the same period that the related gross revenue is recognized, resulting in a reduction of product revenue and the establishment of a current liability which is included as a component of accounts payable and accrued expenses in our consolidated balance sheet.

Commercial Co-Payment Assistance Program

We offer co-pay assistance programs which are intended to provide financial assistance to qualified commercially insured patients with prescription drug copayments required by payers. The calculation of the accrual for co-pay assistance is based on an estimate of claims and the cost per claim that we expect to receive associated with product that has been recognized as revenue but remains in the distribution channel at the end of each reporting period. The adjustments are recorded in the same period the related revenue is recognized, resulting in a reduction of product revenue and the establishment of a current liability which is included as a component of accounts payable and accrued expenses in our consolidated balance sheet.

Distribution Fees

Distribution fees include fees paid to our distributors for the distribution of our product based on contractual rates. In addition, we compensate for data and other administrative activities. Therefore, estimates for these costs are recorded as a reduction of revenue, based on contractual terms.

Inventory

We capitalize inventory costs associated with the manufacturing of ELZONRIS after regulatory approval or when, based on management's judgment, future commercialization is considered probable and the future economic benefit is expected to be realized. Otherwise, such costs are expensed as research and development. We value our inventories at the lower of cost or estimated net realizable value. We determine the cost of our inventories, which includes amounts related to materials and manufacturing overhead, on a first-in, first-out basis. We perform an assessment of the recoverability of capitalized inventory during each reporting period and write down any excess and obsolete inventories to their estimated realizable value in the period in which the impairment is first identified. Such impairment charges, should they occur, are recorded within cost of goods sold. The determination of whether inventory costs will be realizable requires estimates by management. If actual market conditions are less favorable than projected by management, additional write-downs of inventory may be required which would be recorded as a cost of goods sold in the consolidated statement of operations.

Accrued Research and Development Expenses

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

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

Income Taxes

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

We continue to assess our ability to realize our deferred tax assets, which primarily consist of net operating losses, or NOL, carry-forwards. In assessing our ability to realize these deferred tax assets, management considers whether it is more likely than not that some portion or all of the deferred tax assets will be realized. We establish valuation allowances when necessary to reduce deferred tax assets to the amounts expected to be realized. The factors used to assess the likelihood of realization include our latest forecast of future taxable income and available tax planning strategies that could be implemented to realize the net deferred tax assets. As of December 31, 2019, 2018 and 2017, our deferred tax assets had full valuation allowances on them as we did not have sufficient positive evidence to recognize such deferred tax assets.

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

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

As of December 31, 2019, we had net operating losses of \$284.7 million for federal and \$288.5 million for state purposes and research and development credits of \$39.0 million which expire in 2023 through 2038.

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

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

During the fourth quarter of 2019, we completed our analysis and determined no additional changes were necessary to the previously recorded provisional amounts.

Stock-Based Compensation

We account for stock-based compensation in accordance with the provisions of ASC 718, *Compensation—Stock Compensation*. Accordingly, compensation costs related to equity instruments granted are recognized over the requisite service periods of the awards on a straight-line basis at the grant-date fair value calculated by either using a Black-Scholes option pricing model for stock option valuations or the closing stock price on date of grant for restricted stock. On June 20, 2018, the FASB issued ASU No. 2018- 07, *Compensation — Stock Compensation (Topic 718): Improvements to Nonemployee Share-Based Payment Accounting*, which expands the scope of Topic 718 to include share-based payment transactions for acquiring goods and services from nonemployees. The Company early adopted ASU No. 2018-07 on April 1, 2018 and the net impact relating to the adoption was a \$0.2 million decrease to accumulated deficit for the impact prior to April 1, 2018.

Results of Operations

Comparison of Years Ended December 31, 2019 and 2018

Product revenue, net: We began commercial sales of ELZONRIS in January 2019. For the year ended December 31, 2019, we recorded \$43.2 million of net product revenue. We had no product revenue during the year ended December 31, 2018.

Costs of goods sold: Cost of goods sold for the year ended December 31, 2019 was \$4.9 million and relates to an increase in the reserve for inventory obsolescence, resulting from extra ELZONRIS inventory that was previously manufactured to ensure there were no supply shortages, manufacturing costs for ELZONRIS drug product, stability testing on drug product inventory and royalties owed to the licensor of ELZONRIS.

Research and development expense: Research and development expense was \$50.7 million for the year ended December 31, 2019, compared with \$47.7 million for the year ended December 31, 2018, which represents an increase of \$3.0 million. The higher costs were primarily due to an increase in stock-based compensation expenses, achievement of the LLS milestone due to the first commercial sale of ELZONRIS in the first quarter of 2019, and in-licensing costs for SL-1001. Partially offsetting the increased costs were lower expenses during 2019 versus the prior year primarily attributable to higher prior year regulatory-related costs relating to supporting the BLA filing for ELZONRIS in the U.S. coupled with manufacturing of ELZONRIS drug product during 2018 prior to FDA approval, which was expensed as incurred.

Selling, general and administrative expense: Selling, general and administrative expenses were \$66.8 million for the year ended December 31, 2019, compared with \$39.1 million for the year ended December 31, 2018, which represents an increase of \$27.7 million. The increase in costs were primarily attributable to compensation cost of \$17.8 million primarily related to an increase in headcount and other ongoing launch expenses in support of the commercialization of ELZONRIS in the U.S., and pre-launch commercial costs in support of the potential approval and commercialization of ELZONRIS in the EU.

Interest income: Interest income was \$2.5 million for the year ended December 31, 2019, compared with \$1.3 million for the year ended December 31, 2018, representing an increase of \$1.2 million. The increase was primarily due to higher average cash and investment balances during 2019 versus the same period in the prior year resulting primarily from two underwritten follow-on public offerings of stock during 2019 which raised net cash proceeds of \$168.4 million.

Comparison of Years Ended December 31, 2018 and 2017

Research and development expense: Research and development expense was \$47.7 million for the year ended December 31, 2018, compared with \$50.2 million for the year ended December 31, 2017, which represents a decrease of \$2.5 million. The lower costs were primarily driven by the wind-down and completion of the pivotal clinical trial for ELZONRIS partially offset by an increase in regulatory expenses to support the BLA filing for ELZONRIS.

Selling general and administrative expense: Selling, general and administrative expenses were \$39.1 million for the year ended December 31, 2018, compared with \$19.2 million for the year ended December 31, 2017, which represents an increase of \$19.9 million. The higher costs were primarily attributable to a \$16.5 million increase in pre-launch expenses in support of preparing for commercialization of ELZONRIS in BPDCN. Additionally, the higher expense was also due to a \$3.1 million increase in non-cash stock compensation expense due in part to an increase in headcount. We expect commercial related expense will increase for the foreseeable future at a more modest rate to support the commercial launch of ELZONRIS.

Interest income: Interest income was \$1.3 million for the year ended December 31, 2018, compared with \$0.7 million for the year ended December 31, 2017. The increase in income of \$0.6 million is primarily due to higher market interest rates earned on our investment balances.

Liquidity and Capital Resources

Sources of Liquidity

We have financed our operations to date primarily through proceeds from public sales of common stock via our 2013 IPO and subsequent follow-on public stock offerings. With the FDA marketing approval for ELZONRIS received in December 2018, we have generated \$43.2 million of net product revenue from the sale of the product. We have incurred losses and generated negative cash flows from operations since inception.

Since inception and through December 31, 2019, we received net proceeds of \$446.6 million primarily from the public sale of common stock from our 2013 IPO and subsequent follow-on public offerings. On January 18, 2019, we completed an underwritten follow-on public offering of 8,888,889 shares of our common stock, which included the underwriters' exercise in full of the option to purchase 1,333,333 additional shares, at a price of \$9.00 per share. Aggregate gross proceeds from the follow-on public offering, including the exercise of the over-allotment option, were \$92 million, and net proceeds received after underwriting fees and offering expenses were approximately \$86.2 million. On August 8, 2019, we completed a follow-on public offering, selling 5,000,000 shares at an offering price of \$15.25 per share. Additionally, the underwriters exercised in full their over-allotment option to purchase an additional 750,000 shares at an offering price of \$15.25 per share. Aggregate gross proceeds from the follow-on public offering, including the exercise of the over-allotment option, were \$87.7 million, and net proceeds received after underwriting fees and offering expenses were approximately \$82.2 million.

As of December 31, 2019, our cash, cash equivalents and short-term investments totaled \$164.4 million. We primarily invest our cash, cash equivalents, short-term investments in U.S. Treasury and Agency securities and related money market funds and FDIC-insured bank certificates of deposit, with the balance in commercial bank operating accounts. We believe that our existing cash, cash equivalents, short-term investments and long-term investments including cash proceeds received from our two follow-on offerings in 2019 will be sufficient to fund our operations for at least the next two years.

Cash Flows

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

	Year Ended December 31,		
	2019	2018	2017
Net cash used in operating activities	\$ (67,689,973)	\$ (72,181,918)	\$ (49,618,794)
Net cash (used in) provided by investing activities	(99,191,629)	10,796,584	(4,437,050)
Net cash provided by financing activities	170,999,647	66,033,903	48,534,878
Net increase (decrease) in cash and cash equivalents	<u>\$ 4,118,045</u>	<u>\$ 4,648,569</u>	<u>\$ (5,520,966)</u>

Operating activities. The use of cash in all periods resulted primarily from our net losses adjusted for stock-based compensation expense, non-cash depreciation expense and changes in the components of working capital. The net cash used in operating activities in 2019, 2018, and 2017 primarily resulted from research and development expenses as we continued our clinical trial activities relating to ELZONRIS, SL-801, and SL-701, as well as for preclinical work related to SL-1001 and SL-901. Additional research and development costs also include CMC-related expenses for the manufacture of drug substance and drug product of our product candidates in development. We also utilized a significant level of cash from our operating activities to support our commercial organization with selling and marketing activities relating to the launch of ELZONRIS in the U.S. in January 2019.

Investing activities. The net cash provided by and used in investing activities for 2019, 2018, and 2017 reflects purchases and redemptions of short-term and long-term investments within our U.S. Treasury-related investment and bank certificate of deposit portfolios, net of maturities.

Financing activities. The net cash provided by financing activities for 2019 resulted primarily from two follow-on offerings. In January 2019, we completed our fifth follow-on offering resulting in the issuance and sale of 10,222,222 shares of our common stock, which included the exercise in full of the option to purchase 1,333,333 additional shares, which generated gross cash proceeds of \$92.0 million, and net proceeds received after underwriting fees and offering expenses were approximately \$86.2 million. On August 8, 2019, we completed our sixth follow-on public offering, selling 5,000,000 shares of our common stock, which at included the exercise in full of the option to purchase an additional 750,000 shares, which generated gross cash proceeds of \$87.7 million, and net proceeds received after underwriting fees and offering expenses were approximately \$82.2 million.

The net cash provided by financing activities for 2018 resulted primarily from our January 2018 issuance and sale of 4,255,000 shares of our common stock, which included the exercise in full of the option to purchase 555,000 additional shares, which generated gross cash proceeds of \$59.6 million (\$55.7 million cash proceeds, net of expenses). In addition, for the year ended December 31, 2018, we received net proceeds of \$8.3 million from an At-the-Market offering, selling 442,579 shares at an average price of \$19.24 per share. The net cash provided by financing activities for 2017 resulted primarily from our January 2017 issuance and sale of 5,175,000 shares of our common stock which generated gross cash proceeds of \$51.8 million (\$48.2 million cash proceeds, net of expenses). The net cash provided by financing activities for 2019, 2018 and 2017 was also impacted by the issuance of stock related to the 2016 Employee Stock Purchase Plan and exercise of employee and consultant stock options.

Funding Requirements

Our product and product candidates are in clinical or preclinical development, including ELZONRIS for CMML, MF, AML, and potentially other indications, as well as SL-801, SL-1001, SL-901, and SL-701 at various stages of development. We expect to continue to incur significant expenses for the foreseeable future. We anticipate that our expenses will increase if and as we:

- continue the ongoing clinical trials, and initiate additional clinical trials, of our product candidates;
- devise and implement our regulatory strategy, including for our regulatory filings in the U.S. and abroad;
- manufacture alternative formulations of ELZONRIS drug product;
- continue the research and development of ELZONRIS in additional indications and our other product candidates;
- seek to identify additional product candidates;
- acquire or in-license other products and technologies;
- seek marketing approvals for ELZONRIS in additional indications and territories, and seek marketing approvals for our other product candidates should they successfully complete pre-market clinical trials;
- establish, either on our own or with strategic partners, a manufacturing, sales, marketing and distribution infrastructure to commercialize any products for which we may obtain marketing approval;
- continue to incur legal expenses relating to litigation;
- maintain, fortify, leverage and expand our intellectual property portfolio; and
- add and augment operational, financial and management information systems and related personnel, including personnel to support our product development and future global commercialization efforts.

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

- the progress and results of the ongoing and future clinical trials of our product candidates;
- the costs of commercialization activities, present and future, including product sales promotion, marketing, manufacturing and distribution, for our approved product or future additional regulatory approvals;
- the scope, progress, results and costs of research and development, preclinical development, laboratory testing and clinical trials for our product candidates now or in the future;
- the extent to which we acquire or in-license other products and technologies;
- the costs, timing of regulatory preparation and outcome of regulatory review, in the U.S. or abroad, of our product or product candidates;

- income, if any, received from commercial sales of our product or product candidates, should our approved product be approved in additional indications or territories or any of our product candidates, beyond our currently approved product, receive marketing approval;
- the cost of litigation with third parties, if any;
- the costs of preparing, filing and prosecuting patent applications, maintaining and enforcing our intellectual property rights and defending intellectual property-related claims; and
- our ability to establish any future collaboration arrangements on favorable terms, if at all.

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

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

Expected Cash Requirements for Contractual Obligations

The following table presents our expected cash requirements for contractual obligations for each of the following years subsequent to December 31, 2019:

Contractual Obligations and Commitments

The following table summarizes our contractual obligations at December 31, 2019:

	Total	Less than 1 year	1-3 years	3-5 years	More than 5 years
Operating lease obligations (1)	\$ 1,473,101	\$ 1,227,123	\$ 245,978	\$ —	\$ —
Clinical trial CRO obligations (2)	3,308,742	3,308,742	—	—	—
Bioprocessing Contracts (3)	13,649,895	7,649,895	4,575,000	1,425,000	—
License agreements (4)	3,743,120	1,559,555	958,998	474,827	749,740
Regulatory Contracts (5)	1,672,826	1,672,826	—	—	—
Commercial Contracts (6)	1,564,695	1,564,695	—	—	—
Collaboration agreement (7)	1,533,719	1,533,719	—	—	—
Other commitments (8)	2,463,040	2,355,159	107,881	—	—
Total	\$ 29,409,138	\$ 20,871,714	\$ 5,887,857	\$ 1,899,827	\$ 749,740

- (1) Operating lease obligations reflect our lease agreement with respect to our corporate offices at 750 Lexington Avenue, New York, New York, for a monthly rent of \$69,750. The original term of this lease agreement was 42 months and it was set to expire on December 31, 2019. We exercised an option to extend this lease agreement for an additional 12 months to December 31, 2020. In July 2018, we entered into a new leasing agreement for additional office space at 750 Lexington Ave, New York, New York, for a monthly rent of \$23,400. The term of this lease agreement is on a month to month basis. Finance lease obligation is not material and, is therefore, excluded from this amount.

On March 3, 2020, the Company entered into a lease agreement for office space located at 750 Lexington Avenue, Fourth Floor, New York, New York, with lease obligations of approximately \$9.0 million. The commencement date of the lease is being finalized but will not be before May 1, 2020. The lease will expire in 2028. The Company expects to classify the lease as an operating lease. Because the lease had not yet commenced as of December 31, 2019, payments related to this lease are not included in the above table.

- (2) We have agreements in place with various contract research organizations (CROs) to facilitate research, clinical and data management services in connection with our clinical-stage product candidates.
- (3) Includes commitments to our third-party drug substance and drug product manufacturers. Also includes contractual obligations for stability testing on drug substance and drug product inventory.
- (4) We have executed several license agreements. Other than the payments noted in the table above, milestone and royalty payments associated with licensing have not been included as management cannot reasonably estimate if or when they will occur. These agreements include the following:
 - Under a research and license agreement with Scott and White Hospital for ELZONRIS, we are required to pay royalties on annual sales of licensed products.
 - Under three separate license agreements with the University of Pittsburgh, we are required to make aggregate development and regulatory milestone payments associated with SL-701 and pay royalties on net sales of licensed products.
 - Under an exclusive patent and non-exclusive know-how license agreement with the Cambridge University Technical Services Limited, related to our StemScreen® platform technology, we are required to make milestone payments upon specified regulatory events and pay royalties on sales of licensed products.
 - On December 26, 2014, we entered into a license agreement with CanBas, Ltd for SL-801. SL-801 is a small molecule, reversible inhibitor of XPO1. Under the terms of the agreement, CanBas has granted us an exclusive, royalty-bearing, worldwide license, under certain patent rights, know-how and materials to research, develop, make, have made, formulate, use, sell, offer to sell and import SL-801, and any products containing or comprising such compound in finished dosage pharmaceutical form, for the treatment of any disease or condition in humans. We are required to make milestone payments upon the achievement of various clinical development, regulatory and commercial milestones. Additionally, we are required to pay tiered royalties on net sales of licensed products.
 - On December 31, 2017, we entered into a license agreement with UCB Biopharma Sprl (“UCB”) for SL-901. SL-901 is a small molecule kinase inhibitor. UCB has granted us an exclusive, royalty-bearing, worldwide license, under certain patent rights, know-how and materials to research, develop, make, have made, formulate, use, sell, offer to sell and import SL-901, and any products containing or comprising such compound in finished dosage pharmaceutical form. We may be responsible, based on the achievement of specific clinical development, regulatory and sales-based commercial milestones, for certain payments to UCB of up to \$111 million, largely consisting of approval and post-approval payments. Additionally, we may be obligated to pay UCB tiered royalties based on aggregate net sales of products.
 - On March 11, 2019, we entered into a license agreement with the CRT Pioneer Fund LP, or CRT, for SL-1001. SL-1001 is a small molecule RET kinase inhibitor. CRT has granted us an exclusive, royalty-bearing, worldwide license, under certain patent rights, know-how and materials to research, develop, make, have made, formulate, use, sell, offer to sell and import SL-1001, and any products containing or comprising such compound in finished dosage pharmaceutical form. We may be responsible, based on the achievement of specific clinical development, regulatory and sales-based commercial milestones, for certain payments to CRT of up to \$159 million, largely consisting of approval and post-approval payments. Additionally, we may be obligated to pay CRT tiered royalties based on aggregate net sales of products.

- (5) We have agreements in place with various regulatory consultants to assist us with our MAA filing for ELZONRIS.
- (6) We have an agreement in place with a marketing related agency to assist us in the ongoing commercial launch of ELZONRIS.
- (7) In October 2013, we entered into a contract relating to the Therapy Acceleration Program (“the TAP Agreement”) with The Leukemia and Lymphoma Society (“LLS”). LLS is a national voluntary health agency, which, among other activities, encourages and sponsors research relating to blood cancers in order to develop therapies to cure or mitigate these diseases. Pursuant to the TAP Agreement, LLS agreed to provide funding to us, not to exceed \$3.5 million, to support our preclinical and clinical development activities as well as providing an educational program to increase physician & patient awareness of BPDCN. Through December 31, 2019, we have received the full \$3.5 million based on milestones achieved. For the year ended December 31, 2019, we have paid \$2.9 million and a final payment of \$1.5 million in January 2020, pursuant to the agreement as funding for the education program to increase awareness of BPDCN. We are also required to make a one-time payment upon regulatory approval and commercialization of ELZONRIS. Additionally, the Company is required to make payments based on achievement of specific sale-based commercial milestones.
- (8) Other commitments include certain sponsored research.

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

Off-Balance Sheet Arrangements

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

Tax Loss Carryforwards

As of December 31, 2019, we had net operating losses of \$284.7 million for federal and \$288.5 million state purposes, which are available to reduce future taxable income. We also had federal tax credits of approximately \$39.0 million, which may be used to offset future tax liabilities. The net operating loss and tax credit carryforwards will expire at various dates through 2038. Net operating loss and tax credit carryforwards are subject to review and possible adjustment by the Internal Revenue Service and state tax authorities and may become subject to an annual utilization limitation pursuant to the change in ownership rules of Internal Revenue Code Section 382 and 383. The amount of the annual limitation is determined based on the value of our company immediately prior to the ownership change. Subsequent ownership changes may further affect the limitation in future years. At December 31, 2019, we recorded a 100% valuation allowance against our net operating loss and tax credit carryforwards, as we believe it is more likely than not that the tax benefits will not be fully realized.

Recently Adopted Accounting Standards

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

Item 7A. Quantitative and Qualitative Disclosures about Market Risk

We are exposed to market risk related to changes in interest rates. We had cash, cash equivalents, short-term investments and long-term investments of \$164.4 million as of December 31, 2019, \$60.1 million as of December 31, 2018, and \$66.2 million as of December 31, 2017, consisting of cash, U. S. Treasury and Agency securities and Treasury-related money market funds and FDIC-insured bank certificates of deposit. Our primary exposure to market risk is interest rate sensitivity, which is affected by changes in the general level of U.S. interest rates, particularly because our investments are primarily in Treasury-related debt securities and bank certificates of deposit. Our available for sale securities are subject to interest rate risk and will fall in value if market interest rates increase. Due to the short-term duration of our investment portfolio and the low risk profile of our investments, an immediate 100 basis point change in interest rates would not have a material effect on the fair market value of our portfolio.

We contract with CROs and CMOs. We may be subject to fluctuations in foreign currency rates in connection with these agreements. Transactions denominated in currencies other than the functional currency are recorded based on exchange rates at the time such transactions arise. As of December 31, 2019, 2018, and 2017, all of our liabilities were denominated in our functional currency.

Item 8. Consolidated Financial Statements and Supplementary Data

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

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

None.

Item 9A. Controls and Procedures

Evaluation of Disclosure Controls and Procedures

Our management, with the participation of our Chief Executive Officer (principal executive officer) and Chief Accounting Officer (principal financial officer), evaluated the effectiveness of our disclosure controls and procedures as of December 31, 2019. The term “disclosure controls and procedures”, as defined in Rules 13a-15I and 15d-15(e) under the Exchange Act, means controls and other procedures of a company that are designed to ensure that information required to be disclosed by a company in the reports that it files or submits under the Exchange Act is recorded, processed, summarized and reported, within the time periods specified in the SEC’s rules and forms. Disclosure controls and procedures include, without limitation, controls and procedures designed to ensure that information required to be disclosed by a company in the reports that it files or submits under the Exchange Act is accumulated and communicated to the company’s management, including its principal executive and principal financial officers, as appropriate to allow timely decisions regarding required disclosure.

Management recognizes that any controls and procedures, no matter how well designed and operated, can provide only reasonable assurance of achieving their objectives and management necessarily applies its judgment in evaluating the cost-benefit relationship of possible controls and procedures. Based on the evaluation of our disclosure controls and procedures as of December 31, 2019, our Chief Executive Officer and Chief Accounting Officer concluded that, as of such date, our disclosure controls and procedures were effective.

Management’s Report on Internal Control over Financial Reporting

Our management is responsible for establishing and maintaining adequate internal control over financial reporting for our company. Internal control over financial reporting is defined in Rule 13a-15(f) or 15d-15(f) promulgated under the Exchange Act as a process designed by, or under the supervision of, the company’s principal executive and principal financial officers and effected by the company’s board of directors, management and other personnel, to provide reasonable assurance regarding the reliability of financial reporting and the preparation of financial statements for external purposes in accordance with GAAP and includes those policies and procedures that: (i) pertain to the maintenance of records that, in reasonable detail, accurately and fairly reflect the transactions and dispositions of the assets of the company; (ii) provide reasonable assurance that transactions are recorded as necessary to permit preparation of financial statements in accordance with generally accepted accounting principles, and that receipts and expenditures of our company are being made only in accordance with authorizations of management and directors of the company; and (iii) provide reasonable assurance regarding prevention or timely detection of unauthorized acquisition, use, or disposition of our company’s assets that could have a material effect on the financial statements.

Internal control over financial reporting is designed to provide reasonable assurance regarding the reliability of financial reporting and the preparation of financial statements prepared for external purposes in accordance with generally accepted accounting principles. Because of its inherent limitations, internal control over financial reporting may not prevent or detect misstatements. Also, projections of any evaluation of effectiveness to future periods are subject to the risk that controls may become inadequate because of changes in conditions, or that the degree of compliance with the policies or procedures may deteriorate.

Our management assessed the effectiveness of our internal control over financial reporting as of December 31, 2019. In making this assessment, our management used the criteria set forth in the *Internal Control—Integrated Framework (2013)* issued by the Committee of Sponsoring Organizations of the Treadway Commission. Based on its assessment, management (including our Chief Executive Officer and our Chief Financial Officer) concluded that our internal control over financial reporting was effective as of December 31, 2019 based on those criteria.

Changes in Internal Control over Financial Reporting

There were no changes in the Company’s internal control over financial reporting during the fourth quarter of 2019, which were identified in connection with management’s evaluation required by paragraph (d) of Rules 13a-15 and 15d-15 under the Exchange Act, that have materially affected, or are reasonably likely to materially affect, the Company’s internal control over financial reporting.

Attestation Report of Registered Public Accounting Firm

The effectiveness of our internal controls over financial reporting as of December 31, 2019 has been audited by our independent registered accounting firm, Ernst & Young LLP, as stated in their attestation report.

Report of Independent Registered Public Accounting Firm

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

Opinion on Internal Control Over Financial Reporting

We have audited Stemline Therapeutics, Inc.'s internal control over financial reporting as of December 31, 2019, based on criteria established in Internal Control-Integrated Framework issued by the Committee of Sponsoring Organizations of the Treadway Commission (2013 framework), (the COSO criteria). In our opinion, Stemline Therapeutics, Inc. (the Company) maintained, in all material respects, effective internal control over financial reporting as of December 31, 2019, based on the COSO criteria.

We also have audited, in accordance with the standards of the Public Company Accounting Oversight Board (United States) (PCAOB), the 2019 consolidated financial statements of the Company and our report dated March 16, 2020 expressed an unqualified opinion thereon.

Basis for Opinion

The Company's management is responsible for maintaining effective internal control over financial reporting and for its assessment of the effectiveness of internal control over financial reporting included in the accompanying Management's Report on Internal Control Over Financial Reporting. Our responsibility is to express an opinion on the Company's internal control over financial reporting based on our audit. We are a public accounting firm registered with the PCAOB and are required to be independent with respect to the Company in accordance with U.S. the federal securities laws and the applicable rules and regulations of the Securities and Exchange Commission and the PCAOB.

We conducted our audit in accordance with the standards of the PCAOB. Those standards require that we plan and perform the audit to obtain reasonable assurance about whether effective internal control over financial reporting was maintained in all material respects.

Our audit included obtaining an understanding of internal control over financial reporting, assessing the risk that a material weakness exists, testing and evaluating the design and operating effectiveness of internal control based on the assessed risk, and performing such other procedures as we considered necessary in the circumstances. We believe that our audit provides a reasonable basis for our opinion.

Definition and Limitations of Internal Control Over Financial Reporting

A company's internal control over financial reporting is a process designed to provide reasonable assurance regarding the reliability of financial reporting and the preparation of financial statements for external purposes in accordance with generally accepted accounting principles. A company's internal control over financial reporting includes those policies and procedures that (1) pertain to the maintenance of records that, in reasonable detail, accurately and fairly reflect the transactions and dispositions of the assets of the company; (2) provide reasonable assurance that transactions are recorded as necessary to permit preparation of financial statements in accordance with generally accepted accounting principles, and that receipts and expenditures of the company are being made only in accordance with authorizations of management and directors of the company; and (3) provide reasonable assurance regarding prevention or timely detection of unauthorized acquisition, use, or disposition of the company's assets that could have a material effect on the financial statements.

Because of its inherent limitations, internal control over financial reporting may not prevent or detect misstatements. Also, projections of any evaluation of effectiveness to future periods are subject to the risk that controls may become inadequate because of changes in conditions, or that the degree of compliance with the policies or procedures may deteriorate.

/s/ Ernst & Young LLP

Stamford, Connecticut
March 16, 2020

Changes in Internal Control over Financial Reporting

No change in our internal control over financial reporting occurred during the quarter ended December 31, 2019 that has materially affected, or is reasonably likely to materially affect, our internal control over financial reporting.

Item 9B. Other Information

None.

Part III

Item 10. Directors, Executive Officers and Corporate Governance

The information required by this Item is incorporated herein by reference from our Proxy Statement for our 2020 Annual Meeting of Stockholders.

Item 11. Executive Compensation

The information required by this Item is incorporated herein by reference from our Proxy Statement for our 2020 Annual Meeting of Stockholders.

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

The information required by this Item is incorporated herein by reference from our Proxy Statement for our 2020 Annual Meeting of Stockholders.

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

The information required by this Item is incorporated herein by reference from our Proxy Statement for our 2020 Annual Meeting of Stockholders.

Item 14. Principal Accounting Fees and Services.

The information required by this Item is incorporated herein by reference from our Proxy Statement for our 2020 Annual Meeting of Stockholders.

Part IV

Item 15. Exhibits, Financial Statements Schedules.

(a) 1. Financial Statements

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

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2. Financial Statement Schedules

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

3. Exhibits

Exhibit No.	Description
3.1	Restated Certificate of Incorporation of Stemline Therapeutics, Inc., filed as Exhibit 3.1 to Form 8-K on February 6, 2013 (File No. 001-35619) and incorporated herein by reference.
3.2	Certificate of Amendment of Restated Certificate of Incorporation of Stemline Therapeutics, Inc., filed as Exhibit 3.2 to Form 10-Q on August 8, 2017 (File No. 001-35619) and incorporated herein by reference.
3.3	Amended and Restated Bylaws of Stemline Therapeutics, Inc., filed as Exhibit 3.2 to Form 8-K on February 6, 2013 (File No. 001-35619) and incorporated herein by reference.
3.4	Certificate of Amendment of Restated Certificate of Incorporation of Stemline Therapeutics, Inc., filed as Exhibit 3.3 to Form 10-Q on August 14, 2013 (File No. 001-35619) and incorporated herein by reference.

Exhibit No.	Description
<u>3.5</u>	<u>Certificate of Amendment of Restated Certificate of Incorporation of Stemline Therapeutics, Inc., filed as Exhibit 3.1 to Form 8-K on June 28, 2019 (File No. 001-35619) and incorporated herein by reference.</u>
<u>4.1</u>	<u>Specimen certificate evidencing shares of common stock, filed as Exhibit 4.1 to Form S-1/A on June 20, 2012 (File No. 333-180515) and incorporated herein by reference.</u>
<u>4.2</u>	<u>Form of Representative's Warrant Agreement, filed as Exhibit 4.2 to Form S-1/A on November 14, 2012 (File No. 333-180515) and incorporated herein by reference.</u>
<u>4.3</u>	<u>Description of Securities of Stemline Therapeutics, Inc.</u>
<u>10.1†</u>	<u>Research and License Agreement by and among the Company, Scott and White Memorial Hospital, Scott, Sherwood and Brindley Foundation and Arthur E. Frankel, M.D., dated June 15, 2006; as amended by that certain First Amendment to Research and License Agreement dated December 9, 2008, that certain Second Amendment to Research and License Agreement dated March 17, 2010 and that certain Third Amendment to Research and License Agreement dated July 12, 2011, filed as Exhibit 10.1 to Form S-1/A on June 20, 2012 (File No. 333-180515) and incorporated herein by reference.</u>
<u>10.2†</u>	<u>Exclusive License Agreement between the Company and the University of Pittsburgh, dated September 30, 2009, filed as Exhibit 10.2 to Form S-1/A on June 20, 2012 (File No. 333-180515) and incorporated herein by reference.</u>
<u>10.3†</u>	<u>Exclusive Patent and Non-Exclusive Know-How License Agreement between the Company and Cambridge University Technical Services Limited, commenced September 16, 2004, filed as Exhibit 10.3 to Form S-1/A on June 20, 2012 (File No. 333-180515) and incorporated herein by reference.</u>
<u>10.4†</u>	<u>Non-Exclusive License Agreement between the Company and the University of Pittsburgh, dated March 30, 2012, filed as Exhibit 10.4 to Form S-1/A on June 20, 2012 (File No. 333-180515) and incorporated herein by reference.</u>
<u>10.5†</u>	<u>Non-Exclusive License Agreement between the Company and the University of Pittsburgh, dated March 21, 2012, filed as Exhibit 10.5 to Form S-1 on April 2, 2012 (File No. 333-180515) and incorporated herein by reference.</u>
<u>10.6*</u>	<u>Employment Agreement, dated June 15, 2012, between the Registrant and Ivan Bergstein, M.D., filed as Exhibit 10.8 to Form S-1/A on June 20, 2012 (File No. 333-180515) and incorporated herein by reference.</u>
<u>10.7*</u>	<u>Form of Indemnification Agreement between the Registrant and each director, filed as Exhibit 10.9 to Form S-1/A on June 20, 2012 (File No. 333-180515) and incorporated herein by reference.</u>
<u>10.8*</u>	<u>Amended and Restated 2004 Employee, Director and Consultant Stock Plan, filed as Exhibit 10.10 to Form S-1 on April 2, 2012 (File No. 333-180515) and incorporated herein by reference.</u>
<u>10.9*</u>	<u>Form of Incentive Stock Option Agreement under Amended and Restated 2004 Employee, Director and Consultant Stock Plan, filed as Exhibit 10.11 to Form S-1 on April 2, 2012 (File No. 333-180515) and incorporated herein by reference.</u>

Exhibit No.	Description
<u>10.10*</u>	<u>Form of Non-qualified Stock Option Agreement under Amended and Restated 2004 Employee, Director and Consultant Stock Plan, filed as Exhibit 10.12 to Form S-1 on April 2, 2012 (File No. 333-180515) and incorporated herein by reference.</u>
<u>10.11*</u>	<u>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.</u>
<u>10.12*</u>	<u>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.</u>
<u>10.13*</u>	<u>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.</u>
<u>10.14</u>	<u>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.</u>
<u>10.15</u>	<u>Assignment Agreement between the Company and Ivan Bergstein, M.D., effective as of June 15, 2012, filed as Exhibit 10.20 to Form S-1/A on June 20, 2012 (File No. 333-180515) and incorporated herein by reference.</u>
<u>10.16*</u>	<u>Offer Letter between the Company and Kenneth Zuerblis, dated March 8, 2012, filed as Exhibit 10.23 to Form S-1/A on June 20, 2012 (File No. 333-180515) and incorporated herein by reference.</u>
<u>10.17</u>	<u>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.</u>
<u>10.18*</u>	<u>Employment Agreement between the Company and David G. Gionco, dated January 16, 2014, filed as Exhibit 10.1 to Form 8-K on January 23, 2014 (File No. 001-35619) and incorporated herein by reference.</u>
<u>10.19†</u>	<u>License Agreement by and between Stemline Therapeutics, Inc. and the CanBas Co., Ltd, dated December 26, 2014, filed as Exhibit 10.30 to Form 10-K on March 16, 2015 (File No. 001-35619) and incorporated herein by reference.</u>
<u>10.20*</u>	<u>Amendment No.1 to the 2012 Equity Incentive Plan, adopted March 13, 2015, filed as Exhibit 10.31 to Form 10-K on March 16, 2015 (File No. 001-35619) and incorporated herein by reference.</u>
<u>10.21*</u>	<u>Amendment No.1 to the Amended and Restated 2004 Employee, Director and Consultant Stock Plan, adopted March 13, 2015, filed as Exhibit 10.32 to Form 10-K on March 16, 2015 (File No. 001-35619) and incorporated herein by reference.</u>
<u>10.22*</u>	<u>Employment Agreement between the Company and Kenneth Hoberman, dated January 7, 2016, filed as Exhibit 10.1 to Form 8-K on January 13, 2016 (File No. 001-35619) and incorporated herein by reference.</u>

Exhibit No.	Description
10.23	2016 Equity Incentive Plan, adopted May 25, 2016, filed as Appendix A to the Company's Definitive Proxy Statement on April 8, 2016 (File No. 001-35619) and incorporated herein by reference.
10.24	Amendment No. 1 to the 2016 Equity Incentive Plan, adopted June 20, 2017, filed as Exhibit A to the Company's Definitive Proxy Statement on May 1, 2017 (File No. 001-35619) and incorporated herein by reference.
21.1	List of subsidiaries of Stemline Therapeutics, Inc.
23.1	Consent of Ernst & Young LLP.
24.1	Power of Attorney (included on signature page).
31.1	Certificate of principal executive officer pursuant to Rule 13a-14(a)/15d-14(a), as adopted pursuant to Section 302 of the Sarbanes-Oxley Act of 2002.
31.2	Certificate of principal financial officer pursuant to Rule 13a-14(a)/15d-14(a), as adopted pursuant to Section 302 of the Sarbanes-Oxley Act of 2002.
32.1	Certificate of principal executive officer pursuant to 18 U.S.C. § 1350, as adopted pursuant to Section 906 of the Sarbanes-Oxley Act of 2002.
32.2	Certificate of principal financial officer pursuant to 18 U.S.C. § 1350, as adopted pursuant to Section 906 of the Sarbanes-Oxley Act of 2002.
101	The following financial information from Stemline Therapeutics, Inc.'s Annual Report on Form 10-K for the year ended December 31, 2019, formatted in XBRL (eXtensible Business Reporting Language): (i) Consolidated Balance Sheets, (ii) Consolidated Statements of Operations, (iii) Consolidated Statements of Comprehensive Loss, (iv) Consolidated Statements of Stockholders' Equity, (v) Consolidated Statements of Cash Flows, (vi) Notes to Consolidated Financial Statements.

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

* Management contract or compensatory plan, contract or agreement.

Item 16. Form 10-K Summary

None.

STEMLINE THERAPEUTICS, INC.
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Report of Independent Registered Public Accounting Firm

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

Opinion on the Consolidated Financial Statements

We have audited the accompanying balance sheets of Stemline Therapeutics, Inc. (the Company) as of December 31, 2019 and 2018, the related consolidated statements of operations, comprehensive loss, stockholders' equity and cash flows for each of the three years in the period ended December 31, 2019, and the related notes (collectively referred to as the "consolidated financial statements"). In our opinion, the consolidated financial statements present fairly, in all material respects, the financial position of the Company at December 31, 2019 and 2018, and the results of its operations and its cash flows for each of the three years in the period ended December 31, 2019, in conformity with U.S. generally accepted accounting principles.

We also have audited, in accordance with the standards of the Public Company Accounting Oversight Board (United States) (PCAOB), the Company's internal control over financial reporting as of December 31, 2019, based on criteria established in Internal Control-Integrated Framework issued by the Committee of Sponsoring Organizations of the Treadway Commission (2013 framework), and our report dated March 16, 2020 expressed an unqualified opinion thereon.

Basis for Opinion

These consolidated financial statements are the responsibility of the Company's management. Our responsibility is to express an opinion on the Company's consolidated financial statements based on our audits. We are a public accounting firm registered with the PCAOB and are required to be independent with respect to the Company in accordance with the U.S. federal securities laws and the applicable rules and regulations of the Securities and Exchange Commission and the PCAOB.

We conducted our audits in accordance with the standards of the PCAOB. Those standards require that we plan and perform the audit to obtain reasonable assurance about whether the consolidated financial statements are free of material misstatement, whether due to error or fraud. Our audits included performing procedures to assess the risks of material misstatement of the consolidated financial statements, whether due to error or fraud, and performing procedures that respond to those risks. Such procedures included examining, on a test basis, evidence regarding the amounts and disclosures in the consolidated financial statements. Our audits also included evaluating the accounting principles used and significant estimates made by management, as well as evaluating the overall presentation of the consolidated financial statements. We believe that our audits provide a reasonable basis for our opinion.

/s/ Ernst & Young LLP

We have served as the Company's auditor since 2011.

Stamford, Connecticut
March 16, 2020

STEMLINE THERAPEUTICS, INC.
Consolidated Balance Sheets

	December 31,	
	2019	2018
Assets		
Current assets:		
Cash and cash equivalents	\$ 13,561,712	\$ 9,443,667
Short-term investments	150,869,056	50,662,189
Accounts receivable, net	15,120,229	—
Inventories, net	1,151,373	—
Prepaid expenses and other current assets	4,459,127	2,952,996
Total current assets	185,161,497	63,058,852
Property and equipment, net	191,158	222,413
Operating lease right-of-use assets	1,317,598	—
Other Assets	308,751	212,305
Total assets	<u>\$ 186,979,004</u>	<u>\$ 63,493,570</u>
Liabilities and stockholders' equity		
Current liabilities:		
Accounts payable and accrued expenses	\$ 21,952,048	\$ 21,153,062
Operating lease liabilities – current portion	1,169,764	—
Other current liabilities	10,125	65,862
Total current liabilities	23,131,937	21,218,924
Operating lease liabilities	226,306	—
Other Liabilities	4,370	72,591
Total liabilities	<u>23,362,613</u>	<u>21,291,515</u>
Commitments and contingencies (Note 15)		
Stockholders' equity:		
Preferred stock \$0.0001 par value, 5,000,000 shares authorized, none issued and outstanding at December 31, 2019 and 2018	—	—
Common stock \$0.0001 par value, 83,750,000 shares authorized at December 31, 2019 and 53,750,000 shares authorized at December 31, 2018. 50,349,150 shares issued and outstanding at December 31, 2019 and 31,943,186 shares issued and outstanding at December 31, 2018	5,035	3,194
Additional paid-in capital	529,488,474	331,343,484
Accumulated other comprehensive income (loss)	28,171	(56,559)
Accumulated deficit	(365,905,289)	(289,088,064)
Total stockholders' equity	<u>163,616,391</u>	<u>42,202,055</u>
Total liabilities and stockholders' equity	<u>\$ 186,979,004</u>	<u>\$ 63,493,570</u>

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

STEMLINE THERAPEUTICS, INC.
Consolidated Statements of Operations

	Year Ended December 31,		
	2019	2018	2017
Revenue:			
Product revenue, net	\$ 43,216,862	—	—
Income:			
Grant income	—	500,000	898,199
Operating expenses:			
Cost of goods sold	4,893,339	—	—
Research and development	50,724,411	47,725,019	50,242,386
Selling, general and administrative	66,846,633	39,061,667	19,214,207
Total operating expenses	122,464,383	86,786,686	69,456,593
Loss from operations	(79,247,521)	(86,286,686)	(68,558,394)
Other expense, net	(47,697)	(7,505)	(6,330)
Interest expense	(34,706)	(794)	—
Interest income	2,538,273	1,270,620	736,330
Net loss before income taxes	(76,791,651)	(85,024,365)	(67,828,394)
Income tax expense	(25,574)	—	—
Net loss	\$ (76,817,225)	\$ (85,024,365)	\$ (67,828,394)
Net loss per common share:			
Basic and Diluted	\$ (1.83)	\$ (2.99)	\$ (2.94)
Weighted-average shares outstanding:			
Basic and Diluted	42,091,027	28,388,901	23,056,928

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

STEMLINE THERAPEUTICS, INC.
Consolidated Statements of Comprehensive Loss

	Year Ended December 31		
	2019	2018	2017
Net loss	\$ (76,817,225)	\$ (85,024,365)	\$ (67,828,394)
Other comprehensive income (loss):			
Foreign currency translation adjustments	(5,783)	—	—
Unrealized gain (loss) on investments, net of tax	87,889	81,894	(52,308)
Reclassification adjustment for gain on investments included in net loss	2,624	7,505	6,152
Other comprehensive income (loss)	84,730	89,399	(46,156)
Comprehensive loss	<u>\$ (76,732,495)</u>	<u>\$ (84,934,966)</u>	<u>\$ (67,874,550)</u>

STEMLINE THERAPEUTICS, INC.
Consolidated Statements of Stockholders' Equity

	<u>Common Stock</u>		<u>Additional Paid-in Capital</u>	<u>Accumulated Other Comprehensive Income (Loss)</u>	<u>Accumulated Deficit</u>	<u>Total Stockholders' Equity</u>
	<u>Shares</u>	<u>Capital</u>				
Balance, December 31, 2016	19,219,223	\$ 1,922	\$ 193,563,572	\$ (99,802)	\$ (135,742,607)	\$ 57,723,085
Restricted stock grants	914,950	91	(91)	—	—	—
Forfeiture of restricted stock grants	(42,048)	(4)	4	—	—	—
Stock-based compensation	—	—	8,618,520	—	—	8,618,520
ESPP compensation expense	—	—	68,496	—	—	68,496
Adoption of accounting standard update related to stock compensation accounting (ASU 2016-09)	—	—	704,689	—	(704,689)	—
Issuance of common stock in connection with the ESPP	22,518	2	150,922	—	—	150,924
Issuance of common stock in connection with the exercise of stock options	23,952	2	141,738	—	—	141,740
Issuance of common stock in connection with follow-on public offering	5,175,000	518	48,241,696	—	—	48,242,214
Net loss	—	—	—	—	(67,828,394)	(67,828,394)
Other comprehensive loss	—	—	—	(46,156)	—	(46,156)
Balance, December 31, 2017	25,313,595	\$ 2,531	\$ 251,489,546	\$ (145,958)	\$ (204,275,690)	\$ 47,070,429
Restricted stock grants	1,770,126	177	(177)	—	—	—
Forfeiture of restricted stock grants	(63,353)	(6)	6	—	—	—
Stock-based compensation	—	—	13,942,611	—	—	13,942,611
ESPP compensation expense	—	—	84,678	—	—	84,678
Adoption of accounting standard update related to stock compensation accounting (ASU 2018-07)	—	—	(211,991)	—	211,991	—
Issuance of common stock in connection with the ESPP	24,599	2	194,212	—	—	194,214
Issuance of common stock in connection with the exercise of stock options	169,810	17	1,583,780	—	—	1,583,797
Issuance of common stock in connection with the exercise of warrants	30,830	3	352,525	—	—	352,528
Issuance of common stock in connection with follow-on public offering	4,697,579	470	63,908,294	—	—	63,908,764
Net loss	—	—	—	—	(85,024,365)	(85,024,365)
Other comprehensive income	—	—	—	89,399	—	89,399
Balance, December 31, 2018	31,943,186	\$ 3,194	\$ 331,343,484	\$ (56,559)	\$ (289,088,064)	\$ 42,202,055
Stock award – outside services	43,822	4	500,005	—	—	500,009
Restricted stock grants	1,953,940	196	(196)	—	—	—
Forfeiture of restricted stock grants	(86,041)	(9)	9	—	—	—
Stock-based compensation	—	—	26,380,233	—	—	26,380,233
ESPP compensation expense	—	—	266,942	—	—	266,942
Issuance of common stock in connection with ESPP	42,860	4	414,350	—	—	414,354
Issuance of common stock in connection with the exercise of stock options	479,161	48	2,140,660	—	—	2,140,708
Issuance of common stock in connection with follow-on public offering	15,972,222	1,598	168,442,987	—	—	168,444,585
Net loss	—	—	—	—	(76,817,225)	(76,817,225)
Other comprehensive income	—	—	—	84,730	—	84,730
Balance, December 31, 2019	50,349,150	\$ 5,035	\$ 529,488,474	\$ 28,171	\$ (365,905,289)	\$ 163,616,391

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

STEMLINE THERAPEUTICS, INC.
Consolidated Statements of Cash Flows

	Year Ended December 31,		
	2019	2018	2017
Cash flows from operating activities			
Net loss	\$ (76,817,225)	\$ (85,024,365)	\$ (67,828,394)
Adjustments to reconcile net loss to net cash used in operating activities:			
Depreciation and amortization expense	93,980	56,657	14,641
Stock-based compensation expense	26,175,233	13,942,611	8,618,520
Stock award – in-licensing	500,009	—	—
Employee Stock Purchase Plan compensation expense	266,942	84,678	68,496
(Accretion) Amortization of discount/premium paid on marketable securities	(995,858)	(96,723)	140,385
Non-cash lease expense	974,332	—	—
Net loss on sale of marketable securities	2,624	7,505	6,152
Unrealized foreign exchange gains	(15,364)	—	—
Changes in operating assets and liabilities:			
Accounts receivable, net	(15,103,164)	—	—
Inventories, net	(946,373)	—	—
Prepaid expenses and other current assets	(1,506,131)	(2,483,929)	(178,320)
Other assets	(96,446)	—	—
Accounts payable and accrued expenses	797,284	1,410,975	10,457,573
Operating lease liabilities	(1,013,920)	—	—
Other current liabilities	492	(37,366)	25,726
Deferred grant income	—	—	(898,199)
Other liabilities	(6,388)	(41,961)	(45,374)
Net cash used in operating activities	(67,689,973)	(72,181,918)	(49,618,794)
Cash flows from investing activities			
Purchase of furniture and fixtures	(62,726)	(112,870)	(128,782)
Purchase of short and long-term investments	(218,884,238)	(63,201,965)	(61,893,074)
Sale and maturities of short and long-term investments	119,755,335	74,111,419	57,584,806
Net cash (used in) provided by investing activities	(99,191,629)	10,796,584	(4,437,050)
Cash flows from financing activities			
Principal payments on finance lease liabilities	—	(5,401)	—
Proceeds from issuance of common stock from follow-on public offering, net	168,444,585	63,908,764	48,242,214
Proceeds from issuance of common stock from ESPP	414,354	194,215	150,924
Proceeds from exercise of stock options	2,140,708	1,583,797	141,740
Proceed from exercise of warrants	—	352,528	—
Net cash provided by financing activities	170,999,647	66,033,903	48,534,878
Net increase (decrease) in cash and cash equivalents	4,118,045	4,648,569	(5,520,966)
Cash and cash equivalents at beginning of period	9,443,667	4,795,098	10,316,064
Cash and cash equivalents at end of period	\$ 13,561,712	\$ 9,443,667	\$ 4,795,098

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

STEMLINE THERAPEUTICS, INC.
Notes To Consolidated Financial Statements
December 31, 2019

1. Organization, Basis of Presentation and Use of Estimates

Organization

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

The Company consists of Stemline Therapeutics, Inc. in the U.S. and two wholly owned subsidiaries: Stemline Therapeutics, B.V. (“BV”) incorporated in the Netherlands on August 15, 2019 and Stemline Therapeutics Switzerland, GmbH (“GmbH”), incorporated in Switzerland on October 3, 2019. The GmbH is a wholly owned subsidiary of the BV. The BV was set up to support the ELZONRIS Marketing Authorization Application (“MAA”) to the European Medicines Agency (“EMA”) in the European Union (“EU”). The GmbH is providing European pre-launch commercial marketing and medical education support for ELZONRIS.

The Company has incurred losses from operations since inception of \$377.6 million. Since its inception, most of its resources have been dedicated to the discovery, acquisition, preclinical and clinical development, and commercialization of its product and product candidates. In particular, it has expended and will continue to expend substantial resources for the foreseeable future developing and commercializing its product and product candidates, as well as its preclinical product candidates, drug discovery and acquisition efforts. These expenditures include costs associated with general and administrative costs, facilities costs, research and development, acquiring new technologies, manufacturing product and product candidates, conducting preclinical experiments and clinical trials and obtaining regulatory approvals, as well as commercializing. The Company anticipates incurring additional losses until such time, if ever, that it can generate significant sales of its product and product candidates currently in development. The Company expects its research and development expenses to increase significantly in connection with its ongoing and planned clinical trials and related manufacturing efforts, as well as for expenses related to the commercial launch of ELZONRIS. As a result, the Company expects to continue to incur significant and increasing operating losses for the foreseeable future. If adequate funds are not available to the Company on a timely basis, or at all, the Company may be required to terminate or delay clinical trials or other development activities, its marketing efforts for ELZONRIS, or product candidates, or for one or more indications for which it is developing ELZONRIS, or product candidates, or delay its establishment of sales and marketing capabilities or other activities that may be necessary to commercialize its product or product candidates. ELZONRIS is a novel targeted therapy directed to the interleukin-3 receptor- α , or CD123, a target present on a wide range of malignancies as well as some autoimmune disorders. ELZONRIS has been approved by the FDA for the treatment of blastic plasmacytoid dendritic cell neoplasm (“BPDCN”) in adults and in pediatric patients two years and older, in both treatment-naïve and previously treated populations in the United States.

Basis of Presentation

The consolidated financial statements include the financial results of the Company and its subsidiaries. Intercompany accounts, transactions and profits are eliminated in consolidation.

Use of Estimates

The Company's consolidated financial statements are prepared in conformity with generally accepted accounting principles in the U.S. ("U.S. GAAP") which requires management to make estimates and assumptions that affect the reported financial position at the date of the financial statements and the reported results of operations during the reporting period. Such estimates and assumptions affect the reported amounts of assets, liabilities, revenues and expenses and disclosure of contingent assets and liabilities in the financial statements and accompanying notes. Estimates have been prepared using the most current and best available information; however, actual results could differ materially from those estimates.

2. Summary of Significant Accounting Policies

Revenue Recognition

The Company sells ELZONRIS in the U.S. to one customer and one customer internationally through a title distribution channel. The U.S. customer subsequently resells ELZONRIS to a limited number of specialty distributors who, in turn, distribute ELZONRIS to specialty hospitals and the international customer subsequently resells and distributes ELZONRIS outside of the U.S. directly to hospitals in certain countries that participate in the early access program (EAP) in place as of December 31, 2019.

The Company recognizes revenue for each separately identifiable performance obligation in a contract representing a promise to transfer a distinct good or service to a customer. The Company performs the following five steps to determine the amount of revenue to be recognized for each separately identifiable performance obligation: (i) identify the contract(s) with a customer; (ii) identify the performance obligations in the contract; (iii) determine the transaction price; (iv) allocate the transaction price to the performance obligations in the contract; and (v) recognize revenue when (or as) the entity satisfies a performance obligation. The Company only applies the five-step model to contracts when it is probable that the Company will collect the consideration it is entitled to in exchange for the goods or services it transfers to the customer. At contract, the Company assesses the goods or services promised within each contract, determines which goods and services are performance obligations, and assesses whether each promised good or service is distinct. The Company then recognizes as revenue the amount of the transaction price that is allocated to the respective performance obligation when (or as) the performance obligation is satisfied, which is at point in time or upon delivery. The Company determined that the delivery of its product to its customer constitutes a single performance obligation as there are no other promises to deliver goods or services.

The Company records revenue at the product's wholesale acquisition costs and adjusted for applicable government contract fees, product returns, commercial co-payment assistance program transactions, and distribution service fees that are offered within contracts between the Company and its customer, payors, and other indirect customers relating to the sale of its product.

Reserves are recorded at the time of sale to the customer to reflect the Company's best estimates of the amount of consideration expected to be received based on the historical amounts earned, or expected to be claimed, and are classified as reductions of accounts receivable (if the amount is payable to the Customer and right of offset exists) or a current liability (if the amount is payable to a party other than a Customer) on its consolidated balance sheet. The reserves are based on the expected value method and a range of outcomes and are probability weighted in accordance with ASC 606. The amount of variable consideration which is included in the transaction price may be constrained and is included in the net sales price only to the extent that it is probable that a significant reversal in the amount of the cumulative revenue recognized under contracts will not occur in a future period. The Company's analyses contemplate the application of the constraint in accordance with ASC 606. Actual amounts of consideration ultimately received may differ from the Company's estimates. If actual results in the future vary from the Company's estimates, the Company will adjust these estimates, which would affect net product revenue and earnings in the period such variances become known.

Shipping and handling are considered to be fulfillment activities and are not considered separate performance obligations. The Company has assessed the existence of a significant financing component in the agreement with its customer. The payment terms with its customer do not exceed one year and therefore, no amount of consideration has been allocated as a financing component. Taxes collected related to product sales are remitted to governmental authorities and are excluded from revenue.

Government Contracts

The Company has entered into contracts (i) to participate in the Medicaid Drug Rebate Program and the Medicare Part D program, and (ii) to sell to the U.S. Department of Veterans Affairs, 340b entities and other government agencies (“Government Payors”) so that ELZONRIS will be eligible for purchase by, in partial or full reimbursement from, such Government Payors.

The Company estimates the rebates that it will provide to Government Payors for these programs that require rebates. These rebate estimates are based upon (i) the government-mandated discounts applicable to government-funded programs, (ii) information obtained from its customers and (iii) information obtained from other third parties regarding the payor mix for ELZONRIS. The liability for these rebates consists of estimates of claims for the current year and estimated future claims that will be made for product shipments that have been recognized as revenue but remain in the distribution channel inventories at the end of each reporting period. These reserves are recorded in the same period the revenue is recognized, resulting in a reduction of product revenue and accounts receivable (if the amount is payable to the Customer) or a current liability (if the amount is payable to a party other than a Customer), which is included in accounts payable and accrued expenses. For Medicare Part D, the Company estimates the number of patients in the prescription drug coverage gap for whom it will owe an additional discount under the Medicare Part D program.

Product Returns

Consistent with industry practice, the Company offers a limited right of return for product that has been purchased. The Company estimates, on a quarterly basis, the number of vials that are held in inventory throughout the distribution channel which may be returned by its customers. Amounts for estimated product returns are established in the same period that the related revenue is recognized, resulting in a reduction of product revenue and the establishment of a current liability which is included as a component of accounts payable and accrued expenses in the consolidated balance sheet.

Commercial Co-payment Assistance Program

The Company offers a co-payment assistance program where permitted by law and which is intended to provide financial assistance to qualified commercially-insured patients who are required to pay prescription drug copayments based on the terms of their prescription drug insurance plans. The calculation of the accrual for co-payment assistance is based on an estimate of claims and the cost per claim that the Company expects to receive associated with product that has been recognized as revenue but remains in the distribution channel at the end of each reporting period. The adjustments are recorded in the same period the related revenue is recognized, resulting in a reduction of product revenue and the establishment of a current liability which is included as a component of accrued expenses and other current liabilities.

Distribution Fees

Distribution fees include fees paid to the Company’s distributors for the distribution of ELZONRIS based on contractual rates. In addition, the Company compensates for data and other administrative activities. Therefore, estimates for these costs are recorded as a reduction of revenue, based on contractual terms and included as a component of accrued expenses and other current liabilities.

Grant Income Recognition

In October 2013, the Company entered into a contract with the Leukemia and Lymphoma Society, or LLS, whereby LLS agreed to provide funding to the Company not to exceed \$3.5 million. LLS is a national voluntary health organization that, among other activities, encourages and sponsors research relating to blood cancers to develop therapies to cure or mitigate these diseases. This research grant was awarded to the Company to support funding some of the costs for the ongoing ELZONRIS clinical trials. Grant payments received prior to the Company’s performance of work required by the terms of the research grant are recorded as deferred income and recognized as grant income once work is performed and qualifying costs are incurred. The Company has recognized approximately \$0, \$0.5 million, and \$0.9 million of income related to the LLS grant for the years ended December 31, 2019, 2018 and 2017, respectively, which reflect twelve months of income recognized, respectively, on a straight-line basis, based on the Company’s best estimates of the timing of work to be performed and qualifying costs incurred. The Company has received \$3.5 million from LLS to date.

Accounts Receivable, Net

Accounts receivable, net primarily relates to amounts due from the Company's customer. The Company analyzes accounts that are past due for collectability and provides an allowance for receivables when collection becomes doubtful. No reserve has been recorded relating to an allowance for doubtful accounts at December 31, 2019.

Cost of Goods Sold

Cost of goods sold consists of all costs related to manufacturing ELZONRIS finished drug product for sale to the Company's customer. These costs include expenses incurred by the Company's contract manufacturing organizations ("CMOs") to manufacture drug substance and drug product. Cost of goods sold also includes expenses related to labeling and packaging of drug product, indirect manufacturing overhead and stability testing. Royalty costs due to the licensor of ELZONRIS are also recorded in cost of goods sold as a period cost.

Cash Equivalents

Cash equivalents consist of highly liquid instruments purchased with an original maturity of three months or less. The Company primarily invests in highly liquid cash equivalents, investments in U.S. Treasury and Agency securities and related money market funds and FDIC-insured bank certificates of deposit, with the balance in commercial bank operating accounts. At December 31, 2019 and 2018, cash equivalents consisted of deposits in financial institutions and money market mutual funds invested in U.S. treasury securities. The Company maintains its cash deposits and cash equivalents with well-known and stable financial institutions.

Concentration of Credit Risk

Financial instruments that potentially subject the Company to significant concentrations of credit risk consist primarily of cash equivalents. The Company primarily invests cash, cash equivalents and short-term investments in US Treasury and Agency securities and related money market funds and FDIC-insured bank certificates of deposits with the balance in commercial bank operating accounts.

The Company has not experienced any losses from credit risks.

Foreign Currency

The functional currency of the Company's international subsidiaries is generally the Euro. The Company translates the financial statements of its international subsidiaries to U.S. dollars using month-end exchange rates for assets and liabilities, and average exchange rates for revenue, costs and expenses. The Company records translation gains and losses in accumulated other comprehensive income (loss) as a component of stockholders' equity. Foreign currency transaction gains and losses are included within other expense, net in the consolidated statements of operations.

Inventory

The Company capitalizes inventory costs associated with the manufacturing of ELZONRIS after regulatory approval or when, based on management's judgment, future commercialization is considered probable and the future economic benefit is expected to be realized. Otherwise, such costs are expensed as research and development. A significant amount of manufacturing costs for ELZONRIS recognized as revenue during the year ended December 31, 2019 were expensed to research and development prior to FDA approval of ELZONRIS on December 21, 2018.

The Company values its inventories at the lower of cost or estimated realizable value. The Company determines the cost of inventories, which includes amounts related to materials and manufacturing overhead, on a first-in, first-out, or FIFO, method. The Company performs an assessment of the recoverability of capitalized inventory during each reporting period, and it writes down any excess and obsolete inventories to their estimated realizable value in the period in which the impairment is first identified. Such impairment charges, should they occur, are recorded within cost of goods sold. The determination of whether inventory costs will be realizable requires estimates by management. If actual market conditions are less favorable than projected by management, additional write-downs of inventory may be required which would be recorded as a cost of goods sold in the statement of operations.

Investments

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

Fair Value of Financial Instruments

The Company's financial instruments consist principally of cash and cash equivalents, investments, other current assets, accounts payable and accrued liabilities. Cash and cash equivalents, and short-term investments are carried at fair value (see Note 9). Financial instruments including other current assets, accounts payable and accrued liabilities are carried at cost, which approximate fair value given their short-term nature.

Other-Than-Temporary Impairment Losses on Investments

The Company regularly monitors its available-for-sale investments portfolio to evaluate the necessity of recording impairment losses for OTT declines in the fair value of investments. Management makes this determination through the consideration of various factors such as management's intent and ability to retain an investment for a period of time sufficient to allow for any anticipated recovery in market value. OTT impairment losses result in a permanent reduction of the cost basis of an investment. For the years ended December 31, 2019 and 2018, the Company did not realize any investment losses due to OTT declines in fair value.

Property and Equipment

Property and Equipment are stated at cost and depreciated on a straight-line basis over the shorter of their estimated useful lives or related contract terms beginning in the year the asset was placed into service. Normal repair and maintenance costs are expensed as incurred. The useful lives by asset category are as follows:

Furniture and Fixtures	3-5 years
Computer Equipment	5 years
Manufacturing Equipment	7 years

Leasehold improvements are amortized over the lesser of the lease terms or the estimated useful life of the improvements and such amortization is included in depreciation expense.

Leases

The Company leases office space and certain equipment. Under ASC 842, at contract inception, the Company determines whether a contract is or contains a lease and whether the lease should be classified as an operating or finance lease. The Company recognizes operating lease right-of-use assets and operating lease liabilities based on the present value of the future minimum lease payments over the lease term at commencement date. The Company uses incremental borrowing rate based on the information available at commencement date to determine the present value of future payments and the appropriate lease classification. The Company defines initial lease term to include renewal options determined to be reasonably certain. In adoption of ASC 842, the Company elected not to recognize a right-of-use asset and a lease liability for leases with an initial term of 12 months or less; the Company recognizes lease expense for these leases on a straight-line basis over the lease term. For tenant improvement incentives, determined to be a leasehold improvement owned by the Company, it generally records a deferred rent liability and amortizes the deferred rent over the term of the lease as a reduction to rent expense. For rent holidays and rent escalation clauses during the lease term, the company records rental expense on a straight-line basis over the term of the lease. For these lease incentives, the company uses the date of initial possession as the commencement date, which is generally when the Company is given the right of access to the space and begins to make improvements in preparation for intended use.

Finance leases are not material to its consolidated financial statements and the Company is not a lessor in any of its lease arrangements. The Company does not have any material restrictions, covenants or residual value guarantees in its lease agreements.

Impairment of Long-Lived Assets

The Company reviews long-lived assets, the furniture and fixtures, for impairment whenever events or changes in business circumstances indicate that the carrying amount of the assets may not be fully recoverable. An impairment loss would be recognized when estimated undiscounted future cash flows expected to result from the use of the asset and its eventual disposition are less than its carrying amount. The impairment loss, if recognized, would be based on the excess of the carrying value of the impaired asset over its respective fair value. The Company purchased fixed assets during 2019, 2018 and 2017. For the years ended December 31, 2019, 2018 and 2017, the Company did not realize any impairment losses.

Accrued Clinical Development Costs

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

Research and Development Costs

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

Advertising Costs

Advertising costs are expensed as incurred and included in selling, general and administrative expenses. Advertising expenses were \$0.6 million, \$0, and \$0 for the fiscal years ended December 31, 2019, 2018, and 2017, respectively.

Income Taxes

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

The Company continues to assess the ability to realize deferred tax assets, which primarily consist of net operating losses, or NOL, carry-forwards. In assessing the ability to realize these deferred tax assets, the Company considers whether it is more likely than not that some portion or all of the deferred tax assets will be realized. The Company establishes valuation allowances when necessary to reduce deferred tax assets to the amounts expected to be realized. The factors used to assess the likelihood of realization includes the latest forecast of future taxable income and available tax planning strategies that could be implemented to realize the net deferred tax assets. As of December 31, 2019, 2018 and 2017, the Company’s deferred tax assets had full valuation allowances on them as it did not have sufficient positive evidence to recognize such deferred tax assets.

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

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

As of December 31, 2019, the Company had net operating losses of \$284.7 million for federal and \$288.5 million for state purposes and research and development credits of \$39.0 million which expire in 2023 through 2038.

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

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

Tax Loss Carryforwards

As of December 31, 2019, the Company had net operating losses of \$284.7 million for federal and \$288.5 million state purposes, which are available to reduce future taxable income. The Company also had federal tax credits of approximately \$39.0 million, which may be used to offset future tax liabilities. The net operating loss and tax credit carryforwards will expire at various dates through 2038. Net operating loss and tax credit carryforwards are subject to review and possible adjustment by the Internal Revenue Service and state tax authorities and may become subject to an annual utilization limitation pursuant to the change in ownership rules of Internal Revenue Code Section 382 and 383. The amount of the annual limitation is determined based on the value of the company immediately prior to the ownership change. Subsequent ownership changes may further affect the limitation in future years. At December 31, 2019, the Company recorded a 100% valuation allowance against its net operating loss and tax credit carryforwards, as the Company believes it is more likely than not that the tax benefits will not be fully realized.

Stock-Based Compensation

The Company accounts for stock-based compensation in accordance with the provisions of ASC 718, *Compensation—Stock Compensation*. Accordingly, compensation costs related to equity instruments granted are recognized over the requisite service periods of the awards on a straight-line basis at the grant-date fair value calculated using a Black-Scholes option pricing model for stock options and the closing stock price on date of grant for restricted stock. On January 1, 2017, the Company adopted ASU No. 2016-09, *Improvements to Employee Share-Based Payment Accounting*, or ASU 2016-09, which amends Accounting Standards Codification, or ASC, Topic 718, *Compensation — Stock Compensation*. ASU 2016-09 simplifies several aspects of the accounting for share-based payment transactions, including the accounting for forfeitures, income tax consequences, classification of awards as either equity or liabilities, and classification on the statement of cash flows. Upon adoption, the Company is accounting for forfeitures as they occur rather than estimate a forfeiture rate and it has recorded a cumulative-effect adjustment in equity of \$0.7 million on the date of initial adoption. In addition, the standard requires that excess tax benefits and deficiencies that arise upon vesting or exercise of share-based payments be recognized as an income tax benefit and expense in the income statement. Previously, such amounts were recognized as an increase and decrease in additional paid-in capital. The Company has adopted this aspect of the standard prospectively. On June 20, 2018, the FASB issued ASU No. 2018-07, *Compensation — Stock Compensation (Topic 718): Improvements to Nonemployee Share-Based Payment Accounting*, which expands the scope of Topic 718 to include share-based payment transactions for acquiring goods and services from nonemployees. The Company early adopted ASU No. 2018-07 on April 1, 2018 and the net impact relating to the adoption was a \$0.2 million decrease to accumulated deficit for the impact prior to April 1, 2018.

The Company's policy upon exercise of stock options is that shares will be issued as new shares drawing on the Company's 2016 Stock Equity Incentive Plan (the "2016 Plan") share pool that was adopted by the board of directors and approved by the stockholders in May 2016. During June 2017, an amendment was approved by the stockholders to increase the authorized shares under the 2016 plan by 1,200,000 shares. An amendment was approved by the stockholders at the 2018 Annual Meeting of Stockholders on June 21, 2018, to increase the authorized shares under the 2016 plan by 2,900,000 shares. An amendment was approved by the stockholders at the 2019 Annual Meeting of Stockholders on June 25, 2019, to increase the authorized shares under the 2016 plan by 2,500,000 shares. Under the provisions of the 2016 Plan, no option will have a term in excess of 10 years.

In the event a modification is made to an equity award after the grant date, the Company records a change in stock-based compensation expense equal to the incremental fair value of the equity award immediately subsequent to the modification as compared to the fair value of the equity award immediately preceding the modification. During 2018, the Company modified certain outstanding equity award held by employees and certain Directors. These modifications resulted in incremental compensation cost of \$1.1 million for the year ended December 31, 2018.

Segment Information

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

Recent Accounting Pronouncements

In February 2016, the FASB issued *ASU No. 2016-02, Leases (Topic 842)*. ASU Topic 842 supersedes existing lease accounting guidance, including ASC 840, leases. Among other things, ASC 2016-02 requires recognition of a right-of-use ("ROU") asset and liability for future lease payments for contracts that meet the definition of a lease and requires disclosure of certain information about leasing arrangements. On July 30, 2018, the FASB issued ASU No. 2018-11, *Leases (Topic 842): Targeted Improvements*, which, among other things, allows companies to elect an optional transition method to apply the new lease standard through a cumulative-effect adjustment in the period of adoption. The Company adopted the standard as of January 1, 2019, using the optional transition method, and as a result, did not recast prior period financial statements. All prior period amounts are presented under ASC 840.

The Company elected the package of practical expedients permitted under the optional transition method, which among other things, allows us to carryforward the prior lease classifications under ASC 840. The Company did not elect to adopt the hindsight practical expedient and are therefore, maintaining the lease terms it previously determined under ASC 840. In addition, the Company elected to combine the lease and non-lease components for the asset categories comprising its leases and to exclude from balance sheet reporting those leases with initial terms of 12 months or less.

Adoption of the new standard resulted in the recognition of lease assets and lease liabilities of approximately \$2 million on the Company's consolidated balance sheet.

In June 2016, the FASB issued ASU No. 2016-13, *Financial Instruments - Credit Losses (Topic 326): Measurement of Credit Losses on Financial Instruments*. ASU 2016-13 amends the impairment model by requiring entities to use a forward-looking approach based on expected losses to estimate credit losses on certain types of financial instruments, including trade receivables. In November 2018, the FASB issued ASU No. 2018-19, *Codification Improvements to Topic 326, Financial Instruments - Credit Losses*. ASU 2018-19 will affect loans, debt securities, trade receivables, net investments in leases, off balance sheet credit exposures, reinsurance receivables, and any other financial assets not excluded from the scope of this amendment that represent the contractual right to receive cash. ASU 2016-13 and ASU 2018-19 are effective for public entities for fiscal years beginning after December 15, 2019, with early adoption permitted. The Company is currently evaluating the impact of adopting ASU 2016-13 on the Company's financial statements.

In December 2019, the FASB issued ASU 2019-12, *Simplifying the Accounting for Income Taxes*, which simplifies the accounting for income taxes, eliminates certain exceptions within ASC Topic 740, *Income Taxes*, and clarifies certain aspects of the current guidance to promote consistency among reporting entities. Most amendments within the standard are required to be applied on a prospective basis, while certain amendments must be applied on a retrospective or modified retrospective basis. ASU 2019-12 is effective for public entities for fiscal years, and interim periods within those fiscal years, beginning after December 15, 2020. The Company plans to adopt this standard on January 1, 2021 and is currently evaluating the effect on the Company's consolidated financial statements.

Other accounting standards updates adopted and/or issued, but not effective until after December 31, 2019, are not expected to have a material effect on the company's consolidated financial position, annual results of operations and/or cash flows.

3. Net Loss per Common Share

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

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

	Year Ended December 31,		
	2019	2018	2017
Basic and Diluted loss per common share calculation:			
Net loss attributable to common shareholders — basic and diluted	\$ (76,817,225)	\$ (85,024,365)	\$ (67,828,394)
Basic and diluted weighted-average common shares	42,091,027	28,388,901	23,056,928
Basic and diluted net loss per share	\$ (1.83)	\$ (2.99)	\$ (2.94)

The difference between basic and diluted weighted-average common shares generally results from the assumption that dilutive stock options outstanding were exercised, dilutive restricted stock has vested and outstanding warrants are issued. For the years ended December 31, 2019, 2018 and 2017, the Company reported a loss from operations and therefore, all potentially dilutive stock options, restricted stock, and outstanding warrants as of such date were excluded from the computation of diluted net loss per share as their effect would have been anti-dilutive. The total shares of stock options, restricted stock, and outstanding warrants that could potentially dilute earnings per share in the future, but which were not included in the calculation of diluted net loss per share because their effect would have been anti-dilutive were as follows:

	Year Ended December 31		
	2019	2018	2017
Unvested restricted stock	3,002,584	2,794,455	1,724,837
Options outstanding	3,026,401	3,514,018	3,174,964
Warrants	—	—	99,529
Total	<u>6,028,985</u>	<u>6,308,473</u>	<u>4,999,330</u>

4. Investments

The following is a summary of cash and cash equivalents and available-for-sale investments held by the Company at December 31, 2019 and December 31, 2018:

	December 31, 2019			
	Amortized Cost	Gross Unrealized Gains*	Gross Unrealized Losses*	Estimated Fair Value
Cash:				
Cash from operating accounts	\$ 9,838,148	\$ —	\$ —	\$ 9,838,148
Cash equivalents:				
Money market funds	3,723,564	—	—	3,723,564
Total cash and cash equivalents	13,561,712	—	—	13,561,712
Short-term investments:				
Fixed-income treasury portfolio:				
Fannie Mae	6,055,546	712	(2,018)	6,054,240
Federal home loan bank	5,107,715	942	(275)	5,108,382
Federal farm credit bank	2,423,780	2,167	—	2,425,947
Freddie Mac	4,016,826	—	(119)	4,016,707
U.S. treasury securities	133,181,880	93,511	(11,611)	133,263,780
Total short-term investments	150,785,747	97,332	(14,023)	150,869,056
Total	\$ 164,347,459	\$ 97,332	\$ (14,023)	\$ 164,430,768

	December 31, 2018			
	Amortized Cost	Gross Unrealized Gains*	Gross Unrealized Losses*	Estimated Fair Value
Cash:				
Cash from operating accounts	\$ 2,482,621	\$ —	\$ —	\$ 2,482,621
Cash equivalents:				
Money market funds	6,961,046	—	—	6,961,046
Total cash and cash equivalents	9,443,667	—	—	9,443,667
Short-term investments:				
Fixed-income treasury portfolio:				
Fannie Mae	2,013,336	—	(1,146)	2,012,190
Federal home loan bank	6,020,492	—	(10,646)	6,009,846
Freddie Mac	2,512,252	—	(8,530)	2,503,722
U.S. treasury securities	20,123,026	767	(11,553)	20,112,240
Certificate of Deposits	20,024,346	—	(155)	20,024,191
Total short-term investments	50,693,452	767	(32,030)	50,662,189
Total	\$ 60,137,119	\$ 767	\$ (32,030)	\$ 60,105,856

* The gross unrealized gains and losses captured in this footnote is before tax.

At December 31, 2019 and December 31, 2018, the remaining contractual maturities of available-for-sale investments classified as current on the balance sheet were less than 12 months.

There were no available-for-sale securities in a continuous unrealized loss position for greater than twelve months at December 31, 2019 and December 31, 2018. The Company has the ability to hold such securities with an unrealized loss until its forecasted recovery. The Company determined that there was no material change in the credit risk of the above investments. As a result, the Company determined it did not hold any investments with an other-than-temporary impairment as of December 31, 2019.

5. Prepaid Expenses and Other Current Assets

Prepaid expenses and other current assets consist of the following at December 31, 2019 and 2018:

	December 31, 2019	December 31, 2018
Prepaid third-party vendor costs	\$ 3,105,088	\$ 1,851,553
Deposits	555,788	189,000
Prepaid insurance	140,356	69,021
Other receivables	657,895	843,422
Total	<u>\$ 4,459,127</u>	<u>\$ 2,952,996</u>

6. Property and Equipment, Net

Property and equipment, net, consist of the following at December 31, 2019 and 2018:

	December 31, 2019	December 31, 2018
Office furniture and fixtures	\$ 524,840	\$ 519,675
Manufacturing equipment	181,753	107,258
Leasehold improvements	82,694	82,694
Capital lease equipment	—	29,529
Computer equipment	31,206	18,612
Property and equipment	<u>820,493</u>	<u>757,768</u>
Less accumulated depreciation and amortization	(629,335)	(535,355)
Property and equipment, net	<u>\$ 191,158</u>	<u>\$ 222,413</u>

Depreciation and amortization expense was \$93,980, \$56,657, and \$14,641 for the years ended December 31, 2019, 2018 and 2017, respectively.

The Company adopted ASC 842 on January 1, 2019. Pursuant to ASC 842, capital leases are now classified as finance leases and are included in other assets on the consolidated balance sheet.

7. Inventories, Net

Inventories, net, consists of the following:

	December 31, 2019
Raw materials	\$ 49,457
Work-in-process	1,053,956
Finished goods	<u>1,453,083</u>
Inventory at cost	2,556,496
Inventory obsolescence reserves	(1,405,123)
Total inventories, net	<u>\$ 1,151,373</u>

Inventories, net, are related to the Company's approved product, ELZONRIS. The Company had no inventory at December 31, 2018, as the majority of manufacturing costs for ELZONRIS were expensed to research and development prior to FDA approval on December 21, 2018.

The Company continually analyze the manufacturing expiry date and determine its impact on the inventory reserves inclusive of the amount of ELZONRIS inventory currently on hand, type of inventory in the supply chain, and current demand forecasts. The Company recorded a \$1.4 million inventory obsolescence reserve at December 31, 2019 reflecting on hand ELZONRIS inventory that is projected to not sell through to the distribution channel prior to expiry. There were no write-offs of inventory charged against the inventory obsolescence reserve during 2019.

8. Product Revenue, Net

The following table presents the Company's net product revenue by geographic region for the year ended December 31, 2019:

	December 31, 2019
United States	\$ 41,946,502
Europe	1,270,360
Total product revenue, net	\$ 43,216,862

The following table summarizes activity in each of the product revenue allowance and reserve categories for the year ended December 31, 2019:

	Year Ended December 31, 2019				
	Government Rebates	Product Returns	Commercial Co-payment Assistance Programs	Distribution Fees	Total
Beginning balance at December 31, 2018	\$ —	\$ —	\$ —	\$ —	\$ —
Provision related to sales in the current year	3,574,882	1,662,720	129,862	1,211,454	6,578,918
Credits and payments	(2,996,768)	(903,910)	—	(878,164)	(4,778,842)
Balance at December 31, 2019	<u>\$ 578,114</u>	<u>\$ 758,810</u>	<u>\$ 129,862</u>	<u>\$ 333,290</u>	<u>\$ 1,800,076</u>

Reserves for government rebates are recorded as a reduction of accounts receivable (if the amount is payable to the Customer) or a current liability (if the amount is payable to a party other than a Customer), which is included in accounts payable and accrued expenses on the consolidated balance sheet. Product returns, commercial co-payment assistance programs, and distribution fees are recorded as a component of accounts payable and accrued expenses on the consolidated balance sheet.

9. Fair Value Measurements

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

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

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

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

The following fair value hierarchy table presents information about each major category of the Company's financial assets and liability measured at fair value on a recurring basis as of December 31, 2019 and 2018:

	December 31, 2019			Balance at December 31, 2019
	Quoted Prices in Active Markets for Identical Assets (Level 1)	Significant Other Observable Inputs (Level 2)	Significant Unobservable Inputs (Level 3)	
Assets:				
Fixed-income treasury portfolio	\$ 150,869,056	\$ —	\$ —	\$ 150,869,056
Cash and cash equivalents	13,561,712	—	—	13,561,712
Total assets at fair value	<u>\$ 164,430,768</u>	<u>\$ —</u>	<u>\$ —</u>	<u>\$ 164,430,768</u>

	December 31, 2018			Balance at December 31, 2018
	Quoted Prices in Active Markets for Identical Assets (Level 1)	Significant Other Observable Inputs (Level 2)	Significant Unobservable Inputs (Level 3)	
Assets:				
Fixed-income treasury portfolio	\$ 30,637,998	\$ —	\$ —	\$ 30,637,998
Certificate of Deposits	—	20,024,191	—	20,024,191
Cash and cash equivalents	9,443,667	—	—	9,443,667
Total assets at fair value	<u>\$ 40,081,665</u>	<u>\$ 20,024,191</u>	<u>\$ —</u>	<u>\$ 60,105,856</u>

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

10. Accounts Payable and Accrued Expenses

Accounts payable and accrued expenses consist of the following:

	December 31,	
	2019	2018
Accrued research and development costs	\$ 9,012,182	\$ 8,790,920
Accrued commercial costs	4,674,204	4,614,507
Accrued compensation	5,598,551	5,515,002
Accrued sales deduction and allowance	1,360,033	—
Accrued general and administrative costs	708,042	1,545,591
Accrued legal	420,781	687,042
Accrued royalty	178,255	—
Total accounts payable and accrued expenses	<u>\$ 21,952,048</u>	<u>\$ 21,153,062</u>

11. Capital Structure

Initial Public Offering

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

Representative's Warrants

On October 1, 2012, the Company agreed to issue to the representative of the underwriters in the IPO warrants to purchase up to 99,529 shares of the Company's common stock in the event of a successful public offering. The warrants are exercisable for cash or on a cashless basis at a price per share equal to \$15.00. The term of the warrants is five years and they expired on January 28, 2018. During the year ended December 31, 2018, warrants to purchase 99,529 shares of common stock were exercised, resulting in proceeds of approximately \$0.4 million. A portion of these transactions were processed via cashless exercises. As of December 31, 2019, there were no warrants outstanding. Based on a successful public offering in January of 2013, these warrants were issued and accounted for as a cost of issuance. The Company has determined, based upon a Black-Scholes model, that the fair value of the warrants on the date of IPO was \$413,146. The Company has accounted for the fair value of the warrants as a cost of issuance of common stock from the IPO resulting in a charge directly to stockholder's equity.

Common Stock

As of December 31, 2019, the Company was authorized to issue 83,750,000 shares of common stock. Dividends on common stock will be paid when, and if, declared by the board of directors. Each holder of common stock is entitled to vote on all matters and is entitled to one vote for each share held. The Company will, at all times, reserve and keep available, out of its authorized but unissued shares of common stock, sufficient shares to affect the conversion of the shares of the stock options.

Follow-on Public Offerings

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

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

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

On January 26, 2018, the Company completed a fourth follow-on public offering, selling 3,700,000 shares at an offering price of \$14 per share. Additionally, the underwriters exercised in full their over-allotment option to purchase an additional 555,000 shares at an offering price of \$14 per share. Aggregate gross proceeds from the follow-on public offering, including the exercise of the over-allotment option, were \$59.6 million, and net proceeds received after underwriting fees and offering expenses were approximately \$55.7 million.

On January 18, 2019, the Company completed a fifth follow-on public offering, selling 8,888,889 shares at an offering price of \$9 per share. Additionally, the underwriters exercised in full their over-allotment option to purchase an additional 1,333,333 shares at an offering price of \$9 per share. Aggregate gross proceeds from the follow-on public offering, including the exercise of the over-allotment option, were \$92 million, and net proceeds received after underwriting fees and offering expenses were approximately \$86.2 million.

On August 8, 2019, the Company completed a sixth follow-on public offering, selling 5,000,000 shares at an offering price of \$15.25 per share. Additionally, the underwriters exercised in full their over-allotment option to purchase an additional 750,000 shares at an offering price of \$15.25 per share. Aggregate gross proceeds from the follow-on public offering, including the exercise of the over-allotment option, were \$87.7 million, and net proceeds received after underwriting fees and offering expenses were approximately \$82.2 million.

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

12. Grant Income

In October 2013, the Company entered into a contract with the LLS whereby LLS agreed to provide funding to the Company not to exceed \$3.5 million. LLS is a national voluntary health organization that, among other activities, encourages and sponsors research relating to blood cancers to develop therapies to cure or mitigate these diseases. In consideration of funding by LLS and transfer to the Company of any rights LLS may have to any project inventions developed during the term of the agreement, the Company may be required to pay LLS a cash multiple on the LLS funding, less any amount the Company contributes to LLS to support the Company's preclinical and clinical development activities to bring ELZONRIS to commercialization. Through December 31, 2019, the Company have received the full \$3.5 million based on milestones achieved. The Company is required to make a one-time payment upon regulatory approval and commercialization of ELZONRIS. Additionally, the Company is required to make payments based on achievement of specific sale-based commercial milestones. The total amount payable by the Company to LLS will not exceed three (3) times the amount of net funding received from LLS of \$2.9 million. The Company may terminate the license for any or no reason upon 60 days advance written notice to LLS. If either party breaches a material obligation under the agreement and such obligation is not cured within a specified period of time following written notice from the other party, the nonbreaching party may terminate the agreement upon an additional written notice. For the year ended December 31, 2019, the Company paid \$2.9 million pursuant to the agreement as funding for the education program to increase awareness of BPDCN. As of December 31, 2019, a \$1.5 million liability is recorded in accounts payable and accrued expenses on the Company's balance sheet, payable to LLS under this agreement.

13. Stock-Based Compensation

The 2016 Plan was adopted by the board of directors and approved by the stockholders in May 2016. The 2016 Plan authorizes the Company to grant up to 1,812,932 shares of common stock to eligible employees, directors, and non-employee consultants and advisors to the Company. During June 2017, an amendment was approved by the stockholders to increase the authorized shares under the 2016 plan by 1,200,000 shares. An amendment was approved by the stockholders at the 2018 Annual Meeting of Stockholders on June 21, 2018, to increase the authorized shares under the 2016 plan by 2,900,000 shares. An amendment was approved by the stockholders at the 2019 Annual Meeting of Stockholders on June 25, 2019, to increase the authorized shares under the 2016 plan by 2,500,000 shares. Under the provisions of the 2016 Plan, no option will have a term in excess of 10 years.

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

As of December 31, 2019, there were 2,271,695 shares of common stock available for future grants under the 2016 Plan.

Total compensation cost that has been charged against operations related to the above plans was approximately \$26.4 million, \$13.9 million, and \$8.6 million for the years ended December 31, 2019, 2018 and 2017, respectively. The exercise of stock options and the vesting of restricted stock during the year ended December 31, 2019 generated an income tax deduction of approximately \$24.7 million.

The following table summarizes stock-based compensation related to the above plans by expense category for the years ended December 31, 2019, 2018 and 2017:

	Year Ended December 31,		
	2019	2018	2017
Cost of goods sold	612,012	—	—
Research and development	\$ 10,032,543	\$ 5,965,089	\$ 3,732,742
Selling, general and administrative	15,530,678	7,977,522	4,885,778
Total stock-based compensation recorded to income before incomes taxes	26,175,233	13,942,611	8,618,520
Stock-based compensation capitalized in inventory	205,000	—	—
Total stock-based compensation expense	<u>\$ 26,380,233</u>	<u>\$ 13,942,611</u>	<u>\$ 8,618,520</u>

Stock Options

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

The weighted-average key assumptions used in determining the fair value of options granted for the years ended December 31, 2019, 2018 and 2017, are as follows:

	Year Ended December 31,		
	2019	2018	2017
Risk-free interest rate	2.28%	2.91%	1.92%
Expected volatility	65.99%	75.94%	75.76%
Dividend yield	—	—	—
Expected life	6.26 years	6.26 years	6.07 years

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

For the years ended December 31, 2019, 2018 and 2017 the Company issued 479,161 shares, 169,810 shares, and 23,952 shares of the Company's common stock, respectively, upon the exercise of outstanding stock options and received proceeds of approximately \$2.1 million, \$1.6 million, and \$0.1 million, respectively.

As of December 31, 2019, there was approximately \$4.3 million of unrecognized compensation cost related to unamortized stock option compensation which is expected to be recognized over a remaining weighted-average period of approximately 2.19 years.

The Company's stock options outstanding at December 31, 2019 and changes during the year ended December 31, 2019 are presented below:

	Options	Weighted-Average Exercise Price	Weighted-Average Remaining Contractual Life	Aggregate Intrinsic Value
Outstanding at December 31, 2018	3,514,018	\$ 9.59		
Options granted	103,500	12.10		
Options exercised	(479,161)	4.53		
Options forfeited	(111,956)	12.00		
Outstanding at December 31, 2019	<u>3,026,401</u>	<u>\$ 10.39</u>	<u>5.17</u>	<u>\$ 9,317,269</u>
Options exercisable at December 31, 2019	<u>2,301,266</u>	<u>\$ 10.08</u>	<u>4.32</u>	<u>\$ 7,918,620</u>

The aggregate intrinsic value in the previous table reflects the total pretax intrinsic value (the difference between the Company's closing stock price on the last trading day of the period and the exercise price of the options, multiplied by the number of in-the-money stock options) that would have been received by the option holders had all option holders exercised their options on December 31, 2019. The intrinsic value of the Company's stock options changes based on the closing price of the Company's common stock. The total intrinsic value of options exercised (the difference in the market price of the Company's common stock on the exercise date and the price paid by the optionee to exercise the option) was approximately \$4.1 million, \$1.4 million, and \$0.2 million for the years ended December 31, 2019, 2018 and 2017, respectively.

Restricted Stock Awards

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

The Company's non-vested restricted stock at December 31, 2019 and changes during the year ended December 31, 2019 are presented below:

	<u>Number of Shares</u>	<u>Weighted-Average Grant Date Fair Value Per Share</u>
Outstanding at December 31, 2018	2,794,455	\$ 12.79
Shares granted	1,953,940	10.85
Shares vested	(1,659,770)	13.06
Shares forfeited	(86,041)	12.92
Outstanding at December 31, 2019	<u>3,002,584</u>	<u>\$ 11.37</u>

For the year ended December 31, 2019, the Company granted 1,953,940 shares of restricted stock, at a weighted-average grant date fair value of \$10.85 per share amounting to approximately \$21.2 million in total aggregate fair value. At December 31, 2019, approximately 3,002,584 shares remained unvested and there was approximately \$25.4 million of unrecognized compensation cost related to restricted stock which is expected to be recognized over a remaining weighted-average period of approximately 1.75 years.

The total fair value of shares vested during 2019, 2018 and 2017 was approximately \$21.7 million, \$5.8 million, and \$3.8 million, respectively.

Restricted Stock Units

The Company grants restricted stock units (“RSUs”) to its employees. RSUs are recorded as deferred compensation and charged against income on a straight-line basis over the vesting period, usually four years in duration. Compensation cost for RSUs is based on the grant date fair value, which is the closing market price of the Company’s common stock on the grant date, multiplied by the number of units awarded.

The following table summarizes the activity related to the Company’s RSUs for the year ended December 31, 2019:

	<u>Number of Shares</u>	<u>Weighted- Average Grant Date Fair Value Per Unit</u>
Outstanding at December 31, 2018	—	\$ —
Shares granted	93,000	12.71
Shares vested	—	—
Shares forfeited	—	—
Outstanding at December 31, 2019	<u>93,000</u>	<u>\$ 12.71</u>

For the year ended December 31, 2019, the Company granted 93,000 shares of RSUs at a weighted-average grant date fair value of \$12.71 per share amounting to approximately \$1.2 million in total aggregate fair value. As of December 31, 2019, 93,000 shares remained unvested and there was approximately \$1.1 million of unrecognized compensation cost related to restricted stock which is expected to be recognized over a remaining weighted-average period of approximately 3.58 years. The total fair value of RSUs vested during the year ended December 31, 2019 was \$0.

Performance Share Awards

On August 2018, the FDA accepted the Company’s Biologics License Application (“BLA”) for ELZONRIS for the treatment of BPDCN, in adults and in pediatric patients two years and older. As a result of the approval, the underlying performance condition associated with the performance share awards (“PSAs”) were met and the Company recognized approximately \$0.5 million and \$0.3 million of stock compensation expense related to the PSAs for the twelve-month period ended December 31, 2019 and December 31, 2018, respectively.

In addition, ELZONRIS received FDA approval on December 21, 2018 for the treatment BPDCN. As a result of the approval, the underlying performance condition associated with the PSAs were met and the Company recognized approximately \$7.5 million and \$0.9 million of stock compensation expense related to the PSAs for the years ended December 31, 2019 and December 31, 2018, respectively.

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

Awards Granted to Non-Employees

The Company grants stock options, restricted stock, and unrestricted stock to non-employee consultants. On June 20, 2018, the FASB issued ASU No. 2018-07, Compensation — Stock Compensation (Topic 718): Improvements to Nonemployee Share-Based Payment Accounting, which expands the scope of Topic 718 to include share-based payment transactions for acquiring goods and services from nonemployees. The Company early adopted ASU No. 2018-07 on April 1, 2018 and the net impact relating to the adoption was a \$0.2 million decrease to accumulated deficit for the impact prior to April 1, 2018. Total compensation cost that has been charged against operations related to stock-based awards granted to non-employee consultants was approximately \$0.6 million, \$1.9 million, and \$0.8 million for the years ended December 31, 2019, 2018 and 2017, respectively.

Employee Stock Purchase Plan

In September 2015, the Company adopted its 2015 Employee Stock Purchase Plan (the "2015 ESPP"). The 2015 ESPP is qualified as an employee stock purchase plan under Section 423 of the Internal Revenue Code of 1986, as amended (the "IRC"). Under the 2015 ESPP, the Company will grant rights to purchase shares of common stock under the 2015 ESPP at prices not less than 85% of the lesser of (i) the fair value of the shares on the date of grant of such rights or (ii) the fair value of the shares on the date such rights are exercised. Therefore, the 2015 ESPP is considered compensatory under FASB ASC 718 since, along with other factors, it includes a purchase discount of greater than 5%. For the twelve months ended December 31, 2019, 2018 and 2017, the Company recorded approximately \$266,942, \$84,678 and \$68,496, respectively, of compensation expense, related to participation in the 2015 ESPP.

14. Income Taxes

Income before provision (benefit) for taxes consists of the following:

	2019	2018	2017
Earnings before income taxes:			
United States	\$ (77,106,837)	\$ (85,024,365)	\$ (67,828,394)
Foreign	315,186	—	—
Total	<u>\$ (76,791,651)</u>	<u>\$ (85,024,365)</u>	<u>\$ (67,828,394)</u>

For the year ended December 31, 2019, 2018, and 2017, the Company recognized tax expense of (\$25,574), \$0, and \$0, respectively. For years shown, components of the Company's income tax (expense) benefit were as follows:

	2019	2018	2017
Current:			
Federal	\$ —	\$ —	\$ —
State and local	—	—	—
Foreign	(49,634)	—	—
Total current	<u>(49,634)</u>	<u>—</u>	<u>—</u>
Deferred:			
Federal	\$ 24,060	\$ —	\$ —
State and local	—	—	—
Foreign	—	—	—
Total deferred	<u>24,060</u>	<u>—</u>	<u>—</u>
Total tax expense	<u>\$ (25,574)</u>	<u>\$ —</u>	<u>\$ —</u>

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

	Year Ended December 31,		
	2019	2018	2017
Percent of pre-tax income:			
U.S. federal statutory income tax rate	(21.0)%	(21.0)%	(34.0)%
State taxes, net of federal benefit	—	8.3	(3.6)
Permanent items	1.5	1.7	5.6
R&D Credit	—	(8.4)	(14.8)
ASU 2016-09	—	—	(7.2)
Change in state deferred rate	—	—	10.8
Impact of tax reform	—	—	38.2
Deferred Tax Adjustment	1.0	1.1	—
RTP Adjustment	—	(0.1)	—
Other	—	—	—
Change in valuation allowance	18.5	18.4	5.0
Effective income tax rate	—%	—%	—%

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

	December 31,	
	2019	2018
Deferred tax assets:		
Accrued expenses	\$ 2,024,624	\$ 1,282,612
Net operating loss carryforwards	60,892,102	44,990,238
Research and Development	39,005,879	39,005,879
Depreciable and Amortizable Assets	6,062,380	7,484,108
Nonqualified stock compensation	3,138,567	3,751,544
	111,123,552	96,514,381
Less: valuation allowance	(110,841,844)	(96,514,381)
Total deferred tax assets, net of valuation allowance	281,708	—
Deferred tax liabilities: right-of-use assets	(281,708)	—
Net deferred tax asset	\$ —	\$ —

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

Due to the Company's history of losses, the deferred tax assets are fully offset by a valuation allowance at December 31, 2019, 2018 and 2017.

The following table summarizes carryforwards of net operating losses and tax credits as of December 31, 2019:

	Amount	Expiration
Federal net operating losses (pre-tax reform)	\$ 135,509,637	2023-2037
Federal net operating losses (post tax reform)	\$ 149,293,078	Unlimited
State net operating losses	\$ 288,600,546	2023-2039
Research and development credits	\$ 39,005,879	2023-2038

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

The Company did not have unrecognized tax benefits as of December 31, 2019 and does not expect this to change significantly over the next twelve months. As of December 31, 2019, the Company has not accrued interest or penalties related to uncertain tax positions. The Company's tax returns for the years ended December 31, 2010 through December 31, 2019 are still subject to examination by major tax jurisdictions.

The Company will recognize interest and penalties related to uncertain tax positions in income tax expense. As of December 31, 2019, 2018 and 2017, the Company had no accrued interest or penalties related to uncertain tax positions and no amounts have been recognized in the Company's consolidated statements of operations and comprehensive loss.

For all years through December 31, 2019, the Company generated research credits but has not conducted a study to document the qualified activities. This study may result in an adjustment to the Company's research and development credit carryforwards; however, until a study is completed and any adjustment is known, no amounts are being presented as an uncertain tax position for any years. A full valuation allowance has been provided against the Company's research and development credits and, if an adjustment is required, this adjustment would be offset by an adjustment to the deferred tax asset established for the research and development credit carryforwards and the valuation allowance.

The Company is subject to taxation in the United States, various states and foreign jurisdiction. As of December 31, 2019, the Company has no ongoing audits in any US Federal, state or foreign jurisdictions.

15. Commitments and Contingencies

License Agreements

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

Under the Company's license agreements, the Company could be required to pay up to a total of \$375.6 million upon achieving certain milestones, such as the initiation of clinical trials or the granting of patents. From inception through December 31, 2019, the Company has paid or accrued \$9.0 million in payments resulting from the execution of certain agreements, patent approvals, the initiation of sponsor research agreements, and compound development agreements. Milestone payments will also be due upon the issuance of certain patents, the initiation of certain clinical trials, the submission of regulatory applications and certain regulatory approvals, in addition to sales milestones and single digit royalties payable on commercial sales if any occur.

Scott and White Memorial Hospital

In June 2006, the Company entered into a research and license agreement, as amended in December 2008, March 2010 and July 2011 (collectively the "S&W Agreement"), with Scott and White Memorial Hospital and Scott, Sherwood and Brindley Foundation, and its affiliate Scott and White Clinic (collectively "S&W") for the rights to ELZONRIS and SL-501. ELZONRIS is a targeted therapy directed to CD123, and is being developed to treat patients with hematologic cancers. SL-501 is a next generation CD123-targeted therapy and is in preclinical development. The Company have made certain payments to Scott and White for such research services pursuant to the agreement, which through December 31, 2019, totals approximately \$1.4 million in the aggregate. The Company is required to pay single-digit royalties on sales of these products, if any, and a percentage of upfront payments the Company may receive from a sublicensee. Through December 31, 2019, the Company expensed approximately \$0.6 million relating to royalty to Scott and White. The S&W Agreement will expire in its entirety upon the later to occur of (i) the 10th anniversary of the first commercial sale of a product and (ii) the expiration of the last issued patent claiming or covering a product. The Company may terminate the S&W Agreement at its sole discretion at any time after a specified number of days following written notice and either party may terminate for a material breach of the agreement that is not cured within a specified number of days.

University of Pittsburgh

In September 2009, the Company entered into an exclusive license agreement with the University of Pittsburgh (“UP”) that covers patent rights claiming composition of a shortened variant of the IL-13R α 2 peptide (the “UP Agreement”) utilized by the Company in its SL-701 composition. The Company paid UP an upfront license fee that was expensed to research and development cost for the year ended December 31, 2009. In addition to the upfront payment, the Company is required to pay annual fees, milestone payments (which are contingent upon achievement of pre-defined clinical, regulatory and commercial events), and, upon regulatory approval, single-digit royalty payments on net sales, and a percentage of non-royalty revenue from sublicensees, which decreases if the applicable sublicense agreement is entered into after a certain clinical milestone has been met. Through December 31, 2019, the Company has paid an aggregate of approximately \$0.8 million in fees to the University under the agreement. The Company also must make certain payments to the University of up to approximately \$4.2 million upon the achievement of specific regulatory and commercial milestone events. The UP Agreement will expire in its entirety upon the expiration of the last issued patent claiming or covering the product. The Company may terminate the UP Agreement at its sole discretion at any time after a specified number of days following written notice and UP may terminate for a material breach of the agreement by the Company that is not cured within a specified number of days.

In March 2012, the Company entered into a non-exclusive license agreement with UP that covers patent rights claiming use of a shortened peptide of EphA2, which the Company may use for the diagnosis, treatment or prevention of diseases and tumors of the brain and which the Company is currently utilizing in its SL-701 composition. The Company paid UP an initial license fee and is required to pay UP annual license maintenance fees until the first commercial sale of a licensed product, a single-digit royalty on sales, and a minimum annual royalty following the first commercial sale of a licensed product. Through December 31, 2019, the Company has paid an aggregate of approximately \$0.1 million in fees to the University under the agreement. The UP Agreement will expire in its entirety upon the expiration of the last issued patent claiming or covering the product. The Company may terminate the UP Agreement at its sole discretion at any time after a specified number of days following written notice and UP may terminate for a material breach of the agreement by the Company that is not cured within a specified number of days.

In March 2012, the Company entered into a non-exclusive license agreement with UP for the right to use certain information and data contained in the applications for investigational new drugs, or INDs, relating to clinical trials with an earlier version of SL-701 that were conducted by UP. The Company may use the information and data for the development, manufacture, regulatory approval and commercialization of pharmaceutical products. The Company paid UP an initial license fee and will be required to make a payment following a specified regulatory milestone, and a percentage of non-royalty revenue it may receive from any sublicensees. Through December 31, 2019, the Company has paid an aggregate of approximately \$27,500 in fees to the University under the agreement. The UP Agreement will expire in its entirety in March 2032 unless earlier terminated by a party. The Company may terminate the UP Agreement at its sole discretion at any time prior to incorporating or referencing the data or UP INDs, after a specified number of days following written notice, and UP may terminate for a material breach of the agreement by the Company that is not cured within a specified number of days or if the IL-13R α 2 license agreement is terminated.

Leukemia and Lymphoma Society

In October 2013, the Company entered into a contract relating to the Therapy Acceleration Program (“the TAP Agreement”) with the LLS. LLS is a national voluntary health agency which, among other activities, encourages and sponsors research relating to blood cancers to develop therapies to cure or mitigate these diseases. To further its mission, LLS provides research funding to entities that can demonstrate, after LLS’s review process, that their proposed research projects have scientific promise to advance LLS’s effort to find treatments and cures for blood cancers and their complications. Pursuant to the TAP Agreement, LLS agreed to provide funding to the Company not to exceed \$3.5 million to fund the Company’s development program related to the Company’s preclinical and clinical product development activities as well as providing an educational program to increase physician & patient awareness of BPDCN. Through December 31, 2019, the Company has received the full \$3.5 million based on milestones achieved. In addition, Stemline has paid \$2.9 million pursuant to the agreement as funding for the education program to increase awareness of BPDCN. As at December 31, 2019, a \$1.5 million liability is recorded in accounts payable and accrued expenses on the Company’s balance sheet, payable to LLS under this agreement.

In consideration of funding by LLS and transfer to the Company of any rights LLS may have to any project inventions developed during the term of the agreement, the Company may be required to pay LLS a cash multiple on the LLS funding, (LLS funding is the \$3.5 million less any amount Stemline contributes to LLS to support the Company's preclinical and clinical development activities to bring ELZONRIS to commercialization). The total amount payable by the Company to LLS will not exceed three (3) times the amount of net funding received from LLS of \$2.9 million. The Company is required to make a one-time payment upon regulatory approval and commercialization of ELZONRIS. Additionally, the Company is required to make payments based on achievement of specific sale-based commercial milestones. The Company may terminate the license for any or no reason upon 60 days advance written notice to LLS. If either party breaches a material obligation under the agreement and such obligation is not cured within a specified period of time following written notice from the other party, the non-breaching party may terminate the agreement upon an additional written notice.

CanBas, Ltd

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

The Company is responsible to pay the remaining technical advisory fees over the next 1.5 years totaling 165 million Japanese Yen (JPY), if the clinical development continues over this time period. Additionally, the Company must pay CanBas tiered royalties based on aggregate net sales, by the Company or its sublicensees, of products containing the licensed compound until the latest date of a period of ten years following the first commercial sale of each product in each country; the date upon which there are no more valid claims or the expiration or termination of the last regulatory exclusivity period. The royalty rates start in the low single digits and are tiered up based on annual net sales. In the future, the Company may also be responsible, based on the achievement of specific clinical-development, regulatory and sales-based commercial milestones, for certain payments to CanBas of up to \$86 million. The Company has sublicensing rights under this agreement, in which the Company would pay CanBas a standard percentage of the payments received by the Company from a sublicensee.

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

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

UCB Biopharma Sprl

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

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

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

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

CRT Pioneer Fund

In March 2019, the Company entered into a license agreement with CRT Pioneer Fund ("CRT") for SL-1001. SL-1001 is an oral, selective small molecule RET kinase inhibitor. The Company currently conducting the necessary non-clinical studies to enable a regulatory filing to enable first-in-human studies.

CRT has granted the Company an exclusive, royalty-bearing, worldwide license, under certain patent rights, know-how and materials to research, develop, make, have made, formulate, use, sell, offer to sell and import SL-1001, and any products containing or comprising such compound in finished dosage pharmaceutical form. In March 2019, the Company paid CRT an up-front consideration payment of \$1.0 million. In the future, the Company may also be responsible, based on the achievement of specific clinical development, regulatory and sales-based commercial milestones, for certain payments to CRT of up to \$159 million, largely consisting of approval and post-approval payments. The Company has sublicensing rights under this agreement, in which the Company would pay CRT a standard percentage of the payments received by the Company from a sublicensee.

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

The agreement survives until the date upon which the Company's obligation to pay royalties has expired in each specific country, after which the Company's license becomes fully paid, irrevocable, perpetual, non-exclusive and royalty-free. The Company may terminate the license upon 90 days advance written notice to CRT. If either party breaches a material obligation under the agreement and such obligation is not cured within a specified period of time following written notice from the other party, the non-breaching party may terminate the agreement upon an additional written notice.

Other

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

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

Contractual Agreements

The Company has entered into contracts with a pharmaceutical drug substance manufacturer over the past six years totaling \$37.8 million, with services to be rendered on some of these agreements through 2020. From inception through December 31, 2019, the Company has received and paid for services relating to these agreements in the amount of \$32.1 million. In addition, the Company has a commercial supply agreement with a vendor in which the Company is required to manufacture at least one batch during 2020.

The Company has agreements in place with CROs in connection with its clinical programs. The Company's total expenditures in the future would be approximately \$1.1 million assuming the successful advancement of its programs.

Contingencies

On March 15, 2018, the United States District Court for the Southern District of New York ("SDNY") granted a motion to dismiss in its entirety a consolidated shareholder action against the Company, its directors, certain of its officers, and the lead underwriter. This matter originated from lawsuits filed in February 2017. On March 12, 2019, Lead Plaintiffs filed a joint stipulation of settlement in support of their motion for preliminary approval of a settlement of the consolidated shareholder action with the SDNY, after the Plaintiffs voluntarily withdrew their appeal pursuant to Local Rule 42.1. On June 14, 2019, the SDNY entered an order granting preliminary approval of the settlement of the consolidated securities action and, on September 23, 2019, the SDNY granted final approval of the settlement.

On June 25, 2019, a shareholder derivative action was filed in the Supreme Court of the State of New York, New York County. The Company is named solely as a nominal defendant. The suit seeks damages and equitable relief related to the Company's directors' compensation in each year of 2013 through 2018. Given the early stages of this matter, at this time, it is not possible to estimate the amount of any loss, or whether there will be any loss at all.

16. Leases

The Company entered into an operating lease arrangement dated November 1, 2019 for office space in Switzerland. The lease expires in March 2023 and includes approximately \$0.4 million of legally binding minimum lease payments.

As described in Note 1, effective January 1, 2019, the Company adopted ASC 842 using the optional transition method. In accordance with the optional transition method, the Company did not recast the prior period consolidated financial statements and all prior period amounts and disclosures are presented under ASC 840. Finance leases are not material to the consolidated financial statements and are therefore not included in the following disclosures.

Supplemental balance sheet information	December 31, 2019
Operating lease right-of-use assets	\$ 1,317,598
Operating lease liabilities - current portion	1,169,764
Operating lease liabilities	226,306
Total operating lease liabilities	\$ 1,396,070
Weighted Average Remaining Lease Term — Operating Leases	1.75 years
Weighted Average Discount Rate — Operating Leases	7.49%

Supplemental cash flow information related to leases was as follows:

Cash paid for amounts included in the measurement of lease liabilities:	December 31, 2019
Operating cash flows from operating leases	\$ 1,135,686

Maturities of operating lease liabilities as at December 31, 2019 were as follows:

Year Ending December 31,	
2020	\$ 1,227,123
2021	109,323
2022	109,323
2023	27,332
Total future minimum lease payments	1,473,101
Less: imputed interest	(77,031)
Present value of operating lease liabilities	\$ 1,396,070

The Company recognized rent expense of \$1.2 million for the year ended December 31, 2019, primarily related to leases classified as operating leases under ASC 842. Rent expense primarily related to leases classified as operating leases under ASC 840 was \$0.9 million and \$0.8 million for the years ended December 31, 2018, and 2017, respectively. Rent expense is included in selling, general and administrative expenses in the Company's consolidated statements of operations.

As of December 31, 2018, future minimum lease payments under long-term non-cancelable operating leases as classified under ASC 840 were as follows:

Year Ending December 31,	
2019	\$ 1,011,420
2020	847,620
2021	4,425
Total minimum lease payments	\$ 1,863,465

17. Subsequent Events

On March 3, 2020, the Company entered into a lease agreement for office space located at 750 Lexington Avenue, Fourth Floor, New York, New York, with lease obligations of approximately \$9.0 million. The commencement date of the lease is being finalized but will not be before May 1, 2020. The lease will expire in 2028.

18. Selected Quarterly Financial Data (Unaudited)

	Quarters Ended			
	March 31	June 30	September 30	December 31
2019				
Net loss attributable to common stockholders	\$ (27,407,266)	\$ (16,841,422)	\$ (14,888,512)	\$ (17,680,025)
Basic and diluted net loss per common share	\$ (0.73)	\$ (0.42)	\$ (0.34)	\$ (0.38)
2018				
Net loss attributable to common stockholders	\$ (18,416,753)	\$ (18,928,703)	\$ (21,043,996)	\$ (26,634,914)
Basic and diluted net loss per common share	\$ (0.69)	\$ (0.66)	\$ (0.73)	\$ (0.92)

SIGNATURES

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

Date: March 16, 2020

STEMLINE THERAPEUTICS, INC.

By: /s/ Ivan Bergstein, M.D.

Ivan Bergstein, M.D.

Chairman, President and Chief Executive Officer
(Principal Executive Officer)

Date: March 16, 2020

By: /s/ David G. Gionco

David G. Gionco

Senior Vice President of Finance and Chief Accounting Officer
(Principal Financial and Accounting Officer)

POWER OF ATTORNEY

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

Pursuant to the requirements of the Securities Exchange Act of 1934, as amended, this Form 10-K has been signed by the following persons on behalf of the Registrant on March 16, 2020, and in the capacities indicated:

Signatures	Title
<u>/s/ Ivan Bergstein, M.D.</u> Ivan Bergstein, M.D.	Chairman, President and Chief Executive Officer (Principal Executive Officer)
<u>/s/ David G. Gionco</u> David G. Gionco	Senior Vice President of Finance and Chief Accounting Officer (Principal Financial and Accounting Officer)
<u>/s/ Ron Bentsur</u> Ron Bentsur	Director
<u>/s/ Darren Cline</u> Darren Cline	Director
<u>/s/ Alan Forman</u> Alan Forman	Director
<u>/s/ Daniel Hume</u> Daniel Hume	Director
<u>/s/ Mark Sard</u> Mark Sard	Director
<u>/s/ Kenneth Zuerblis</u> Kenneth Zuerblis	Director

**DESCRIPTION OF THE REGISTRANT'S SECURITIES
REGISTERED PURSUANT TO SECTION 12 OF THE
SECURITIES EXCHANGE ACT OF 1934**

When used herein, the terms "we," "our," and "us" refer to Stemline Therapeutics, Inc.

DESCRIPTION OF COMMON STOCK

Common Stock

Under the terms of our restated certificate of incorporation, as amended, we are authorized to issue a total of 83,750,000 shares of common stock, par value \$0.0001 per share, and 5,000,000 shares of preferred stock, par value \$0.0001 per share. 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 of March 16, 2020, there were 50,375,079 shares of our common stock issued and outstanding, held by 188 record stockholders.

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 sole discretion of our board of directors.

Voting Rights

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 restated certificate of incorporation that relates solely to the terms of one or more outstanding series of preferred stock. An election of directors by our stockholders will 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.

Liquidation and Dissolution

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, conversion or similar 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.

Transfer Agent

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

Anti-Takeover Provisions***Section 203 of the Delaware General Corporation Law***

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 restated certificate of incorporation, as amended, and our amended and restated by-laws 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 amended and restated 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.

Stockholder action; special meeting of stockholders

Our restated certificate of incorporation, as amended, 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 restated certificate of incorporation, as amended, and our amended and restated by-laws 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.

DESCRIPTION OF PREFERRED STOCK

Preferred Stock

Under the terms of our restated certificate of incorporation, as amended, our board of directors is authorized to issue up to 5,000,000 shares of preferred stock, par value \$0.0001 per share. Our board of directors may issue shares of preferred stock in one or more series without stockholder approval, and 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. We may amend from time to time our restated certificate of incorporation, as amended, to increase the number of authorized shares of preferred stock. Any such amendment would require the approval of the holders of a majority of the voting power of the shares entitled to vote thereon. As of March 16, 2020, we have 5,000,000 shares of preferred shares authorized, but no shares of preferred stock outstanding.

It is not possible to state the actual effect of the issuance of any shares of preferred stock upon the rights of the holders of common stock until the board of directors determines the specific rights of the holders of preferred stock. However, effects of the issuance of preferred stock include restricting dividends on common stock, diluting the voting power of common stock, impairing the liquidation rights of common stock, and making it more difficult for a third party to acquire us, which could have the effect of discouraging a third party from acquiring, or deterring a third party from paying a premium to acquire, a majority of our outstanding voting stock. The preferred stock will, when issued, be fully paid and non-assessable.

DESCRIPTION OF WARRANTS

We may issue warrants to purchase shares of our common stock and/or preferred stock in one or more series together with other securities or separately.

DESCRIPTION OF DEBT SECURITIES

We may offer debt securities which may be senior, subordinated or junior subordinated and may be convertible. The terms of our debt securities will be issued in one or more series under an indenture to be entered into between us and a trustee. The terms of the debt securities will include those stated in the indenture and those made part of the indenture by reference to the Trust Indenture Act of 1939, as in effect on the date of the indenture. We have filed a copy of the form of indenture as an exhibit to our Form S-3 Registration Statement on March 15, 2019. The indenture will be subject to and governed by the terms of the Trust Indenture Act of 1939.

Debt Securities

The aggregate principal amount of debt securities that may be issued under the indenture is unlimited. The debt securities may be issued in one or more series as may be authorized from time to time pursuant to a supplemental indenture entered into between us and the trustee or an order delivered by us to the trustee.

General

One or more series of debt securities may be sold as “original issue discount” securities. These debt securities would be sold at a substantial discount below their stated principal amount, bearing no interest or interest at a rate which at the time of issuance is below market rates. One or more series of debt securities may be variable rate debt securities that may be exchanged for fixed rate debt securities.

Debt securities may be issued where the amount of principal and/or interest payable is determined by reference to one or more currency exchange rates, commodity prices, equity indices or other factors. Holders of such debt securities may receive a principal amount or a payment of interest that is greater than or less than the amount of principal or interest otherwise payable on such dates, depending upon the value of the applicable currencies, commodities, equity indices or other factors.

The term “debt securities” includes debt securities denominated in U.S. dollars or, if specified in the applicable prospectus supplement, in any other freely transferable currency or units based on or relating to foreign currencies.

Global Securities

The debt securities of a series may be issued in whole or in part in the form of one or more global securities that will be deposited with, or on behalf of, a depository identified in the prospectus supplement. Global securities will be issued in registered form and in either temporary or definitive form. Unless and until it is exchanged in whole or in part for the individual debt securities, a global security may not be transferred except as a whole by the depository for such global security to a nominee of such depository or by a nominee of such depository to such depository or another nominee of such depository or by such depository or any such nominee to a successor of such depository or a nominee of such successor. The specific terms of the depository arrangement with respect to any debt securities of a series and the rights of and limitations upon owners of beneficial interests in a global security will be described in the applicable prospectus supplement.

Governing Law

The indenture and the debt securities shall be construed in accordance with and governed by the laws of the State of New York.

DESCRIPTION OF UNITS

We may issue, in one more series, units comprised of shares of our common stock or preferred stock, warrants to purchase common stock or preferred stock, debt securities or any combination of those securities. Each unit will be issued so that the holder of the unit is also the holder of each security included in the unit. Thus, the holder of a unit will have the rights and obligations of a holder of each included security. The unit agreement under which a unit is issued may provide that the securities included in the unit may not be held or transferred separately, at any time or at any time before a specified date.

We may evidence units by unit certificates that we issue under a separate agreement. We may issue the units under a unit agreement between us and one or more unit agents. If we elect to enter into a unit agreement with a unit agent, the unit agent will act solely as our agent in connection with the units and will not assume any obligation or relationship of agency or trust for or with any registered holders of units or beneficial owners of units. We will indicate the name and address and other information regarding the unit agent in the applicable prospectus supplement relating to a particular series of units if we elect to use a unit agent.

STEMLINE THERAPEUTICS, INC.
List of Subsidiaries

Stemline Therapeutics, Inc. does not have any subsidiaries.

Consent of Independent Registered Public Accounting Firm

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

- (1) Registration Statement (Form S-8 No. 333-188115) pertaining to Stemline Therapeutics, Inc. 2012 Equity Incentive Plan and Stemline Therapeutics, Inc. Amended and Restated 2004 Employee, Director and Consultant Stock Plan,
- (2) Registration Statement (Form S-8 No. 333-206303) pertaining to Stemline Therapeutics, Inc. 2015 Employee Stock Purchase Plan,
- (3) Registration Statements (Form S-8 No. 333-211784, Form S-8 No. 333-219796, Form S-8 No. 333-226049, and, Form S-8 No. 333-232445) pertaining to Stemline Therapeutics, Inc. 2016 Equity Incentive Plan,
- (4) Registration Statement (Form S-3 No. 333-219794) of Stemline Therapeutics, Inc., and
- (5) Registration Statement (Form S-3 No. 333-230341) of Stemline Therapeutics, Inc.;

of our reports dated March 16, 2020, with respect to the financial statements of Stemline Therapeutics, Inc. and the effectiveness of internal control over financial reporting of Stemline Therapeutics, Inc. included in this Annual Report (Form 10-K) of Stemline Therapeutics, Inc. for the year ended December 31, 2019.

/s/ Ernst & Young LLP

Stamford, Connecticut
March 16, 2020

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

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

1. I have reviewed this annual report on Form 10-K of Stemline Therapeutics, Inc.;
2. Based on my knowledge, this report does not contain any untrue statement of a material fact or omit to state a material fact necessary to make the statements made, in light of the circumstances under which such statements were made, not misleading with respect to the period covered by this report;
3. Based on my knowledge, the financial statements, and other financial information included in this report, fairly present in all material respects the financial condition, results of operations and cash flows of the registrant as of, and for, the periods presented in this report;
4. The registrant's other certifying officer(s) and I are responsible for establishing and maintaining disclosure controls and procedures (as defined in Exchange Act Rules 13a-15(e) and 15d-15(e)) and internal control over financial reporting (as defined in Exchange Act Rules 13a-15(f) and 15d-15(f)) for the registrant and have:
 - a) Designed such disclosure controls and procedures, or caused such disclosure controls and procedures to be designed under our supervision, to ensure that material information relating to the registrant, including its consolidated subsidiaries, is made known to us by others within those entities, particularly during the period in which this report is being prepared;
 - b) Designed such internal control over financial reporting, or caused such internal control over financial reporting to be designed under our supervision, to provide reasonable assurance regarding the reliability of financial reporting and the preparation of financial statements for external purposes in accordance with generally accepted accounting principles;
 - c) Evaluated the effectiveness of the registrant's disclosure controls and procedures and presented in this report our conclusions about the effectiveness of the disclosure controls and procedures, as of the end of the period covered by this report based on such evaluation; and
 - d) Disclosed in this report any change in the registrant's internal control over financial reporting that occurred during the registrant's most recent fiscal quarter (the registrant's fourth fiscal quarter in the case of an annual report) that has materially affected, or is reasonably likely to materially affect, the registrant's internal control over financial reporting; and
5. The registrant's other certifying officer and I have disclosed, based on our most recent evaluation of internal control over financial reporting, to the registrant's auditors and the audit committee of the registrant's board of directors (or persons performing the equivalent functions):
 - a) All significant deficiencies and material weaknesses in the design or operation of internal control over financial reporting which are reasonably likely to adversely affect the registrant's ability to record, process, summarize and report financial information; and
 - b) Any fraud, whether or not material, that involves management or other employees who have a significant role in the registrant's internal control over financial reporting.

Date: March 16, 2020

/s/ Ivan Bergstein, M.D.

Ivan Bergstein, M.D.

Chief Executive Officer

Principal Executive Officer

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

I, David G. Gionco, certify that:

1. I have reviewed this annual report on Form 10-K of Stemline Therapeutics, Inc.;
2. Based on my knowledge, this report does not contain any untrue statement of a material fact or omit to state a material fact necessary to make the statements made, in light of the circumstances under which such statements were made, not misleading with respect to the period covered by this report;
3. Based on my knowledge, the financial statements, and other financial information included in this report, fairly present in all material respects the financial condition, results of operations and cash flows of the registrant as of, and for, the periods presented in this report;
4. The registrant's other certifying officer(s) and I are responsible for establishing and maintaining disclosure controls and procedures (as defined in Exchange Act Rules 13a-15(e) and 15d-15(e)) and internal control over financial reporting (as defined in Exchange Act Rules 13a-15(f) and 15d-15(f)) for the registrant and have:
 - a) Designed such disclosure controls and procedures, or caused such disclosure controls and procedures to be designed under our supervision, to ensure that material information relating to the registrant, including its consolidated subsidiaries, is made known to us by others within those entities, particularly during the period in which this report is being prepared;
 - b) Designed such internal control over financial reporting, or caused such internal control over financial reporting to be designed under our supervision, to provide reasonable assurance regarding the reliability of financial reporting and the preparation of financial statements for external purposes in accordance with generally accepted accounting principles;
 - c) Evaluated the effectiveness of the registrant's disclosure controls and procedures and presented in this report our conclusions about the effectiveness of the disclosure controls and procedures, as of the end of the period covered by this report based on such evaluation; and
 - d) Disclosed in this report any change in the registrant's internal control over financial reporting that occurred during the registrant's most recent fiscal quarter (the registrant's fourth fiscal quarter in the case of an annual report) that has materially affected, or is reasonably likely to materially affect, the registrant's internal control over financial reporting; and
5. The registrant's other certifying officer and I have disclosed, based on our most recent evaluation of internal control over financial reporting, to the registrant's auditors and the audit committee of the registrant's board of directors (or persons performing the equivalent functions):
 - a) All significant deficiencies and material weaknesses in the design or operation of internal control over financial reporting which are reasonably likely to adversely affect the registrant's ability to record, process, summarize and report financial information; and
 - b) Any fraud, whether or not material, that involves management or other employees who have a significant role in the registrant's internal control over financial reporting.

Date: March 16, 2020

/s/ David G. Gionco

David G. Gionco

Chief Accounting Officer

Principal Financial and Accounting Officer

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

In connection with the annual report of Stemline Therapeutics, Inc. (the "Company") on Form 10-K for the period ended December 31, 2019 as filed with the Securities and Exchange Commission (the "Report"), I, Ivan Bergstein, M.D., Chief Executive Officer of the Company, certify, pursuant to 18 U.S.C. §1350, as adopted pursuant to §906 of the Sarbanes-Oxley Act of 2002, that, based on my knowledge:

- 1) The Report fully complies with the requirements of Section 13(a) or 15(d) of the Securities Exchange Act of 1934, as amended; and
- 2) The information contained in the Report fairly presents, in all material respects, the financial condition and results of operations of the Company.

Date: March 16, 2020

/s/ Ivan Bergstein, M.D.

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

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

In connection with the annual report of Stemline Therapeutics, Inc. (the "Company") on Form 10-K for the period ended December 31, 2019 as filed with the Securities and Exchange Commission (the "Report"), I, David G. Gionco, Chief Accounting Officer of the Company, certify, pursuant to 18 U.S.C. §1350, as adopted pursuant to §906 of the Sarbanes-Oxley Act of 2002, that, based on my knowledge:

- 1) The Report fully complies with the requirements of Section 13(a) or 15(d) of the Securities Exchange Act of 1934, as amended; and
- 2) The information contained in the Report fairly presents, in all material respects, the financial condition and results of operations of the Company.

Date: March 16, 2020

/s/ David G. Gionco

David G. Gionco

Chief Accounting Officer

Principal Financial and Accounting Officer
