UNITED STATES
SECURITIES AND EXCHANGE COMMISSION

Washington, D.C. 20549

FORM 10-Q

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

For the quarterly period ended March 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	**45-0522567**
(State or other jurisdiction of incorporation or organization)	(I.R.S. Employer Identification No.)

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

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

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 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	☐	Accelerated filer	☒
Non-accelerated filer	☐	Smaller reporting company	☒
		Emerging growth company	☐

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

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

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

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

There were 43,684,777 shares of the registrant's common stock, $0.0001 par value, outstanding as of May 9, 2019.

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This Quarterly Report on Form 10-Q 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 Quarterly Report on Form 10-Q are the property of their respective owners.

SPECIAL CAUTIONARY NOTICE REGARDING FORWARD-LOOKING STATEMENTS

This quarterly report on Form 10-Q ("Form 10-Q") 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-Q and include statements regarding our intentions, beliefs, projections, outlook, analyses or current expectations concerning, among other things, our history of net operating losses and uncertainty regarding our ability to obtain capital and achieve profitability, our ability to develop and commercialize our product candidates, our ability to advance our development programs, enroll our trials, and achieve clinical endpoints, our ability to use or expand our technology to build a pipeline of product candidates, our ability to obtain and maintain regulatory approval of our product candidates and comply with ongoing regulatory requirements, our ability to successfully operate in a competitive industry and gain market acceptance by physician, provider, patient, and payor communities, our reliance on third parties, unstable economic or market conditions, and our ability to obtain and adequately protect intellectual property rights for our product candidates.

By their nature, forward-looking statements involve risks and uncertainties because they relate to events, competitive dynamics, and healthcare, regulatory and scientific developments and depend on the economic circumstances that may or may not occur in the future or may occur on longer or shorter timelines than anticipated. Although we believe that we have a reasonable basis for each forward-looking statement contained in this Form 10-Q, 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-Q. 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-Q, 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., Europe, and other regions;
- the success of our commercial infrastructure buildout in the U.S., Europe, and other regions;
- the success and timing of our clinical trials and preclinical studies for our product candidates, including site initiation, institutional review board approval, scientific review committee approval, patient accrual, safety, tolerability and efficacy data observed;
- the success and timing of our regulatory filings for ELZONRIS or any of our product candidates, including for approval in the U.S., Europe, and other regions;
- 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 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 in the U.S., Europe and other regions;
- the loss of key scientific or management personnel;
- changes in regulations in the U.S., Europe and other regions;
- 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 product candidates;
- our ability to maintain the license agreements for our products and product candidates;
- our ability to obtain and maintain authorization from regulatory authorities for use of our products and product candidates for the initiation and conduct of clinical trials;
- 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; and
- our ability to gain access to products we 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-Q 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-Q. You should also read carefully the factors described in the "Risk Factors" section of this Form 10-Q 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-Q.

This Form 10-Q 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: FINANCIAL INFORMATION

Item 1. Financial Statements.

STEMLINE THERAPEUTICS, INC.
Balance Sheets

	March 31, 2019 (Unaudited)	December 31, 2018
Assets		
Current assets:		
Cash and cash equivalents	$ 24,014,510	$ 9,443,667
Short-term investments	100,337,983	50,662,189
Accounts receivable	5,618,900	—
Inventories	848,493	—
Prepaid expenses and other current assets	3,120,926	2,952,996
Total current assets	133,940,812	63,058,852
Property and equipment, net	273,399	222,413
Right-of-use asset, net	1,738,680	—
Other assets	212,305	212,305
Total assets	$ 136,165,196	$ 63,493,570
Liabilities and stockholders' equity		
Current liabilities:		
Accounts payable and accrued expenses	$ 25,413,021	$ 21,153,062
Right-of-use liability - current portion	1,023,678	—
Other current liabilities	6,021	65,862
Total current liabilities	26,442,720	21,218,924
Right-of-use liability	818,303	—
Other liabilities	12,011	72,591
Total liabilities	27,273,034	21,291,515
Stockholders' equity:		
Preferred stock $0.0001 par value, 5,000,000 shares authorized, none issued and outstanding at March 31, 2019 and December 31, 2018	—	—
Common stock $0.0001 par value, 53,750,000 shares authorized at March 31, 2019 and December 31, 2018. 43,576,081 shares issued and outstanding at March 31, 2019 and 31,943,186 shares issued and outstanding at December 31, 2018	4,358	3,194
Additional paid-in capital	425,410,295	331,343,484
Accumulated other comprehensive loss	(27,161)	(56,559)
Accumulated deficit	(316,495,330)	(289,088,064)
Total stockholders' equity	108,892,162	42,202,055
Total liabilities and stockholders' equity	$ 136,165,196	$ 63,493,570

See accompanying unaudited notes.

STEMLINE THERAPEUTICS, INC.
Statements of Operations
(Unaudited)

	Three Months Ended March 31,	
	2019	**2018**
Revenue:		
Product revenue, net	$ 5,048,590	$ —
Operating expenses:		
Cost of goods sold	85,728	—
Research and development	16,953,822	12,708,058
Selling, general and administrative	15,953,968	5,938,600
Total operating expenses	32,993,518	18,646,658
Loss from operations	(27,944,928)	(18,646,658)
Other expense	(4,616)	(3,897)
Interest income	538,584	233,802
Net loss before income taxes	(27,410,960)	(18,416,753)
Income tax benefit	3,694	—
Net loss	$ (27,407,266)	$ (18,416,753)
Net loss per common share:		
Basic and Diluted	$ (0.73)	$ (0.69)
Weighted-average shares outstanding:		
Basic and Diluted	37,550,931	26,845,983

See accompanying unaudited notes.

STEMLINE THERAPEUTICS, INC.
Statements of Comprehensive Loss
(Unaudited)

	Three Months Ended March 31,	
	2019	**2018**
Net loss	$ (27,407,266)	$ (18,416,753)
Other comprehensive gain (loss):		
Unrealized gain (loss) on investments, net of tax	25,074	(8,316)
Reclassification adjustment for loss on investments included in net loss	4,324	3,897
Other comprehensive gain (loss)	29,398	(4,419)
Comprehensive loss	$ (27,377,868)	$ (18,421,172)

See accompanying unaudited notes.

STEMLINE THERAPEUTICS, INC.
Statement of Stockholders' Equity
(Unaudited)

	Common Stock		Additional Paid-in Capital	Accumulated Other Comprehensive Income (Loss)	Accumulated Deficit	Total Stockholders' Equity (Deficit)
	Shares	Capital				
Balance, December 31, 2018	31,943,186	$ 3,194	$ 331,343,484	$ (56,559)	$ (289,088,064)	$ 42,202,055
Stock award — in-licensing	43,822	4	500,005	—	—	500,009
Restricted stock grants	1,366,471	137	(137)	—	—	—
Forfeiture of restricted stock grants	(13,125)	(1)	1	—	—	—
Stock-based compensation expense	—	—	7,206,092	—	—	7,206,092
Employee Stock Purchase Plan compensation expense	—	—	24,853	—	—	24,853
Issuance of common stock in connection with the ESPP	11,005	1	102,565	—	—	102,566
Issuance of common stock in connection with the exercise of stock options	2,500	—	15,825	—	—	15,825
Issuance of common stock in connection with secondary public offering, net	10,222,222	1,023	86,217,607	—	—	86,218,630
Net loss	—	—	—	—	(27,407,266)	(27,407,266)
Other comprehensive income	—	—	—	29,398	—	29,398
Balance, March 31, 2019	43,576,081	$ 4,358	$ 425,410,295	$ (27,161)	$ (316,495,330)	$ 108,892,162

	Common Stock		Additional Paid-in Capital	Accumulated Other Comprehensive Loss	Accumulated Deficit	Total Stockholders' Equity (Deficit)
	Shares	Capital				
Balance, December 31, 2017	25,313,595	$ 2,531	$ 251,489,546	$ (145,958)	$ (204,275,690)	$ 47,070,429
Restricted stock grants	541,254	54	(54)	—	—	—
Stock-based compensation expense	—	—	2,206,427	—	—	2,206,427
Employee Stock Purchase Plan compensation expense	—	—	18,876	—	—	18,876
Issuance of common stock in connection with the ESPP	6,650	1	49,741	—	—	49,742
Issuance of common stock in connection with the exercise of stock options	69,201	7	1,089,909	—	—	1,089,916
Issuance of common stock in connection with the exercise of warrants	30,830	3	352,527	—	—	352,530
Issuance of common stock in connection with secondary public offering, net	4,255,000	426	55,650,476	—	—	55,650,902
Net loss	—	—	—	—	(18,416,753)	(18,416,753)
Other comprehensive loss	—	—	—	(4,419)	—	(4,419)
Balance, March 31, 2018	30,216,530	$ 3,022	$ 310,857,448	$ (150,377)	$ (222,692,443)	$ 88,017,650

See accompanying unaudited notes.

STEMLINE THERAPEUTICS, INC.
Statements of Cash Flows
(Unaudited)

	Three Months Ended March 31,	
	2019	2018
Cash flows from operating activities		
Net loss	$ (27,407,266)	$ (18,416,753)
Adjustments to reconcile net loss to net cash used in operating activities:		
Depreciation	23,510	11,786
Stock-based compensation expense	7,206,092	2,206,427
Stock award — in-licensing	500,009	—
Employee Stock Purchase Plan compensation expense	24,853	18,876
Amortization of premium paid on marketable securities	(96,065)	(3,100)
Net loss on sale of marketable securities	4,324	3,897
Changes in operating assets and liabilities:		
Accounts receivable	(5,618,900)	—
Inventories	(848,493)	—
Prepaid expenses and other current assets	(167,930)	(628,575)
Right-of-use asset, net	(1,738,680)	—
Accounts payable and accrued expenses	4,259,959	(253,580)
Right-of-use liability - current portion	1,023,678	—
Right-of-use liability	818,303	—
Other liabilities	(120,421)	(24,205)
Net cash used in operating activities	(22,137,027)	(17,085,227)
Cash flows from investing activities		
Purchase of fixed assets	(74,495)	—
Purchase of marketable securities	(81,499,610)	(44,688,793)
Sale and maturities of marketable securities	31,944,954	15,066,670
Net cash used in investing activities	(49,629,151)	(29,622,123)
Cash flows from financing activities		
Proceeds from issuance of common stock from follow-on public offering, net	86,218,630	55,650,902
Proceeds from issuance of common stock from ESPP	102,566	49,742
Proceeds from exercise of stock options	15,825	1,089,916
Proceeds from exercise of warrants	—	352,530
Net cash provided by financing activities	86,337,021	57,143,090
Net increase in cash and cash equivalents	14,570,843	10,435,740
Cash and cash equivalents at beginning of period	9,443,667	4,795,098
Cash and cash equivalents at end of period	$ 24,014,510	$ 15,230,838

See accompanying unaudited notes.

STEMLINE THERAPEUTICS, INC.
Notes to Unaudited Financial Statements
March 31, 2019

1. Organization and Basis of Presentation

Organization

Stemline Therapeutics, Inc. (the "Company") is a commercial-stage biopharmaceutical company focused on discovering, acquiring, developing and commercializing novel oncology therapeutics. The Company's activities to date have primarily consisted of advancing ELZONRIS™ (tagraxofusp-erzs; SL-401) through the clinical and regulatory process, launching and commercializing ELZONRIS, preparing for this launch and commercialization efforts including building out a sales, marketing, and reimbursement infrastructure, developing and implementing its global regulatory and commercial strategies, developing its clinical and preclinical stage programs including ELZONRIS in additional indications and other product candidates, expanding and strengthening its intellectual property portfolio, identifying and acquiring additional product and technology rights, investor relations efforts, and raising capital. The Company was incorporated in Delaware on August 8, 2003 and has its principal office in New York, New York.

The Company has incurred losses from operations since inception of $328.2 million. Since its inception, most of its resources have been dedicated to the discovery, acquisition, preclinical and clinical development, regulatory strategy and implementation, and commercialization. In particular, it has expended and will continue to expend, substantial resources for the foreseeable future commercializing its approved product, developing its approved product for potential additional indications, developing its additional clinical stage product candidates, developing its preclinical stage product candidates, and continuing its asset acquisition efforts. These expenditures include costs associated with general and administrative, facilities, research and development, acquiring new technologies, manufacturing product and product candidates, conducting clinical trials and preclinical experiments, seeking regulatory input, including approvals, as well as commercializing any products approved for sale, including its approved product, ELZONRIS, in blastic plasmacytoid dendritic cell neoplasm ("BPDCN"). The Company anticipates incurring additional losses until such time, if ever, that it can generate significant sales of its approved product and/or the potential approval of its currently approved product in additional territories and/or indications or its other product candidates currently in development. The Company expects its research and development expenses to increase moderately in connection with its ongoing and planned clinical trials and related manufacturing efforts, as well as for expenses related to the U.S. commercial launch of ELZONRIS and pursuit of potential regulatory approval and commercialization in additional ex-U.S. territories. The Company also anticipates that its selling, general and administrative expenses will be higher in future periods due to commercialization and ongoing optimization and build out of its commercial infrastructure and regulatory compliance systems to support the continued commercialization of ELZONRIS in the U.S. and potentially additional ex-U.S. territories.

As a result, the Company expects to continue to incur 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 delay or terminate commercialization, clinical trials or other development activities for its product and product candidates, for one or more indications or territories, or delay or terminate its establishment of sales and marketing capabilities, or other activities, that may be necessary to commercialize its products and product candidates.

Basis of Presentation

The accompanying unaudited financial statements have been prepared in accordance with the United States generally accepted accounting principles ("GAAP") and with the instructions to Form 10-Q and Article 10 of Regulation S-X. Accordingly, they do not include all information and footnotes required by GAAP for complete financial statements. In the opinion of management, the accompanying financial statements include all adjustments (including normal recurring adjustments) considered necessary for fair presentation of the Company's financial position, results of operations and cash flows for the periods presented. Operating results for the current interim period are not necessarily indicative of the results that may be expected for the fiscal year ending December 31, 2019, or any future periods. This Form 10-Q should be read in conjunction with the audited financial statements and accompanying notes in the Company's Annual Report on Form 10-K for the year ended December 31, 2018. The Company believes that its existing cash, cash equivalents, short-term investments, and long-term investments will be sufficient to cover its cash flow requirements for at least the next twelve months from the issuance date of these financial statements.

Use of Estimates

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

2. Summary of Significant Accounting Policies

The Company's significant accounting policies are described in Note 2 of the Notes to the Financial Statements included in the Company's Annual Report on Form 10-K for the year ended December 31, 2018. During the three months ended March 31, 2019, the changes to the significant accounting policies mainly relate to the commercialization of ELZONRIS, which includes product net revenue, accounts receivable, and inventory.

Revenue Recognition

Effective January 1, 2018, the Company 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 step assessment: (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 entity will collect the consideration it is entitled to in exchange for the goods or services it transfers to the customer. At contract inception and once the contract is determined to be within the scope of ASC 606, 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. 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. 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.

Product Revenue, Net: The Company has obtained marketing approval from the U.S. Food and Drug Administration, ("FDA") to sell ELZONRIS in the United States market. The Company sells ELZONRIS to its customer through its title distribution channel. The customer subsequently resells ELZONRIS to a limited number of specialty distributors who in turn distribute ELZONRIS to specialty hospitals.

The Company recognizes revenue on sales of ELZONRIS when its customer obtains control of the product, which occurs at a point in time, typically upon delivery. Product revenues are recorded at the product's wholesale acquisition costs, net of applicable reserves for variable consideration. Components of variable consideration include government contracts, product returns, commercial co-payment assistance program transactions, 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 a current liability. Overall, these reserves reflect our best estimates of the amount of consideration to which we are entitled with respect to 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. The Company's analyses contemplate the application of the constraint in accordance with ASC 606. For the three months ended March 31, 2019, the Company determined a material reversal of revenue would not likely occur in a future period for the estimates detailed below and, therefore, the transaction price was not reduced further. 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.

Government Contracts: The Company have 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 the establishment of a current liability, which is included in accrued expenses and other current liabilities. 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.

The Company estimates the rebates that it will provide to Government Payors for those 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.

Product Returns: Consistent with industry practice, the Company offers a limited right of return for product that has been purchased. To estimate sales with a right of return, the Company will 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 accrued expenses and other current liabilities.

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.

Accounts Receivable, Net

Accounts receivable, net primarily relates to amounts due from the Company's customer, net of applicable revenue reserves. The Company analyzes accounts that are past due for collectability and provides an allowance for receivables when collection becomes doubtful. Given the nature and limited history of collectability of the Company's accounts receivable, an allowance for doubtful accounts is not deemed necessary at March 31, 2019.

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. The majority of manufacturing costs for ELZONRIS units recognized as revenue during the three months ended March 31, 2019 were expensed to research and development prior to FDA approval on December 21, 2018.

The Company values its inventories at the lower of cost or estimated net realizable value. The Company determines the cost of its inventories, which includes amounts related to materials and manufacturing overhead, on a first-in, first-out basis. 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 product revenues. 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 product sales in the statement of operations and comprehensive loss.

Recently Issued Accounting Pronouncements

In May 2014, the Financial Accounting Standards Board ("FASB"), issued a comprehensive new revenue recognition Accounting Standards Update ("ASU"), *Revenue From Contracts With Customers (Topic 606) (ASU 2014-09).* ASU 2014-09 provides guidance to clarify the principles for recognizing revenue. This guidance includes the required steps to achieve the core principle that an entity should recognize income to depict the transfer of promised goods or services to customers in an amount that reflects the consideration to which the entity expects to be entitled in exchange for those goods or services. This guidance is effective for fiscal years and interim periods beginning after December 15, 2017. Early adoption is permitted for fiscal years and interim periods beginning after December 15, 2016. The Company adopted this guidance on January 1, 2018, using the full retrospective method. Any future contracts with customers will be accounted for under the new guidance effective January 1, 2018.

As noted below in Note 10 to the Company's Financial Statements, the Company has received grant income from the Leukemia and Lymphoma Society ("LLS") to fund the Company's development program related to the Company's preclinical and clinical product development activities. The Company has determined that LLS is not a customer as defined by Topic 606. The Company recognizes grant income when there is reasonable assurance of compliance with the conditions of the grant and reasonable assurance that the grant income will be received based on the Company's best estimates of work performed and qualifying costs incurred.

In January 2016, the FASB issued a new ASU, *Recognition and Measurement of Financial Assets and Financial Liabilities (ASU 2016-01).* ASU 2016-01 amends the guidance in U.S. GAAP on the classification and measurement of financial instruments. Although the ASU retains many current requirements, it significantly revises an entity's accounting related to (1) the classification and measurement of investments in equity securities and (2) the presentation of certain fair value changes for financial liabilities measured at fair value. The ASU also amends certain disclosure requirements associated with the fair value of financial instruments. The new standard is effective for fiscal years and interim periods within those fiscal years beginning after December 15, 2017, with early adoption permitted for certain changes. The Company adopted this guidance on January 1, 2018 and it had no impact on the financial position, results of operations or cash flows.

In February 2016, the FASB issued ASU 2016-02, Leases (Topic 842) *(ASU 2016-02)*, which supersedes ASC 840, Leases. The amendments in this update will increase the transparency and comparability among organizations by recognizing lease assets and lease liabilities on the balance sheet and disclosing key information about leasing arrangements. Under ASU 2016-02, a lessee will recognize in the statement of financial position a liability to make lease payments (the lease liability) and a right-of-use asset representing its right to use the underlying asset for the lease term. The recognition, measurement, and presentation of expenses and cash flows arising from a lease by a lessee have not significantly changed from current GAAP. ASU 2016-02 retains a distinction between finance leases and operating leases. The classification criteria for distinguishing between finance leases and operating leases will be substantially similar to the classification criteria for distinguishing between capital leases and operating leases under prior GAAP. Under the standard, disclosures are required to meet the objective of enabling users of financial statements to assess the amount, timing, and uncertainty of cash flows arising from leases.

The guidance permits a practical expedient with regards to initial adoption, allowing adopters the option to apply the new leases standard prospectively at the adoption date and recognize a cumulative-effect adjustment to the opening balance of retained earnings in the period of adoption. Under this expedient, comparative periods presented in the

financial statements in which the new lease standard is adopted will continue to be presented in accordance with prior GAAP.

The Company adopted this standard on January 1, 2019 using the prospective application method practical expedient. The adoption of this standard had an impact on our balance sheet, recognizing a right-of-use asset and lease liability of approximately 2 million. Refer to Note 8 for disclosure requirements related to the adoption of this standard.

In August 2016, the FASB issued ASU 2016-15, *Statement of Cash Flows (Topic 230), Classification of Certain Cash Receipts and Cash Payments (ASU 2016-15)*. ASU 2016-15 clarifies how entities should classify certain cash receipts and cash payments on the Statement of Cash Flows and amends certain disclosure requirements of ASC 230. The guidance will generally be applied retrospectively and is effective for public business entities for fiscal years beginning after December 15, 2017, and interim periods within those years. For all other entities, it is effective for fiscal years beginning after December 15, 2018, and interim periods within fiscal years beginning after December 15, 2019. Early adoption is permitted, including adoption in an interim period. If an entity early adopts the guidance in an interim period, any adjustments should be reflected as of the beginning of the fiscal year that includes that interim period. An entity that elects early adoption must adopt all of the guidance in the same period. The Company adopted this guidance on January 1, 2018 and it had no impact on the Statement of Cash Flows.

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). 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)*. 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. Adoption of ASU 2016-13 will not have a significant impact on the Company's financial statements.

In May 2017, the FASB issued *ASU No. 2017-09, Compensation — Stock Compensation (Topic 718), Scope of Modification Accounting (ASU No. 2017-09)*. ASU 2017-09 provides guidance on the types of changes to the terms or conditions of share-based payment awards to which an entity would be required to apply modification accounting. ASU 2017-09 is applied prospectively to awards modified on or after the effective date. The Company adopted ASU 2017-09 on January 1, 2018. The adoption of this standard did not have a material impact to the Company's Balance Sheet, Statement of Operations, or Statement of Cash Flows.

On June 20, 2018, the FASB issued *ASU No. 2018-07, Compensation — Stock Compensation (Topic 718): Improvements to Nonemployee Share-Based Payment Accounting (ASU No. 2018-07)*, which expands the scope of Topic 718 to include share-based payment transactions for acquiring goods and services from nonemployees. Entities should apply the requirements of Topic 718 to nonemployee awards except for specific guidance on inputs to an option pricing model and the period of time equity awards vest and the pattern of cost recognition over that period. ASU No. 2018-07 is effective for public business entities for fiscal years, and interim periods within those fiscal years, beginning after December 15, 2018. Early adoption is permitted and the Company adopted ASU No. 2018-07 on April 1, 2018. 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. In addition, the Company has elected to account for forfeitures of nonemployee awards as they occur.

In August 2018, the FASB issued *ASU 2018-13, Fair Value Measurement (Topic 820): Disclosure Framework-Changes to the Disclosure Requirements for Fair Value Measurement (ASU 2018-13)*, which adds disclosure requirements to Topic 820 for the range and weighted average of significant unobservable inputs used to develop Level 3 fair value measurements. This ASU is effective for interim and annual reporting periods beginning after December 15, 2019. The adoption of ASU 2018-13 is not expected to have an impact on the Company's financial statements.

In August 2018, the SEC adopted the final rule under SEC Release No. 33-10532, Disclosure Update and Simplification, amending certain disclosure requirements that were redundant, duplicative, overlapping, outdated or superseded. In addition, the amendments expanded the disclosure requirements on the analysis of stockholders' equity for interim financial statements. Under the amendments, an analysis of changes in each caption of stockholders' equity presented in the balance sheets must be provided in a note or separate statement. The analysis should present a reconciliation of the beginning balance to the ending balance of each period for which a statement of comprehensive income is required to be filed. As such, the Company adopted these SEC amendments on November 5, 2018 and will present the analysis of changes in stockholders' equity in its interim financial statements in its March 31, 2019 Form 10-Q. The Company does not anticipate that the adoption of these SEC amendments will have a material effect on the Company's financial statements.

In November 2018, ASU 2018-18 was issued to provide clarity on when transactions between entities in a collaborative arrangement should be accounted for under the new revenue standard, ASC 606. In determining whether transactions in collaborative arrangements should be accounted under the revenue standard, the update specifies that entities shall apply unit of account guidance to identify distinct goods or services and whether such goods and services are separately identifiable from other promises in the contract. ASU 2018-18 also precludes entities from presenting transactions with a collaborative partner which are not in scope of the new revenue standard together with revenue from contracts with customers. The accounting update is effective January 1, 2020 and early adoption is permitted. The adoption of ASU 2018-18 is not expected to have an impact on the Company's financial statements.

In December 2018, the FASB issued *ASU 2018-20, Leases (Topic 842):* Narrow-Scope Improvements for Lessors *(ASU 2018-20)*, which addressed implementation questions arising from stakeholders in regard to ASU 2016-02, Leases. Specifically addressed in this update were issues related to 1) sales taxes and other similar taxes collected from lessees, 2) certain lessor costs, and 3) recognition of variable payments for contracts with lease and nonlease components. The amendments in this ASU are effective in the same time-frame as ASU 2016-02 as discussed above. The Company incorporated this ASU into its assessment and adoption of ASU 2016-02.

3. Liquidity and Capital Resources

As of March 31, 2019, the Company has approximately $124.4 million in cash, cash equivalents, and short and long-term investment securities. The Company primarily invests in highly liquid cash equivalents, short-term investments and long-term investments in U.S. Treasury and Agency securities and related money market funds and FDIC-insured bank certificates of deposit, with the balance in commercial bank operating accounts.

4. Prepaid Expenses and Other Current Assets

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

	March 31, 2019	December 31, 2018
Prepaid third party vendor costs	$ 1,376,116	$ 1,851,553
Deposits	189,000	189,000
Prepaid insurance	657,669	69,021
Other receivable	898,141	843,422
Total	$ 3,120,926	$ 2,952,996

5. Property and Equipment, Net

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

	March 31, 2019	December 31, 2018
Office furniture and fixtures	$ 519,675	$ 519,675
Manufacturing equipment	181,753	107,258
Leasehold improvements	82,694	82,694
Capital lease equipment	29,529	29,529
Computer equipment	18,612	18,612
Property and equipment	832,263	757,768
Less accumulated depreciation	(558,864)	(535,355)
Property and equipment, net	$ 273,399	$ 222,413

Depreciation expense was $23,510 and $11,786 for the three-month periods ended March 31, 2019 and 2018, respectively.

6. Fair Value Measurements

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

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

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

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

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 March 31, 2019 and December 31, 2018:

	March 31, 2019			
	Quoted Prices in Active Markets for Identical Assets (Level 1)	Significant Other Observable Inputs (Level 2)	Significant Unobservable Inputs (Level 3)	Balance at March 31, 2019
Assets:				
Fixed-income treasury portfolio	$ 71,811,331	$ —	$ —	$ 71,811,331
Certificate of Deposits	—	28,526,652	—	28,526,652
Cash and cash equivalents	24,014,510	—	—	24,014,510
Total assets at fair value	$ 95,825,841	$ 28,526,652	$ —	$ 124,352,493

	December 31, 2018			
	Quoted Prices in Active Markets for Identical Assets (Level 1)	Significant Other Observable Inputs (Level 2)	Significant Unobservable Inputs (Level 3)	Balance at December 31, 2018
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	$ 40,081,665	$ 20,024,191	$ —	$ 60,105,856

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

	March 31, 2019			
	Amortized Cost	Gross Unrealized Gains*	Gross Unrealized Losses*	Estimated Fair Value
Cash:				
Cash from operating accounts	$ 3,151,188	$ —	$ —	$ 3,151,188
Cash equivalents:				
Money market funds	20,863,322	—	—	20,863,322
Total cash and cash equivalents	$ 24,014,510	$ —	$ —	$ 24,014,510
Short-term investments:				
Fixed-income treasury portfolio:				
Federal home loan bank	1,003,875	—	(2,220)	1,001,655
Freddie Mac	999,981	—	(4,485)	995,496
U.S. Treasury Securities	69,793,815	24,250	(3,886)	69,814,179
Certificate of Deposits	28,534,366	805	(8,518)	28,526,653
Total short-term investments	100,332,037	25,055	(19,109)	100,337,983
Total	$ 124,346,547	$ 25,055	$ (19,109)	$ 124,352,493

	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 March 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, and the remaining contractual maturities of available-for-sale investments classified as long-term were less than two years.

There were no available-for-sale securities in a continuous unrealized loss position for greater than twelve months at March 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 March 31, 2019.

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 expenses. Cash and cash equivalents, short-term investments and long-term investments are carried at fair value (see above). Financial instruments including other current assets, accounts payable and accrued expenses are carried at cost, which approximate fair value given their short-term nature.

7. Inventory

Inventory consists of the following:

	March 31, 2019
Raw materials	$ —
Work-in-process	650,722
Finished goods	197,771
Total Inventory	$ 848,493

Inventory is related to our approved product, ELZONRIS. There were no write downs for excess and obsolete inventory during the three months ended March 31, 2019.

8. Leases

The Company has leases for office facilities as well as for certain equipment. The operating lease portfolio is related to two office spaces and the financing lease relate to office equipment that was acquired in prior year. As of March 31, 2019, the Company has not entered into any new lease arrangements classified as a finance lease. Operating leases are included in operating lease right-of-use assets, current operating lease liabilities and operating lease liabilities on the Company's balance sheet. The Company has elected the package of practical expedients which applies to leases that commenced before the adoption date. By electing the package of practical expedients, the Company did not need to reassess the following; whether any existing contracts are or contain leases, the lease classification for any existing leases and initial direct costs for any existing leases.

Right-of-use asset and operating lease liabilities are recognized based on the present value of future minimum lease payments over the lease term at commencement date. When the implicit rate of the lease is not provided or cannot be determined, the Company used the incremental borrowing rate based on the information available at the commencement date to determine the present value of future payments. Lease terms may include options to extend or terminate the lease when it is reasonably certain that we will exercise those options. Lease expense for minimum lease payments is recognized on a straight-line basis over the lease term. Operating lease right-of-use asset is presented within right-of-use asset, net. The current portion of operating lease liabilities are presented within right-of-use liability - current portion and the non-current portion of operating lease liabilities are presented within right-of-use liability on the Balance Sheet. Finance lease assets are included in Property, plant and equipment - net, and the finance lease obligations are included in Other current liabilities debt, and in Other liabilities on the Balance Sheet. Components of lease expense and other information as follows:

	March 31, 2019
Lease Expense	
Operating Lease Cost	$ 264,693
Financing Lease Cost:	
Amortization of right-of-use assets	2,460
Interest on lease liability	292
Total financing lease cost	2,752
Total Lease Cost	$ 267,445

Balance sheet information related to leases was as follows:	March 31, 2019
Operating Leases	
Operating right-of-use asset, net	$ 1,738,680
Operating right-of-use liability - current portion	1,023,678
Operating right-of-use liability	818,303
Total operating lease liabilities	$ 1,841,981
Financing Leases	
Property, plant and equipment	$ 29,529
Accumulated depreciation	(8,202)
Property, plant and equipment - net	$ 21,327
Other current liabilities	9,754
Other liabilities	12,011
Total Financing lease liabilities	$ 21,765
Weighted Average Remaining Lease Term — Operating Leases	1.75 years
Weighted Average Remaining Lease Term — Financing Leases	2.16 years
Weighted Average Discount Rate — Operating Leases	7.32%
Weighted Average Discount Rate — Financing Leases	5%

Future minimum lease payments under non-cancellable leases as of March 31, 2019 were as follows:

	Operating Leases	Financing Leases
2019	$ 838,350	$ 7,965
2020	1,117,800	10,620
2021	—	4,425
Total future minimum lease payments	1,956,150	23,010
Less imputed interest	(114,169)	(1,245)
Total	$ 1,841,981	$ 21,765

Supplemental cash flow information related to leases was as follows:

Cash paid for amounts included in the measurement of lease liabilities:	March 31, 2019
Operating cash flows from operating leases	$ 279,450
Operating cash flows from finance leases	292
Financing cash flows from finance leases	2,363

9. Accounts Payable and Accrued Expenses

Accounts payable and accrued expenses consist of the following at March 31, 2019 and December 31, 2018:

	March 31, 2019	December 31, 2018
Accrued research and development costs	$ 13,350,177	$ 8,790,920
Accrued compensation	2,643,983	5,515,002
Accrued legal	1,342,389	687,042
Accrued commercial costs	5,599,300	4,614,507
Accrued general and administrative costs	1,829,046	1,545,591
Accrued sales deduction and allowance	648,126	—
Total accounts payable and accrued expenses	$ 25,413,021	$ 21,153,062

10. Common Stock

On January 18, 2019, the Company completed a 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.

As of March 31, 2019 and December 31, 2018, the Company was authorized to issue 53,750,000 shares of common stock.

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

11. Accumulated Other Comprehensive Loss

The changes in accumulated balances for each component of other comprehensive loss are as follows:

	Three Months Ended March 31,	
	2019	2018
Balance at beginning of period	$ (56,559)	$ (145,958)
Other comprehensive income (loss) before reclassification	25,074	(8,316)
Amounts reclassified from accumulated other comprehensive loss*	4,324	3,897
Total other comprehensive income (loss)	29,398	(4,419)
Balance at end of period	$ (27,161)	$ (150,377)

*Amounts reclassified affect other income in the Statements of Operations.

12. Product revenue reserves and allowances

As of March 31, 2019, the Company's sole source of product revenue has been from sales of ELZONRIS in the United States. The following table summarizes activity in each of the product revenue allowance and reserve categories for the three months ended March 31, 2019:

	March 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	172,688	202,769	52,181	142,672	570,310
Adjustments related to prior period sale	—	—	—	—	—
Credits and payments made	—	—	—	—	—
Ending balance at March 31, 2019	$ 172,688	$ 202,769	$ 52,181	$ 142,672	$ 570,310

Government rebates, product returns, commercial co-payment assistance programs, and distribution fees are recorded as a component of accrued expenses on the balance sheet.

13. Net Loss Per Common Share

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

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

	Three Months Ended March 31,	
	2019	2018
Basic and diluted net loss per common share calculation:		
Net loss	$ (27,407,266)	$ (18,416,753)
Basic and diluted weighted-average common shares	37,550,931	26,845,983
Basic and diluted net loss per share	$ (0.73)	$ (0.69)

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 three-month periods ended March 31, 2019 and 2018, the Company reported a loss from operations and therefore, all potentially dilutive stock options and restricted stock 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 and restricted stock that could potentially dilute earnings per share in the future, but which were not included in the calculation of diluted net loss per share because their affect would have been anti-dilutive were as follows:

	Three Months Ended March 31,	
	2019	2018
Restricted stock	$ 3,582,612	$ 1,840,125
Options outstanding	3,535,018	3,128,627
Total	$ 7,117,630	$ 4,968,752

14. Grant Income

In October 2013, the Company entered into a contract with the Leukemia and Lymphoma Society, or LLS. 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. 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. LLS agreed to provide funding to the Company not to exceed $3.5 million to fund the Company's development program related to the Company's preclinical and clinical product development activities. Through March 31, 2019, the Company has received $3.5 million based on milestones achieved. The agreement terminates when there are no longer any payment obligations for either LLS or Stemline.

15. Income Taxes

The Company recorded $3,694 income tax benefit related to intraperiod tax allocations for the three-month period ended March 31, 2019 and no income tax provisions or benefits were recorded for the three-month period ended March 31, 2018, due to the fact that the Company cannot benefit from its net operating losses or other deferred tax assets. The Company does not currently have the ability to carry back losses to previous years to recover taxes paid and future utilization of these losses is uncertain.

The Company files income tax returns in the United States and in the State of New York. The Company's 2015 tax year is currently being audited by the Internal Revenue Service and there are no ongoing audits in state taxing jurisdictions.

Deferred income taxes reflect the net tax effects of temporary differences between the carrying amounts of assets and liabilities for financial reporting purposes and their basis for income tax purposes and the tax effects of net operating loss and tax credit carryforwards.

Valuation allowances reduce deferred tax assets to the amounts that are more likely than not to be realized. As of March 31, 2019, the Company has recorded additional deferred tax assets which are fully offset by a valuation allowance. Realization of the deferred tax assets is dependent on generating sufficient taxable income in the future. At present, the likelihood of the Company being able to fully utilize its deferred income tax benefits against future income is uncertain.

16. Stock-Based Compensation

The Company's 2016 Stock Equity Incentive Plan (the "2016 Plan") was adopted by the board of directors and approved by the stockholders in May 2016. The 2016 Plan authorizes the Company to grant up to 1,812,932 shares of common stock to eligible employees, directors, and non-employee consultants and advisors to the Company. Under the provisions of the 2016 Plan, no option will have a term in excess of 10 years. In 2017, the Company's stockholders approved an increase of 1,200,000 shares authorized under the 2016 Plan and another increase of 2,900,000 shares authorized in 2018.

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. 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 March 31, 2019, there were 350,667 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 $7.2 million and $2.2 million for the three-month periods ended March 31, 2019 and 2018, respectively. As a result of the valuation allowance against the Company's deferred tax assets, there was no net adjustment to retained earnings for the change in accounting for unrecognized windfall tax benefits.

The following table summarizes stock-based compensation related to the above plans by expense category for the three-month periods ended March 31, 2019 and 2018, respectively:

	Three Months Ended March 31,	
	2019	2018
Research and development	$ 3,106,059	$ 970,624
Selling, general and administrative	4,100,033	1,235,803
Total	$ 7,206,092	$ 2,206,427

The following table summarizes the stock-based compensation capitalized to inventory:

	Three Months Ended March 31,	
	2019	2018
Stock-based compensation expense capitalized to inventory	$ 55,557	$ —

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 three-month periods ended March 31, 2019 and 2018, respectively are as follows:

	Three Months Ended March 31,	
	2019	2018
Weighted-average volatility	66.47%	77.96%
Weighted-average risk-free interest rate	2.59%	2.63%
Weighted-average expected term in years	6.26	6.26
Dividend yield	—	—

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 three-month period ended March 31, 2019, the Company issued 2,500 shares of the Company's common stock upon the exercise of outstanding stock options and received proceeds of approximately $15,825. As of March 31, 2019, there was approximately $6.7 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 2.45 years.

The following table summarizes the activity related to the Company's stock options for the three months ended March 31, 2019:

	Options	Weighted-Average Exercise Price	Weighted-Average Remaining Contractual Life	Aggregate Intrinsic Value
Outstanding at December 31, 2018	3,514,018	$ 9.59	6.08	$ 10,087,201
Options granted	45,500	10.90		
Options exercised	(2,500)	6.33		
Options forfeited	(22,000)	15.65		
Outstanding at March 31, 2019	3,535,018	$ 9.57	5.87	$ 17,220,252
Options exercisable at March 31, 2019	2,550,614	$ 8.89	4.84	$ 14,283,344

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 quarter ended March 31, 2019 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 March 31, 2019. The intrinsic value of the Company's stock options changes based on the closing price of the Company's common stock.

Restricted Stock

The Company grants restricted stock to its employees, directors, and non-employee consultants. Restricted stock is recorded as deferred compensation and charged against income on a straight-line basis over the vesting period, which ranges from immediate to four years in duration. If vesting of the award is based on a performance or market condition, awards vest based on the derived service period which is the estimated period of time that would be required to satisfy the performance or market condition. 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 following table summarizes the activity related to the Company's restricted stock for the three months ended March 31, 2019:

	Number of Shares	Weighted-Average Grant Date Fair Value Per Share
Outstanding at December 31, 2018	2,794,455	$ 12.79
Shares granted	1,366,471	11.16
Shares vested	(565,189)	10.59
Shares forfeited	(13,125)	15.65
Outstanding at March 31, 2019	3,582,612	$ 12.50

For the three-month period ended March 31, 2019, the Company granted 1,366,471 shares of restricted stock at a weighted-average grant date fair value of $11.16 per share amounting to approximately $15.3 million in total aggregate fair value. As of March 31, 2019, 3,582,612 shares remained unvested and there was approximately $36.6 million of unrecognized compensation cost related to restricted stock which is expected to be recognized over a remaining weighted-average period of approximately 2.05 years. The total fair value of restricted stock vested during the three-month periods ended March 31, 2019 and 2018 was approximately $6.0 million and $3.5 million, respectively.

Performance Share Awards

On August 2018, the FDA accepted the Company's Biologics License Application, or 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, or PSAs, were met. The Company recognized approximately $0.2 million and $21,739 of stock compensation expense related to the performance share awards, or PSAs, for three-month period ended March 31, 2019 and March 31, 2018, respectively.

In addition, ELZONRIS received FDA approval on December 21, 2018 for the treatment of patients with BPDCN. As a result of the approval, the underlying performance condition associated with the PSAs were met and the Company recognized approximately $2.5 million and $0 of stock compensation expense related to the PSAs for the three-month period ended March 31, 2019 and March 31, 2018, respectively.

For awards with performance conditions, such as obtaining regulatory 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-Employee Consultants

The Company grants stock options, restricted stock, and unrestricted stock to non-employee consultants. The Company measures the fair value of stock-based awards issued to non-employees and records expense over the requisite service period. Total compensation cost charged against operations related to stock-based awards granted to non-employee consultants was approximately $0.2 million and $0.2 million for the three-month periods ended March 31, 2019 and 2018, 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 ("Rights") 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%. The Company recorded approximately $24,853 and $18,876 of compensation expense for the three months ended March 31, 2019 and 2018, respectively, related to participation in the 2015 ESPP.

17. Commitments and Contingencies

The Company has entered into research and development agreements with third-parties for the development of oncology product candidates and technologies. According to these agreements, the Company typically funds the development of such assets and potentially makes development-based milestone payments, and royalty and sales-based milestone

payments based on net sales should the product candidates be approved for marketing. The timing and the amounts of milestone and royalty payments in the future are not certain.

The Company has also entered into license agreements, including ones with licenses to certain intellectual property rights, in the field of oncology and other indications. The Company is generally required to make upfront payments as well as other payments upon successful completion of preclinical, clinical, regulatory or sales milestones, should such milestones occur. In addition, these agreements generally would require the Company to pay royalties on sales of the products arising from these agreements, should a product candidate under the license agreement receive regulatory approval. These agreements generally permit the Company to terminate the agreement with no significant continuing obligation.

Under the Company's research and development and/or license agreements, if the Company were to achieve certain milestones, primarily late stage clinical trial events, marketing approval, and sales, the Company could be required to pay up to a total of $375.6 million in future periods. As of March 31, 2019, the Company has paid or accrued $7.3 million in payments pursuant to such agreements. If a product candidate under such agreements were to receive marketing approval, royalty payments, largely single digit, are payable on commercial sales of certain products.

The Company has committed to make potential future milestone and royalty payments to third-parties as part of its research and development and 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 guaranteed 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 $32.4 million, with services to be rendered on some of these agreements through 2019. From inception through March 31, 2019, the Company has received and paid for services relating to these agreements in the amount of $27.0 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 2019.

The Company has agreements in place with contract research organizations, or CROs, in connection with its clinical programs. The Company's total expenditures in the future would be approximately $3.1 million assuming the successful advancement of its programs.

Agreement with the Leukemia and Lymphoma Society

In October 2013, the Company entered into a contract with the Leukemia and Lymphoma Society, or LLS. LLS is a national voluntary health organization 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. LLS agreed to provide funding to the Company not to exceed $3.5 million to fund the Company's development program related to the Company's preclinical and clinical product development activities. Through March 31, 2019, the Company has received $3.5 million based on milestones achieved.

For the three-month period ended March 31, 2019, the Company recorded expense of approximately $4.4 million relating to the achievement of a post-approval milestone.

Contingencies

On March 15, 2018, the United States District Court for the Southern District of New York 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 United States District Court for the Southern District of New York, after the Plaintiffs voluntarily withdrew their appeal pursuant to Local Rule 42.1.

Item 2. 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 of our financial condition and results of operations together with our financial statements and related notes included in our audited financial statements and notes thereto for the year ended December 31, 2018, and Management's Discussion and Analysis of Financial Condition and Results of Operations included in our Annual Report on Form 10-K for the year ended December 31, 2018, to which the reader is directed for additional information.

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; SL-401), 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.

BPDCN 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 acute myeloid leukemia, or AML, non-Hodgkin's lymphoma, or NHL, acute lymphocytic leukemia, or ALL, myelodysplastic syndrome, or MDS, chronic myelomonocytic leukemia, or CMML, other malignancies with skin manifestations as well as certain 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 highly 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. CD123 has been associated with poor outcomes in AML.

ELZONRIS was granted by the FDA, Breakthrough Therapy Designation, or BTD, for the treatment of BPDCN in August 2016, and Orphan Drug Designation, or ODD, for AML in February 2011 and ODD for BPDCN in June 2013. ELZONRIS was granted, by the European Medicines Agency, or EMA, ODD for AML in September 2015 and ODD for BPDCN in November 2015.

In addition to ELZONRIS, our pipeline of product candidates also includes: SL-801, SL-701, SL-901 and SL-1001.

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. ELZONRIS became commercially available in the United States by prescription in January 2019, when we commenced sales and shipments to our customer who, in turn, ships product to oncology hospital sites via sales to specialty distributors.

In January 2019, Stemline submitted a Marketing Authorization Application, or MAA, to the EMA, seeking potential approval of ELZONRIS for the treatment of adult patients with BPDCN. The MAA was validated, and is currently under review, by the EMA. The MAA was granted accelerated assessment by the EMA in November 2018.

In the U.S., ELZONRIS is approved for the treatment of BPDCN in adult and pediatric patients two years and older, in both treatment-naïve and previously-treated populations. The ELZONRIS label contains a boxed warning for capillary leak syndrome, or CLS, which may be life-threatening or fatal if not properly managed, and can occur in patients receiving ELZONRIS. Physicians are advised to monitor for signs and symptoms of CLS and take actions as recommended in the full prescribing information.

Stemline's commercial group is focused on effectively launching ELZONRIS in the U.S., as well as preparing for a potential European launch. The commercial function is comprised of a wide range of functions including commercial operations, sales, marketing, 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 believe the aforementioned functions represent the necessary infrastructure to support a successful launch of ELZONRIS in BPDCN.

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 launched our BPDCN disease awareness campaign at the American Society of Hematology, or ASH, annual meeting. One of the campaign's primary goals is to try to ensure that multidisciplinary healthcare professionals, including hematologist-oncologists, dermatologists, pathologists, and allied healthcare professionals are appropriately testing for CD123 to bring the diagnosis of BPDCN to the forefront and to limit misdiagnoses and underdiagnoses. The campaign highlights the importance of CD123 as a key diagnostic marker for correct patient diagnosis.

Access to ELZONRIS remains a top priority within our managed care group with key success criteria identified as removing hurdles to product access and reimbursement. We have set up a formal 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. Marketing, sales and medical affairs staffing efforts are scaled up to the "right size." European staffing and infrastructure needs have been assessed and we are enacting a similar "right size" approach to meet the needs of a potential European Union launch. We are also seeking to broaden the commercial potential of ELZONRIS, globally, through ongoing clinical trials in additional indications including CMML, myelofibrosis, or MF, AML, as well as additional planned trials in other indications. Additional planned trials include maintenance therapy after stem cell transplant in patients with BPDCN and AML subsets enriched for CD123 expression and/or with BPDCN-like features.

Clinical pipeline product candidates

SL-801

SL-801 is a structurally novel, oral, small molecule, reversible inhibitor of Exportin-1, or XPO1, a nuclear transport protein implicated in a variety of malignancies. SL-801 has demonstrated preclinical in vitro and in vivo antitumor activity against a wide array of solid and hematologic cancers. SL-801's potential ability to reversibly bind XPO1 may offer the possibility to mitigate side effects and help optimize the therapeutic index. We are currently enrolling patients with advanced solid tumors in a Phase 1 dose escalation trial of single agent SL-801. The dosing regimen for SL-801 was revised in light of the occurrence of non-dose limiting gastrointestinal effects. We have resumed dosing at 70 mg/day with a new schedule, days 1-2 every 7 days of a 28-day cycle, and we expect to provide further data updates later this year.

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-13Rα2, EphA2, and survivin; two of these synthetic peptides (IL-13Rα2 and survivin) are mutant and believed to enhance immune activity. We completed a Phase 2 trial of SL-701 in adult patients with second-line glioblastoma, or GBM. 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-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 partial response, or 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 also believe that SL-901 may have utility in certain non-oncologic orphan diseases and preclinical work in this area is ongoing. We are currently evaluating plans to enable a new regulatory filing, including an Investigational New Drug (IND) application and IND-enabling work, to continue clinical dose escalation and exploration.

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 candidate 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 planned, and we expect to begin clinical studies of SL-1001 in 2020.

SL-501

SL-501 is a novel CD123-targeted therapy in preclinical development that has shown potency, in vitro and in vivo, against several hematologic tumor types, including AML, CMML, Hodgkin's lymphoma, and 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 MAA filings, preparation for the commercialization and launch of 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. For the three months ended March 31, 2019, we have generated $5.0 million in net product revenue for ELZONRIS.

From inception through March 31, 2019, we have received net proceeds of $364.4 million from the sale of common stock through the initial public offering and five 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.

We have never been profitable and our net loss from operations for the three months ended March 31, 2019 and 2018 was $27.4 million and $18.4 million, respectively. 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 commercialize our approved product in the U.S., seek approval and build out a commercial infrastructure in Europe, advance our approved product through clinical trials to seek regulatory approval in additional indications, advance our other product candidates through clinical trials to seek regulatory approval and, when and if approved, commercialize such product(s) and product candidates, as well as conducting manufacturing campaigns and various preclinical activities. Accordingly, we may need additional financing to support our continuing operations. We may 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 if 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 income 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 Footnote 17: Commitments and Contingencies for more information on legal proceedings.

Financial Operations Overview

Product Revenues

Total revenue consists of net sales of ELZONRIS, which was approved by the FDA on December 21, 2018 and launched in the U.S. in January 2019. Net sales represents the gross sales of ELZONRIS in the U.S. less provisions for product sales allowances and accruals. These provisions include trade allowances, rebates, chargebacks, discounts, and product returns. Although we expect net sales to increase over time, the provisions for product sales and allowances may fluctuate based on the mix of sales to different customer segments, rates of returns, and/or changes in our accrual estimates.

Research and Development Expenses

The following table shows our research and development expenses for the three-month periods ended March 31, 2019 and 2018, respectively:

	Three Months Ended March 31,	
	2019	2018
ELZONRIS	$ 9,116,428	$ 8,006,591
Other product candidates	1,722,290	1,055,074
Personnel expenses	5,550,846	3,136,482
Other expenses	564,258	509,911
Total	$ 16,953,822	$ 12,708,058

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 or New Drug Application, or NDA, and equivalent foreign regulatory submission requirements;
- nonclinical costs;

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

We anticipate that our 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 candidates compared to other therapies;
- the costs, timing and outcome of regulatory interactions, submissions, and potential approvals;
- our ability to market and commercialize, either on our own or with strategic partners, and achieve market acceptance and reimbursement for any of our product candidates that we are developing or may develop in the future;
- our ability to manufacture, at a reasonable expense, adequate supplies or our product candidates for use in planned and future clinical trials and/or commercial distribution in the event of a successful regulatory approval; and
- the costs of preparing, filing, prosecuting, defending and enforcing patents and other intellectual property.

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

development. A similar result could occur if we experience significant delays in the progress of, including enrollment in, any clinical trials.

Selling, General and Administrative Expenses

Selling, general and administrative expenses consist primarily of salaries and related costs for personnel, including stock-based compensation expense. The primary functions included in our selling, general and administrative expenses are commercial, 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 the build out of a commercial infrastructure and regulatory compliance systems to support a potential commercial launch of ELZONRIS in the European Union, if marketing approval is obtained from the EMA.

Interest Income

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

Critical Accounting Policies and Estimates

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

For a discussion of our critical accounting estimates, see the section entitled "Management's Discussion and Analysis of Financial Condition and Results of Operations" in our Annual Report on Form 10-K for the year ended December 31, 2018. During the three months ended March 31, 2019, the changes to the significant accounting policies mainly relate to the commercialization of ELZONRIS, which includes product net revenue, accounts receivable, and inventory.

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 customer. The trade payment terms with our 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.

Product Revenue, Net: We have obtained marketing approval from FDA to sell ELZONRIS in the United States market. We sell ELZONRIS 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 a current liability. Overall, these reserves reflect our best estimates of the amount of consideration to which we are entitled with respect to 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. For the three months ended March 31, 2019, we determined a material reversal of revenue would not likely occur in a future period for the estimates detailed below and, therefore, the transaction price was not reduced further. 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 would 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 the establishment of a current liability, which is included in accrued expenses and other current liabilities. 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 its customer 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 product that has been purchased. To estimate sales with a right of return, we will 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 accrued expenses and other current liabilities.

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 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 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.

Accounts Receivable, Net

Accounts receivable, net primarily relates to amounts due from our customer, net of applicable revenue reserves. We analyze accounts that are past due for collectability and provides an allowance for receivables when collection becomes doubtful. Given the nature and limited history of collectability of our accounts receivable, an allowance for doubtful accounts is not deemed necessary at March 31, 2019.

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. The majority of manufacturing costs for ELZONRIS units recognized as revenue during the three months ended March 31, 2019 were expensed to research and development prior to FDA approval on December 21, 2018.

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 we 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 product revenues. 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 product sales in the statement of operations and comprehensive loss.

Recent Accounting Pronouncements

In May 2014, the FASB issued a comprehensive new revenue recognition ASU, *Revenue From Contracts With Customers (Topic 606) (ASU 2014-09).* ASU 2014-09 provides guidance to clarify the principles for recognizing revenue. This guidance includes the required steps to achieve the core principle that an entity should recognize income to depict the transfer of promised goods or services to customers in an amount that reflects the consideration to which the entity expects to be entitled in exchange for those goods or services. This guidance is effective for fiscal years and interim periods beginning after December 15, 2017. Early adoption is permitted for fiscal years and interim periods beginning after December 15, 2016. We adopted this guidance on January 1, 2018 using the full retrospective method. Any future contracts with customers will be accounted for under the new guidance effective January 1, 2018.

As noted above in Note 10 to our Financial Statements, the Company has received grant income from the Leukemia and Lymphoma Society ("LLS"), to fund the Company's development program related to the Company's preclinical and clinical product development activities. The Company has determined that LLS is not a customer as defined by Topic 606. We recognize grant income when there is reasonable assurance of compliance with the conditions of the grant and reasonable assurance that the grant income will be received based on the Company's best estimates of work performed and qualifying costs incurred.

In January 2016, the FASB issued a new ASU, *Recognition and Measurement of Financial Assets and Financial Liabilities (ASU 2016-01).* ASU 2016-01 amends the guidance in U.S. GAAP on the classification and measurement of financial instruments. Although the ASU retains many current requirements, it significantly revises an entity's accounting related to (1) the classification and measurement of investments in equity securities and (2) the presentation of certain fair value changes for financial liabilities measured at fair value. The ASU also amends certain disclosure requirements associated with the fair value of financial instruments. The new standard is effective for fiscal years and interim periods within those fiscal years beginning after December 15, 2017, with early adoption permitted for certain changes. We adopted this guidance on January 1, 2018 and it had no impact on the Balance Sheet, Statement of Operations, or Statement of Cash Flows.

In February 2016, the FASB issued ASU 2016-02, Leases (Topic 842) *(ASU 2016-02)*, which supersedes ASC 840, Leases. The amendments in this update will increase the transparency and comparability among organizations by recognizing lease assets and lease liabilities on the balance sheet and disclosing key information about leasing arrangements. Under ASU 2016-02, a lessee will recognize in the statement of financial position a liability to make lease payments (the lease liability) and a right-of-use asset representing its right to use the underlying asset for the lease term. The recognition, measurement, and presentation of expenses and cash flows arising from a lease by a lessee have not significantly changed from current GAAP. ASU 2016-02 retains a distinction between finance leases and operating leases. The classification criteria for distinguishing between finance leases and operating leases will be substantially similar to the classification criteria for distinguishing between capital leases and operating leases under prior GAAP. Under the standard, disclosures are required to meet the objective of enabling users of financial statements to assess the amount, timing, and uncertainty of cash flows arising from leases.

The guidance permits a practical expedient with regards to initial adoption, allowing adopters the option to apply the new leases standard prospectively at the adoption date and recognize a cumulative-effect adjustment to the opening balance of retained earnings in the period of adoption. Under this expedient, comparative periods presented in the financial statements in which the new lease standard is adopted will continue to be presented in accordance with prior GAAP.

We adopted this standard on January 1, 2019 using the prospective application method practical expedient. The adoption of this standard had an impact on our Balance Sheet, recognizing a right-of-use asset and lease liability of approximately 2 million. Refer to Note 8 of the financial statements for disclosure requirements related to the adoption of this standard.

In August 2016, the FASB issued ASU 2016-15, *Statement of Cash Flows (Topic 230), Classification of Certain Cash Receipts and Cash Payments (ASU 2016-15)*. ASU 2016-15 clarifies how entities should classify certain cash receipts and cash payments on the Statement of Cash Flows and amends certain disclosure requirements of ASC 230. The guidance will generally be applied retrospectively and is effective for public business entities for fiscal years beginning after December 15, 2017, and interim periods within those years. For all other entities, it is effective for fiscal years beginning after December 15, 2018, and interim periods within fiscal years beginning after December 15, 2019. Early adoption is permitted, including adoption in an interim period. If an entity early adopts the guidance in an interim period, any adjustments should be reflected as of the beginning of the fiscal year that includes that interim period. An entity that elects early adoption must adopt all of the guidance in the same period. We adopted this guidance on January 1, 2018 and it had no impact on the Statement of Cash Flows.

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). 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 No. 2018-19)*. 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. Adoption of ASU 2016-13 will not have a significant impact on our financial statements.

In May 2017, the FASB issued ASU No. 2017-09, Compensation — Stock Compensation (Topic 718), Scope of Modification Accounting *(ASU No. 2017-09)*. ASU 2017-09 provides guidance on the types of changes to the terms or conditions of share-based payment awards to which an entity would be required to apply modification accounting. ASU 2017-09 is applied prospectively to awards modified on or after the effective date. We adopted ASU 2017-09 on January 1, 2018. The adoption of this standard did not have a material impact to our Balance Sheet, Statement of Operations, or Statement of Cash Flows.

On June 20, 2018, the FASB issued ASU No. 2018-07, Compensation — Stock Compensation (Topic 718): Improvements to Nonemployee Share-Based Payment Accounting *(ASU No. 2018-07)*, which expands the scope of Topic 718 to include share-based payment transactions for acquiring goods and services from nonemployees. Entities should apply the requirements of Topic 718 to nonemployee awards except for specific guidance on inputs to an option pricing model and the period of time equity awards vest and the pattern of cost recognition over that period. ASU No. 2018-07 is effective for public business entities for fiscal years, and interim periods within those fiscal years, beginning after December 15, 2018. Early adoption is permitted and we adopted ASU No. 2018-07 on April 1, 2018. We 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. In addition, we have elected to account for forfeitures of nonemployee awards as they occur.

In August 2018, the FASB issued ASU 2018-13, Fair Value Measurement (Topic 820): Disclosure Framework-Changes to the Disclosure Requirements for Fair Value Measurement *(ASU 2018-13)*, which adds disclosure requirements to Topic 820 for the range and weighted average of significant unobservable inputs used to develop Level 3 fair value measurements. This ASU is effective for interim and annual reporting periods beginning after December 15, 2019. The adoption of ASU 2018-13 is not expected to have an impact on our financial statements.

In August 2018, the SEC adopted the final rule under SEC Release No. 33-10532, Disclosure Update and Simplification, amending certain disclosure requirements that were redundant, duplicative, overlapping, outdated or superseded. In addition, the amendments expanded the disclosure requirements on the analysis of stockholders' equity for interim financial statements. Under the amendments, an analysis of changes in each caption of stockholders' equity presented in the balance sheets must be provided in a note or separate statement. The analysis should present a reconciliation of the beginning balance to the ending balance of each period for which a statement of comprehensive income is required to be filed. As such, we adopted these SEC amendments on November 5, 2018 and will present the analysis of changes in stockholders' equity in its interim financial statements in its March 31, 2019 Form 10Q. We do not anticipate that the adoption of these SEC amendments will have a material effect on our financial statements.

In November 2018, ASU 2018-18 was issued to provide clarity on when transactions between entities in a collaborative arrangement should be accounted for under the new revenue standard, ASC 606. In determining whether transactions in collaborative arrangements should be accounted under the revenue standard, the update specifies that entities shall apply unit of account guidance to identify distinct goods or services and whether such goods and services are separately identifiable from other promises in the contract. ASU 2018-18 also precludes entities from presenting transactions with a collaborative partner which are not in scope of the new revenue standard together with revenue from

contracts with customers. The accounting update is effective January 1, 2020 and early adoption is permitted. The adoption of ASU 2018-18 is not expected to have an impact on our financial statements.

In December 2018, the FASB issued ASU 2018-20, Leases (Topic 842): Narrow-Scope Improvements for Lessors *(ASU 2018-20)*, which addressed implementation questions arising from stakeholders in regard to ASU 2016-02, Leases. Specifically addressed in this update were issues related to 1) sales taxes and other similar taxes collected from lessees, 2) certain lessor costs, and 3) recognition of variable payments for contracts with lease and nonlease components. The amendments in this ASU are effective in the same time-frame as ASU 2016-02 as discussed above. We incorporated this ASU into its assessment and adoption of ASU 2016-02.

Results of Operations

Comparison of Three Months Ended March 31, 2019 and 2018

Product net revenue. We began commercial sales of ELZONRIS within the U.S., in January 2019, following receipt of FDA marketing approval on December 21, 2018. For the quarter ended March 31, 2019, we recorded $5.0 million of product revenue. We had no product revenue during the first quarter of 2018.

Costs of goods sold. Cost of goods sold for the first quarter of 2019 was $0.1 million and relates primarily to royalties owed to the licensor of ELZONRIS. The majority of manufacturing costs for ELZONRIS units recognized as revenue during the three months ended March 31, 2019 were expensed to research and development prior to FDA approval on December 21, 2018. We had no cost of goods sold during the first quarter of 2018.

Research and development expense. Research and development expense was $17.0 million for the quarter ended March 31, 2019, compared with $12.7 million for the quarter ended March 31, 2018, representing an increase of $4.3 million. The higher costs were primarily due to the $4.4 million expense recorded during the quarter ended March 31, 2019 relating to a milestone payment payable to LLS following FDA approval of our BLA for ELZONRIS and first commercial sale.

Selling, general and administrative expense. Selling, general and administrative expense was $16.0 million for the quarter ended March 31, 2019, compared with $5.9 million for the quarter ended March 31, 2018, representing an increase of $10.1 million. The increase in costs were primarily attributable to launch expenses in support of the commercialization of ELZONRIS and compensation costs related to increase in headcount to support the commercial launch.

Interest income. Interest income was $0.5 million for the quarter ended March 31, 2019, compared with $0.2 million for the quarter ended March 31, 2018, representing an increase of $0.3 million. The increase was primarily due to higher average cash and investment balances during 2019 versus the prior year.

Liquidity and Capital Resources

Sources of Liquidity

As of March 31, 2019, our cash, cash equivalents and short-term investments totaled $124.4 million. We primarily invest our cash, cash equivalents, and 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, and short-term investments will be sufficient to fund our operations for at least the next two years.

We have financed our operations to date primarily through proceeds from public sales of common stock via our 2013 initial public offering, or IPO, and subsequent follow-on public offerings. Since inception through March 31, 2019, we received net proceeds of $364.4 million from these offerings. We expect our operations to be partially funded in the future by cash inflows from revenues from ELZONRIS. We generated $5.0 million of net product revenues from ELZONRIS for the three months ended March 31, 2019. We have incurred losses and generated negative cash flows from operations since inception. On January 18, 2019, the Company completed its 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.

Cash Flows

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

	Three Months Ended March 31,	
	2019	2018
Net cash used in operating activities	$ (22,137,027)	$ (17,085,227)
Net cash used in investing activities	(49,629,151)	(29,622,123)
Net cash provided by financing activities	86,337,021	57,143,090
Net increase in cash and cash equivalents	$ 14,570,843	$ 10,435,740

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 during the three months ended March 31, 2019 and 2018 resulted from research and development expenses as we continue our clinical trial activities relating to ELZONRIS, SL-801, and SL-701. Additional research and development costs also include CMC-related expenses for the manufacture of drug substance and drug product of our product candidates in development. Our cash from operating activities was also effected by the manufacturing of commercial drug product during the three-month period ended March 31, 2019. Additionally, our use of cash reflected significant commercial infrastructure expenses to support the U.S. commercial launch of ELZONRIS in December 2018.

Investing activities. The net cash provided by and used in financing activities for the three months ended March 31, 2019 and 2018, respectively, 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 the three months ended March 31, 2019 resulted primarily from our January 2019 issuance and sale of 10,222,222 common shares via our follow-on public offering. We sold 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. The net cash provided by financing activities for the three months ended March 31, 2018 resulted primarily from our January 2018 issuance and sale of 4,255,000 common shares via our follow-on public offering. We sold 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.

Funding Requirements

Our product candidates are in clinical or preclinical development, including ELZONRIS for CMML, MF, AML, and potentially other indications. 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 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 our ongoing litigation;
- maintain, leverage and expand our intellectual property portfolio; and
- add 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 future commercialization activities, including product sales promotion, marketing, manufacturing and distribution, for our approved product;
- 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 of our product or product candidates;
- income, if any, received from commercial sales of our product or product candidates, should 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, as we can generate substantial product 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 funds 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 funds through equity or debt financings when needed, we may be required to delay, limit, reduce or terminate our product development or future commercialization efforts or grant rights to develop and market product candidates that we would otherwise prefer to develop and market ourselves.

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 March 31, 2019, we had net operating losses of $238.4 million for federal and $242.2 million for state purposes, which are available to reduce future taxable income. We also had federal tax credits of approximately $39 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, except for net operating losses generated starting on January 1, 2019 and going forward, which have an unlimited life. 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 an ownership change. Subsequent ownership changes may further affect the limitation in future years. At March 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.

Item 3. 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 $124.4 million as of March 31, 2019 and $106.2 million as of March 31, 2018, consisting of cash, U.S. Treasury and Agency securities, 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 fair market 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 March 31, 2019 and March 31, 2018, all of our liabilities were denominated in our functional currency.

Item 4. Controls and Procedures.

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, as of the end of the period covered by this Quarterly Report on Form 10-Q, the effectiveness of our disclosure controls and procedures. 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 officer 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 March 31, 2019, our Chief Executive Officer and Chief Accounting Officer concluded that, as of such date, our disclosure controls and procedures were effective.

Changes to Internal Controls Over Financial Reporting

There has been no change in our internal controls over financial reporting that occurred during the period covered by this report that has materially affected, or is reasonably likely to materially affect, our internal control over financial reporting.

Limitations on the Effectiveness of Controls

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

PART II: OTHER INFORMATION

Item 1. Legal Proceedings.

From time to time, we are involved in legal proceedings in the ordinary course of our business. Refer to Footnote 17: Commitments and Contingencies for more information on legal proceedings.

Item 1A. Risk Factors.

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

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

We are heavily dependent on the success of our product candidates and clinical product candidates and we cannot provide any assurance that any of our 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. We currently generate no income from our clinical product candidates, and we may never be able to develop or commercialize a marketable drug from those clinical product candidates.

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 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 market or promote any of our product candidates before we receive regulatory approval from the FDA or comparable foreign regulatory authorities, and we may never receive such regulatory approval for 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 uses 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 regulatory approval for trial initiation. Further, the FDA, an independent review committee, or IRC, or an oncologic drugs advisory committee, or ODAC, may not agree with the interpretation by our investigators or us of the clinical safety and efficacy of our product candidates, and our product candidates may not receive regulatory approval.

If we do not receive regulatory approvals for our product candidates, we may not be able to continue our operations. Even if we successfully obtain regulatory approvals to market our product candidates, our income will be dependent, in part, upon the prescribing information, adoption within clinical practice guidelines, and the size of the markets in the territories for which we gain regulatory approval and have commercial rights. In addition, our income will be dependent, in part, upon the market acceptance of our product once approved, as well as upon reimbursement and

coverage, among other things. If the markets for patient subsets that we plan to target are not as significant as we estimate, we may not generate significant income from sales of such products, if approved.

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 of 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 and potentially in 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 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 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 BPDCN where there had been limited epidemiologic and published data, and databases, and no previous drug approvals and 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 or halted 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 on the circumstances;
- failure of our third-party contractors, including CROs and CMOs, or our investigators, to comply with regulatory requirements or otherwise meet contractual obligations in a timely manner;
- delays or failure in obtaining the necessary approvals from regulators, institutional review boards, or IRBs, or scientific review committees, or SRCs, in order to commence or continue a clinical trial;
- our inability to manufacture, or obtain from third-parties, adequate supply of drug substance, drug product or adjuvant therapies sufficient to complete our preclinical studies and clinical trials;
- risk of loss of drug product, adjuvants and/or other components of the product, due to third-party storage and distribution of such supplies;

- the FDA, or other regulatory authority, issuing a clinical hold or requiring alterations to any of our study designs, including extending a study or requiring new studies, 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;
- receipt by a competitor of marketing approval for a product targeting an indication that one of our product candidates targets, such that we are not "first to market" with our product candidate;
- governmental or regulatory delays and changes in regulatory leadership, requirements, policy and guidelines;
- differing interpretations of data by the FDA or similar foreign regulatory agencies; 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. For example, we have observed serious adverse events, including deaths, from or relating to capillary leak syndrome, or CLS, with ELZONRIS. The occurrence of these and other adverse events could jeopardize or preclude our ability to develop ELZONRIS in additional indications, including but not limited to CMML, obtain or maintain marketing approval for, or successfully commercialize, market, and sell any or all of our product candidates for one or more indications.

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 marketing authorization applications, and ultimately obtain marketing approval for our product candidates in a timely manner, or at all.

In any clinical trial of a product candidate, the results of such trial may not be adequate to support submission of a marketing application or marketing approval. Because our product candidates are intended for use in life-threatening diseases, in many cases we ultimately intend to seek marketing approval for each product candidate based on the

results of a single clinical trial, which may be open-label and single-group in nature. As a result, these trials may receive enhanced scrutiny from the FDA. For any such trial, if the FDA disagrees with our choice or definition of primary endpoint, or the results for the primary endpoint are not robust or significant or clinically beneficial enough, including relative to 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, spleen response, and/or response duration, in which case the FDA may refuse to approve a BLA or NDA based on such intended pivotal trial, despite meeting a primary endpoint of the study. The FDA may also require the completion of additional clinical trials before or as a condition for approving our product candidates.

If we experience delays in the completion of, or a termination of, any clinical trial of our product candidates, the commercial prospects of our product candidates will be harmed, which will have a negative impact on our ability to commence product sales and generate product 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.

Reports of adverse events or other safety concerns involving ELZONRIS and 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.

Reports of adverse events or other safety concerns involving ELZONRIS and our clinical drug candidates could interrupt, delay or halt our clinical trials. For example, CLS is a known, sometimes fatal, and well-documented side effect of ELZONRIS. Reports of additional 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. Further, patients receiving ELZONRIS or our product candidates with co-morbid diseases and/or indications not previously well-studied 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.

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

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

This drug development risk is heightened by any changes in ongoing and future clinical trials compared to completed clinical trials. As product candidates are advanced through preclinical studies to early and 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, 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-13Ra2 mutant peptide, EphA2 peptide, a new survivin mutant peptide, and a tetanus toxoid peptide. An earlier version of this immunotherapy, which included IL-13Ra2 mutant and EphA2 peptides, was mixed with additional peptides in previous studies, including a different survivin peptide in some studies.

- In the initial stage of our SL-701 corporate-sponsored trial, we used granulocyte-macrophage-colony-stimulating factor, or GM-CSF, and imiquimod as the immunostimulants. In the second stage of our SL-701 trial, we used poly-ICLC as the immunostimulant, which was the immunostimulant used, along with an earlier version of SL-701, in the previous investigator-sponsored study but is not currently commercially available. If the poly-ICLC regimen is found to be superior, it would require successful 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 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. We are currently combining ELZONRIS with pomalidomide in myeloma and have combined SL-701 with bevacizumab and immunostimulants in brain cancer.

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.

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 is unpredictable and depends upon numerous factors, including the substantial discretion of the regulatory authorities, which could include the prerequisite of an advisory panel, e.g. ODAC, review. In addition, approval policies, regulations, or the type and amount of preclinical, CMC, clinical pharmacology, and clinical data necessary to gain approval may change during the course of a product candidate's clinical development and may vary among jurisdictions. We may be required to undertake and complete certain additional preclinical, CMC, clinical pharmacology, bioanalytical, immunogenicity, or clinical studies to generate additional data required to support the submission of an 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. For example, we hope to have a meeting with the FDA to discuss the clinical development of ELZONRIS in CMML and potentially other indications. The FDA feedback may be difficult to implement or not implementable at all. Also, the FDA may require additional studies to support regulatory approval, which could result in a delay in our clinical programs and/or a delayed or unsuccessful regulatory filing, or no filing at all.

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 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 achieve the price we intend

to charge for our product candidates or obtain satisfactory reimbursement or coverage for our product candidates. Moreover, in many foreign countries, a product candidate must be approved for reimbursement before it can be approved for sale in that country and the reimbursement may be suboptimal. Any of the foregoing scenarios could materially harm the commercial prospects for our product candidates.

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

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

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

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 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 may, on further study, be shown to have harmful effects, to have characteristics that indicate it is unlikely to be safe and effective, 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 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 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;
- reduced or no protection over intellectual property rights;
- unexpected changes in tariffs, trade barriers, and regulatory requirements;
- economic weakness, including inflation, or political instability in particular foreign economies and markets;
- 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.;
- 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.

Also, see the Risk Factor titled “International expansion of our business exposes us to business, legal, regulatory, political, operational, financial, economic, and other risks associated with conducting business outside of the U.S.” 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.

International expansion of our business exposes us to business, legal, regulatory, political, operational, financial, economic, and other risks associated with conducting business outside of the U.S.

Part of our business strategy involves international expansion, including establishing and maintaining operations outside of the U.S. and establishing and maintaining relationships with health care providers, payors, government officials, distributors and manufacturers globally. Conducting business internationally involves a number of risks, including:

- multiple conflicting and changing laws and regulations such as tax laws, export and import restrictions, employment laws, anti-bribery and anti-corruption laws, regulatory requirements and other governmental approvals, permits and licenses;

- 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;
- difficulties in managing foreign operations;
- 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, and exposure to foreign currency exchange rate fluctuations;
- reduced protection for intellectual property rights;
- business interruptions resulting from geopolitical actions, economic instability, or natural disasters, including, but not limited to, wars and terrorism, political unrest, outbreak of disease, earthquakes, boycotts, curtailment of trade, and other business restrictions;
- failure to comply with foreign laws, regulations, standards and regulatory guidance governing the collection, use, disclosure, retention, security and transfer of personal data, including the European Union General Data Privacy Regulation, which introduces strict requirements for processing personal data of individuals within the European Union; and
- failure to comply with the Foreign Corrupt Practices Act, including its books and records provisions and its anti-bribery provisions, the United Kingdom Bribery Act 2010, and similar antibribery and anticorruption laws in other jurisdictions.

Also, see the Risk Factor titled "If we obtain approval to market any products outside of the U.S., a variety of risks associated with international operations could materially adversely affect our business." Any of these risks, if encountered, could significantly harm our future international expansion and operations and, consequently, negatively impact our financial condition, results of operations, and cash flows.

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 market withdrawal and we may be subject to 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 indicated uses 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, field alert or biological product deviation reporting, adverse event reporting, advertising, promotion and recordkeeping for the product will be subject to extensive and ongoing regulatory compliance requirements. These requirements include submissions of safety and other post-marketing information and reports, as well as continued compliance with cGMPs for commercial manufacturing and good clinical practices, or GCPs, for any clinical trials that we conduct post-approval. 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 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, obtains regulatory approval, the product may not gain market acceptance among physicians, third-party payors, patients and the medical community. For example, current cancer treatments such as chemotherapy and radiation therapy are well established in the medical community, and doctors may continue to rely on these treatments. The degree of market acceptance of ELZONRIS and any other product candidates for which we receive approval for commercial sale depends on a number of factors, including:

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

- the requirement for in-patient versus out-patient 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 (e.g., Medicare or Medicaid) and made changes to the coverage requirements under the Medicare Part D program. In 2012, the Supreme Court upheld the ACA in response to a lawsuit alleging that the individual mandate was unconstitutional. The Supreme Court held that the individual mandate and corresponding penalty was constitutional because it would be considered a tax by the federal government. The Supreme Court also upheld federal subsidies for purchasers of insurance through federally facilitated exchanges in a decision released in June 2015. 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 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.

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. While this decision has no immediate legal effect on the ACA and its provisions, this lawsuit is ongoing. Most recently, 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 applies 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. The new abbreviated regulatory pathway establishes legal authority for the FDA to review and approve biosimilar biologics, including the possible designation of a biosimilar as "interchangeable." The FDA defines an interchangeable biosimilar as a product that (1) can be expected to produce the same clinical result as the reference product in any given patient and (2), where the product is administered more than once, in terms of safety or diminished efficacy, presents no greater risk when switching between the biosimilar and its reference product than the risk of using the reference product alone. Under the BPCIA, an application for a biosimilar product cannot be submitted to the FDA until four years after, and approval by the FDA cannot be made effective until 12 years after, the date of the first licensure of the reference product. The law is complex and is only beginning to be interpreted by the FDA and the courts. For example, in June 2017, the United States Supreme Court, in Sandoz, Inc. v. Amgen Inc., issued an opinion potentially impacting the previously understood effective market exclusivity period. The BPCIA's ultimate impact, implementation, and meaning are subject to uncertainty. While it is uncertain when this development and approval process may be fully adopted by the FDA, the manner in which FDA implements the process could have a material adverse effect on the future commercial prospects for our biological products. In December 2018, FDA released guidance on biosimilar development, including questions and answers on biosimilars and biosimilarity. Although the guidance contains non-binding recommendations, they do describe the FDA's thinking on this topic. As policies and priorities shift, such guidance also may change.

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 March 31, 2019 of approximately $328.2 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 our plan to expand our discovery, research and development activities. We believe that our existing cash, cash equivalents, short-term investments and long-term investments, including the cash proceeds received from our follow-on public offering during the first quarter of 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.

As a result of these and other factors currently unknown to us, we may need to seek additional funds sooner than planned, including through public or private equity, debt financings or other sources, such as strategic partnerships and alliances and licensing arrangements. In addition, we may seek additional capital due to favorable market conditions or strategic considerations even if we believe we have sufficient funds for our current or future operating plans.

Our future capital requirements depend on many factors, including:

- the number of product candidates we pursue and the specific capital requirements to develop each;
- 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 with 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 at anticipated levels, reimbursement for our product or coverage is 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 amounts 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 product candidate development activities;
- obtain required regulatory approvals for the development and commercialization of our product candidates;
- maintain, defend, leverage and expand our intellectual property portfolio;
- build, deploy, and maintain sales, distribution and marketing capabilities, either on our own or in collaboration with strategic partners should our 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;

- 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 Astellas Pharma U.S., Inc., Bionomics Limited, Blueprint Medicines Corp., Boehringer Ingelheim GmbH, Celldex Therapeutics, Inc., Eli Lilly and Co., Ignyta, Inc. (a Roche company), Inspyr Therapeutics, Inc., Geron Corp., GlaxoSmithKline plc, Macrogenics Inc., Micromet, Inc. (an Amgen, Inc. company), OncoMed Pharmaceuticals, Inc., Pfizer Inc., Roche Holding AG, Sanofi U.S. LLC, Stemcentrx, Inc. (an AbbVie company), Sumitomo Dainippon Pharma Co. Ltd., TG Therapeutics, Inc., Verastem, Inc., AbbVie, Agios, Inc., Ambit Biosciences Corporation (now a Daiichi Sankyo company), Amgen, Astex Pharmaceuticals (now an Otsuka Pharmaceutical company), Celator Pharmaceuticals (now a Jazz Pharmaceuticals company), Celgene Corporation, Cellectis, CTI BioPharma, Cyclacel Pharmaceuticals, Inc., Eisai Co. Ltd., Genmab, Genzyme Corporation (now a Sanofi company), Humanigen, Inc., Immunogen, Incyte Corporation, Impact Biomedicines (now a Celgene company), Janssen Pharmaceutical Companies of Johnson & Johnson, Karyopharm Therapeutics, Inc., Kura Oncology, Inc., MustangBio, Inc., Novartis AG, Seattle Genetics, Inc., Sunesis Pharmaceuticals, Inc., and Xencor, Roche Holding AG, Eisai Co. Ltd.), Merck & Co., Inc., Agenus Inc., Bristol-Myers Squibb, Inc., Cortice Biosciences, Inc., CytRx Corporation, GenSpera, Inc., GlaxoSmithKline plc., ImmunoCellular Therapeutics, Ltd., Northwest Biotherapeutics, Inc., Novartis AG, Roche Holding AG, and others.

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

- design and develop products that address an unmet medical need or demonstrate a superior benefit/risk profile to other products in the market;
- conduct successful preclinical 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 May 10, 2019, we had 91 full-time employees, which may make us more reliant on our individual employees than companies with a greater number of employees. Although none of these individuals has informed us to date that he or she intends to retire or resign in the near future, the loss of services of any of these individuals or one or more of our other members of senior management could delay or prevent the successful development of our product pipeline, completion of our ongoing and future clinical trials or the commercialization and successful marketing launch of our product candidates.

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

In addition, we rely on consultants and advisors, including scientific and clinical advisors, to assist us in formulating our research and development and commercialization strategy. Our consultants and advisors may also be engaged with 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 may encounter difficulties in managing our growth and expanding our operations successfully.

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

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 preparation 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. Due to the limited experience of our management team in managing a company with this anticipated growth, we may not be able to effectively manage the expansion of our operations or recruit and train additional qualified personnel. 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 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 will be 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 broadly applicable fraud and abuse and other healthcare laws and regulations that may constrain the business or financial arrangements and relationships through which we market, sell and distribute our products for which we obtain marketing approval. Restrictions under applicable federal, state and foreign healthcare laws and regulations include the following:

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

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

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

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

- 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 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 will bear all of the risk related to the development of any such product candidates;
- the competitiveness of any product candidate that is commercialized could be reduced; and
- with respect to our platform technology, StemScreen®, we may not realize its potential as a means of identifying and validating new cancer therapies.

We rely on third-party manufacturers to produce and supply our 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 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 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 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.

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.

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

We 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 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.

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. 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 have to participate in interference proceedings declared by the United States Patent and Trademark Office, or USPTO, to determine priority of invention in the U.S. The costs of these proceedings could be substantial and it is possible that our efforts to establish priority of invention would be unsuccessful, resulting in a material adverse effect on our U.S. patent position. As a result, the issuance, scope, validity, enforceability and commercial value of our patent rights are highly uncertain. Our pending and future patent applications may not result in patents being issued which protect our technology or products, in whole or in part, or which effectively prevent others from commercializing competitive technologies and products. Changes in either the patent laws or interpretation of the patent laws in the U.S. and other countries may diminish the value of our patents or narrow the scope of our patent protection. For example, the federal courts of the U.S. have taken an increasingly dim view of the patent eligibility of certain subject matter, such as naturally occurring nucleic acid sequences, amino acid sequences and certain methods of utilizing same, which include their detection in a biological sample and diagnostic conclusions arising from their detection. Such subject matter, which had long been a staple of

the biotechnology and biopharmaceutical industry to protect their discoveries, is now considered, with few exceptions, ineligible in the first instance for protection under the patent laws of the U.S. Accordingly, we cannot predict the breadth of claims that may be allowed or enforced in our patents or in those licensed from a third-party.

Recent patent reform legislation could increase the uncertainties and costs surrounding the prosecution of our patent applications and the enforcement or defense of our issued patents. On September 16, 2011, the Leahy-Smith America Invents Act, or the Leahy-Smith Act, was signed into law. The Leahy-Smith Act includes a number of significant changes to 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 now provides a quicker and less expensive process for challenging issued patents. 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. However, the Leahy-Smith Act and its implementation could increase the uncertainties and costs surrounding the prosecution of our patent applications and the enforcement or defense of our issued patents, all of which could have a material adverse effect on our business and financial condition.

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

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

Our patents and patent applications may not be sufficient to protect our 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-13Rα2 peptide, as well as an issued European patent and allowed Japanese patent directed to the SL-701 composition, we currently do not have any U.S. composition of matter patent protection for SL-701. We do, however, have foreign pending patent applications, as well as issued patents in Australia and Mexico, which would cover certain uses of SL-701. While we have a non-exclusive license to issued U.S. patents directed to methods of use for the EphA2 peptide, we currently do not have any composition of matter patent protection, although we do have rights to foreign pending patent applications that seek to cover certain uses of this peptide. While we have filed U.S. and foreign patent applications directed to methods of use of a new survivin mutant peptide for use in SL-701, we currently do not have any composition-of-matter patent protection. With respect to SL-801, we have licensed composition of matter patents issued in the U.S. and abroad directed to the SL-801 compound. With respect to SL-901, we have licensed composition of matter patents issued in the U.S. and abroad directed to the SL-901 compound. 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. While we have an issued patent in Canada and a patent application pending in the U.S. directed to our StemScreen® technology, we currently have no issued patents covering StemScreen® in the U.S. Although we have various patent applications pending in the U.S. and abroad that we anticipate may result in additional protection for ELZONRIS, our clinical drug candidates and StemScreen®, there can be no assurance that any of these applications will result in an issued patent, or that if they issue, they will provide additional meaningful protection for these assets. Our inability to obtain adequate patent protection for our product candidates or platform technology could adversely affect our business.

Issued patents covering one or more of our product 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 product 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 product 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.

The issuance of a patent does not foreclose challenges to its inventorship, scope, validity or enforceability. Therefore, our owned and licensed patents may be challenged in the courts or patent offices in the United States and abroad. Such challenges may result in loss of exclusivity or in patent claims being narrowed, invalidated or held unenforceable, in whole or in part, which could limit our ability to stop others from using or commercializing similar or identical technology and products, or limit the duration of the patent protection of our technology and products. Given the amount of time required for the development, testing and regulatory review of new product candidates, patents protecting such product candidates might expire before or shortly after such product candidates are commercialized. As a result, our owned and licensed patent portfolio may not provide us with sufficient rights to exclude others from commercializing products similar or identical to ours.

Claims that our product 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 litigation could be existing patents or patents that issue in the future. Our failure to successfully defend against any claims that our product candidates or platform technology infringe the rights of third-parties could also adversely affect our business. Regardless of the outcome of any litigation, defending the litigation may be expensive, time-consuming and distracting to management. For example, we are aware of third-party patents with certain claims 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 product 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. Patent litigation could also expose us to significant monetary damages. This could harm our business significantly.

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

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

If third-parties successfully assert intellectual property rights against us, we might be barred from using certain aspects of our platform technology or barred from developing and commercializing certain products. Prohibitions against using certain technologies, or prohibitions against commercializing certain products, could be imposed by a court or by a settlement agreement between 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.

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

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

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

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 license from CanBas Co., Ltd. for SL-801 in all worldwide territories other than Japan, Korea, Taiwan, and China. 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. We also hold licenses from academic institutions relating to intellectual property underlying ELZONRIS and our product candidates and our StemScreen® platform technology. We expect to enter into additional license agreements as part of the development of our business.

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

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

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

upon insolvency. From time to time, we have had to request extensions of our development obligations contained in some of our license agreements, and we may need to seek further extensions in the future.

Although we have obtained such extensions in the past, there can be no assurance that our licensors will continue to extend the development timelines or other milestones contained in our license agreements. If these in-licenses are terminated, or if the underlying patents fail to provide the intended market exclusivity, we could lose our rights to develop and commercialize 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.

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

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

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

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

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

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

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

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

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

- others may be able to make compounds that are the same as or similar to our product 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 applicable laws provide a safe harbor exemption from infringement liability for certain research purposes, and then use the information learned from such activities to develop competitive products for sale in our major commercial markets;
- we may not develop additional proprietary technologies that are patentable; and
- the intellectual property rights of others may have an adverse effect on our business.

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

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

Risks Related to Our Common Stock

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

The market price of our common stock may be highly volatile, and could be subject to wide fluctuations in response to various factors, some of which are beyond our control. Since our initial public offering which occurred in January 2013, the price of our common stock has ranged from $3.88 per share to $47.25 per share. The stock market in general and the market for biopharmaceutical companies in particular have experienced extreme volatility that has often been unrelated to the operating performance of particular companies. The market price for our common stock may be influenced by many factors, including:

- results from or delays of clinical trials of our product 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;
- our ability to commercialize our approved product candidates;
- 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 19.4% of our outstanding capital stock. As a result, if these stockholders were to choose to act together, they would be able to exert substantial influence over all matters submitted to our stockholders for approval, as well as our management and affairs. For example, these persons, if they choose to act together, would exert substantial influence over the election of directors and approval of any merger, consolidation or sale of all or substantially all of our assets. This concentration of voting power could delay or prevent an acquisition of our company on terms that other stockholders may desire.

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

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

- establish a classified board of directors such that not all members of the board are elected at one time;

- allow the authorized number of our directors to be changed only by resolution of our board of directors;

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

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

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

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

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

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

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

their shares of our common stock, and could also affect the price that some investors are willing to pay for our common stock.

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

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

We 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 2. Unregistered Sales of Equity Securities and Use of Proceeds.

None.

Item 3. Defaults upon Senior Securities.

None.

Item 4. Mine Safety Disclosures.

Not Applicable.

Item 5. Other Information.

None.

Item 6. Exhibits.

The exhibits listed on the Exhibit Index are either filed or furnished with this report.

EXHIBIT INDEX

Exhibit Number	Description of Document
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., dated June 19, 2013, filed as Exhibit 3.3 to Form 10-Q on August 14, 2013 (File No. 001-35619) and incorporated herein by reference.
3.3	Certificate of Amendment of Restated Certificate of Incorporation of Stemline Therapeutics, Inc., dated June 20, 2017, filed as Exhibit 3.2 to Form 10-Q on August 8, 2017 (File No. 001-35619) and incorporated herein by reference.
3.4	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.
31.1	Certification 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, dated May 10, 2018.
31.2	Certification of Principal Financial and Accounting Officer pursuant to Rule 13a-14(a)/15d-14(a), as adopted pursuant to Section 302 of the Sarbanes-Oxley Act of 2002, dated May 10, 2018.
32.1	Certification of Principal Executive Officer pursuant to 18 U.S.C. §1350, as adopted pursuant to Section 906 of the Sarbanes-Oxley Act of 2002, dated May 10, 2018.
32.2	Certification of Principal Financial and Accounting Officer pursuant to 18 U.S.C. §1350, as adopted pursuant to Section 906 of the Sarbanes-Oxley Act of 2002, dated May 10, 2018.
101	Interactive data files pursuant to Rule 405 of Regulation S-T: (i) Balance Sheets, (ii) Statements of Operations, (iii) Statements of Comprehensive Loss, (iv) Statements of Stockholders' Equity, (v) Statements of Cash Flows, and (vi) the Notes to Financial Statements.

SIGNATURES

Pursuant to the requirements of the Securities Exchange Act of 1934, the registrant has duly caused this report to be signed on its behalf by the undersigned, thereunto duly authorized.

Date: May 10, 2019

STEMLINE THERAPEUTICS, INC.

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

Date: May 10, 2019

By: /s/ David G. Gionco
David G. Gionco
Vice President of Finance and Chief Accounting Officer
(Principal Financial and Accounting Officer)

Exhibit 31.1

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

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

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

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

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

4. The registrant's other certifying officer and I are responsible for establishing and maintaining disclosure controls and procedures (as defined in Exchange Act Rules 13a-15(e) and 15d-15(e)) 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: May 10, 2019

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

Exhibit 31.2

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

I, David G. Gionco, certify that:

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

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

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

4. The registrant's other certifying officer and I are responsible for establishing and maintaining disclosure controls and procedures (as defined in Exchange Act Rules 13a-15(e) and 15d-15(e)) 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: May 10, 2019

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

Exhibit 32.1

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

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

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

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

Date: May 10, 2019

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

Exhibit 32.2

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

In connection with the quarterly report of Stemline Therapeutics, Inc. (the "Company") on Form 10-Q for the period ended March 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: May 10, 2019

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