UNITED STATES
SECURITIES AND EXCHANGE COMMISSION

Washington, D.C. 20549

FORM 10-Q

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

For the quarterly period ended March 31, 2020

OR

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

For the transition period from to

Commission File Number 001-35619

STEMLINE THERAPEUTICS, INC.

(Exact name of registrant as specified in its charter)

Delaware	**45-0522567**
(State or other jurisdiction of incorporation or organization)	(I.R.S. Employer Identification 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)

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**

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 o

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 o

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	o	Accelerated filer	x
Non-accelerated filer	o	Smaller reporting company	x
		Emerging growth company	o

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. o

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

There were 52,510,785 shares of the registrant's common stock, $0.0001 par value, outstanding as of May 11, 2020.

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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 products and product candidates and comply with ongoing regulatory requirements, our ability to successfully operate in a competitive industry and gain market acceptance by physician, provider, patient, and payor communities, our reliance on third parties, unstable economic or market conditions, and our ability to obtain and adequately protect intellectual property rights for our product candidates.

By their nature, forward-looking statements involve risks and uncertainties because they relate to events, competitive dynamics, and healthcare, regulatory and scientific developments and depend on the economic circumstances that may or may not occur in the future or may occur on longer or shorter timelines than anticipated. Although we believe that we have a reasonable basis for each forward-looking statement contained in this Form 10-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. and abroad;
- the success of our commercial infrastructure buildout, launch, and commercialization in the U.S. and potentially in other regions of the world should ELZONRIS be approved for marketing by ex-U.S. regulatory authorities for any indication or by U.S. regulatory authorities for any as yet unapproved indication or should any of our other product candidates be approved for marketing in the U.S. or abroad;
- the success and timing of our clinical trials for ELZONRIS in unapproved indications and our other product candidates, including risks relating to regulatory authority approval, institutional review board approval, scientific review committee approval, site initiation, patient accrual, trial results including safety and efficacy, and the relevance of trial results to potentially viable regulatory pathways and commercialization efforts;
- the possibility that results of clinical trials are not predictive of safety and efficacy results of our products or product candidates in broader or additional patient populations;
- our ability to adhere to ongoing compliance requirements of all health authorities to which we are subject, in the U.S. and abroad;
- our ability to obtain and maintain adequate reimbursement for our products;
- product quality, efficacy or safety concerns resulting in complaints, adverse events, product recalls or regulatory action;
- the risk that estimates regarding the number of patients with the diseases that our products and product candidates are designed to treat are inaccurate, do not predict, or are not reflective of actual numbers;
- our products not gaining acceptance among patients, providers and/or third party payors, including governmental agencies, for certain approved indications, due to cost or otherwise;

- our ability to maintain or increase sales of ELZONRIS;
- the adequacy of our pharmacovigilance and drug safety reporting processes;
- the successful development and implementation of sales and marketing campaigns;
- the loss of key management, scientific, or other personnel;
- the success and timing of any regulatory filings for ELZONRIS, or any of our other product candidates, including for approval in the U.S., Europe, and other regions for any indication(s);
- changes in regulations in the U.S., Europe and any other regions where ELZONRIS may be approved, marketed and sold;
- new products, new product candidates or new uses for existing products or technologies introduced or announced, by our competitors, and the timing of these introductions or announcements;
- our available cash and investments;
- the accuracy of our estimates regarding expenses, future income or revenue, capital requirements and needs for additional financing;
- our ability to obtain additional funding;
- our ability to obtain and maintain intellectual property protection for our products and product candidates;
- delays, interruptions, or failures in the manufacture, supply and distribution of our products and/or our product candidates;
- our ability to maintain the license agreements for our products and product candidates;
- the ability of our third-party manufacturers to manufacture and supply our products, and the performance of, and our reliance on, our third-party manufacturers and suppliers;
- the performance of our third-party vendors, including clinical research organizations, clinical trial sponsors, and clinical trial investigators;
- the success of our preclinical, non-clinical, and pre-investigational new drug, or pre-IND, efforts;
- our ability to gain access to products we may plan to use in combination studies; and
- our ability to form corporate partnerships, should that be an avenue we choose to pursue.

In addition to the general factors listed above, on May 3, 2020, we entered into an Agreement and Plan of Merger (the “Merger Agreement”) with Berlin-Chemie AG, a company formed under the laws of Germany (“Berlin-Chemie”), and Mercury Merger Sub, Inc., a Delaware corporation and a wholly owned subsidiary of Berlin-Chemie (“Purchaser”). Additional factors specifically pertaining to the proposed transaction could also cause the results of performance to differ materially from those expressed in forward looking statements, including:

- the occurrence of any event, change, or other circumstance that could give rise to the right of one or both of the parties to terminate the Merger Agreement, which could materially and adversely affect our business, and cause the stock price to decline;
- the occurrence of any event, change, or other circumstance that could cause the transactions contemplated by the Merger Agreement to be uncompleted or delayed;
- the fact that there is a merger pending could have an adverse effect on our business and results of operations;

- legal proceedings related to the merger may be initiated and the outcome of any such proceedings may be adverse to us;
- the diversion of management and employee attention may detract from our ability to obtain regulatory approval for and, if approved, to successfully commercialize ELZONRIS in the European Union;
- the Merger Agreement may restrict us from engaging in advantageous business activities outside of our ordinary course of business without Purchaser's consent; and
- being unable to respond effectively to competitive pressures, industry developments and future opportunities as a result of the proposed merger.

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.
Condensed Consolidated Balance Sheets
(Unaudited)

	March 31, 2020	December 31, 2019
Assets		
Current assets:		
Cash and cash equivalents	$ 29,230,953	$ 13,561,712
Short-term investments	122,292,549	150,869,056
Accounts receivable, net	11,879,913	15,120,229
Inventories, net	1,389,173	1,151,373
Prepaid expenses and other current assets	4,277,739	4,459,127
Total current assets	169,070,327	185,161,497
Property and equipment, net	191,258	191,158
Operating lease right-of-use assets	1,056,149	1,317,598
Other assets	293,466	308,751
Total assets	$ 170,611,200	$ 186,979,004
Liabilities and stockholders' equity		
Current liabilities:		
Accounts payable and accrued expenses	$ 21,838,342	$ 21,952,048
Operating lease liabilities – current portion	909,295	1,169,764
Other current liabilities	10,252	10,125
Total current liabilities	22,757,889	23,131,937
Operating lease liabilities	204,773	226,306
Other liabilities	1,759	4,370
Total liabilities	22,964,421	23,362,613
Commitments and contingencies (Note 17)		
Stockholders' equity:		
Preferred stock $0.0001 par value, 5,000,000 shares authorized, none issued and outstanding at March 31, 2020 and December 31, 2019	—	—
Common stock $0.0001 par value, 83,750,000 shares authorized at March 31, 2020 and December 31, 2019. 52,504,598 shares issued and outstanding at March 31, 2020 and 50,349,150 shares issued and outstanding at December 31, 2019	5,250	5,035
Additional paid-in capital	534,241,439	529,488,474
Accumulated other comprehensive Income	598,884	28,171
Accumulated deficit	(387,198,794)	(365,905,289)
Total stockholders' equity	147,646,779	163,616,391
Total liabilities and stockholders' equity	$ 170,611,200	$ 186,979,004

See accompanying notes to unaudited condensed consolidated financial statements.

STEMLINE THERAPEUTICS, INC.
Condensed Consolidated Statements of Operations
(Unaudited)

	Three Months Ended March 31,	
	2020	**2019**
Revenue:		
Product revenue, net	$ 8,872,634	$ 5,048,590
Operating expenses:		
Cost of goods sold	640,986	85,728
Research and development	11,543,316	16,953,822
Selling, general and administrative	18,541,378	15,953,968
Total operating expenses	30,725,680	32,993,518
Loss from operations	(21,853,046)	(27,944,928)
Other expense, net	(14,808)	(4,324)
Interest expense	(35,154)	(292)
Interest income	643,249	538,584
Net loss before income taxes	(21,259,759)	(27,410,960)
Income tax (expense) benefit	(33,746)	3,694
Net loss	$ (21,293,505)	$ (27,407,266)
Net loss per common share:		
Basic and Diluted	$ (0.45)	$ (0.73)
Weighted-average shares outstanding:		
Basic and Diluted	47,674,613	37,550,931

See accompanying notes to unaudited condensed consolidated financial statements.

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

	Three Months Ended March 31,	
	2020	**2019**
Net loss	$ (21,293,505)	$ (27,407,266)
Other comprehensive income (loss):		
Foreign currency translation adjustments	(19,802)	—
Unrealized gain on investments, net of tax	590,515	25,074
Reclassification adjustment for loss on investments included in net loss	—	4,324
Other comprehensive income	570,713	29,398
Comprehensive loss	$ (20,722,792)	$ (27,377,868)

See accompanying notes to unaudited condensed consolidated financial statements.

STEMLINE THERAPEUTICS, INC.

Condensed Consolidated Statement of Stockholders' Equity

(Unaudited)

	Common Stock		Additional Paid-in Capital	Accumulated Other Comprehensive Income	Accumulated Deficit	Total Stockholders' Equity (Deficit)
	Shares	Capital				
Balance, December 31, 2019	50,349,150	$ 5,035	$ 529,488,474	$ 28,171	$ (365,905,289)	$ 163,616,391
Restricted stock grants	2,123,554	212	(212)	—	—	—
Forfeiture of restricted stock grants	(16,404)	(2)	2	—	—	—
Stock-based compensation expense	—	—	4,482,774	—	—	4,482,774
Employee Stock Purchase Plan compensation expense	—	—	55,028	—	—	55,028
Issuance of common stock in connection with the ESPP	37,333	4	191,141	—	—	191,145
Issuance of common stock in connection with the exercise of stock options	10,965	1	24,232	—	—	24,233
Net loss	—	—	—	—	(21,293,505)	(21,293,505)
Other comprehensive income	—	—	—	570,713	—	570,713
Balance, March 31, 2020	52,504,598	$ 5,250	$ 534,241,439	$ 598,884	$ (387,198,794)	$ 147,646,779

	Common Stock		Additional Paid-in Capital	Accumulated Other Comprehensive 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

See accompanying notes to unaudited condensed consolidated financial statements.

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

	Three Months Ended March 31,	
	2020	**2019**
Cash flows from operating activities		
Net loss	$ (21,293,505)	$ (27,407,266)
Adjustments to reconcile net loss to net cash used in operating activities:		
Depreciation and amortization expense	14,044	23,510
Stock-based compensation expense	4,460,774	7,206,092
Stock award — in-licensing	—	500,009
Employee Stock Purchase Plan compensation expense	55,028	24,853
Accretion of premium paid on marketable securities	(100,338)	(96,065)
Non-cash lease expense	277,362	—
Unrealized foreign exchange loss	14,808	—
Net loss on sale of marketable securities	—	4,324
Changes in operating assets and liabilities:		
Accounts receivable, net	3,240,316	(5,618,900)
Inventories, net	(215,800)	(848,493)
Other assets	15,284	—
Prepaid expenses and other current assets	180,906	(167,930)
Operating right-of-use assets	—	(1,738,680)
Accounts payable and accrued expenses	(121,945)	4,259,959
Operating lease liabilities	(297,914)	1,841,981
Other liabilities	(2,494)	(120,421)
Net cash used in operating activities	(13,773,474)	(22,137,027)
Cash flows from investing activities		
Purchase of fixed assets	(14,523)	(74,495)
Purchase of marketable securities	(10,047,640)	(81,499,610)
Sale and maturities of marketable securities	39,315,000	31,944,954
Net cash provided by (used in) investing activities	29,252,837	(49,629,151)
Cash flows from financing activities		
Proceeds from issuance of common stock from follow-on public offering, net	—	86,218,630
Proceeds from issuance of common stock from ESPP	191,145	102,566
Proceeds from exercise of stock options	24,233	15,825
Net cash provided by financing activities	215,378	86,337,021
Effect of exchange rate changes on cash and cash equivalents	(25,500)	—
Net increase in cash and cash equivalents	15,669,241	14,570,843
Cash and cash equivalents at beginning of period	13,561,712	9,443,667
Cash and cash equivalents at end of period	$ 29,230,953	$ 24,014,510

See accompanying notes to unaudited condensed consolidated financial statements.

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) through the clinical and regulatory process, launching and commercializing ELZONRIS, 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 evaluating ELZONRIS in additional indications and other product candidates in various indications, 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 consists of Stemline Therapeutics, Inc. in the U.S. and two wholly owned subsidiaries: Stemline Therapeutics, B.V. ("BV") incorporated in the Netherlands on August 15, 2019 and Stemline Therapeutics Switzerland, GmbH ("GmbH"), incorporated in Switzerland on October 3, 2019. The GmbH is a wholly owned subsidiary of the BV. The BV was set up to support the ELZONRIS Marketing Authorization Application ('MAA") to the European Medicines Agency ("EMA") in the European Union ("EU"). The GmbH is providing European pre-launch commercial marketing and medical education support for ELZONRIS.

The Company has incurred losses from operations since inception of $398.9 million. Since its inception, most of its resources have been dedicated to drug discovery and acquisition, intellectual property, manufacturing, preclinical and clinical development of product candidates, regulatory strategy and implementation, and commercialization of ELZONRIS, an FDA-approved product. In particular, the Company has expended and will continue to expend substantial resources for the foreseeable future commercializing its approved product in the U.S. and potentially abroad, should it be approved outside the U.S., 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 from its approved product and/or from the potential approval of its currently approved product in additional indications, and/or in additional territories, or its other product candidates. The Company expects its research and development expenses to increase 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 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 ex-U.S. territories.

As a result, the Company may 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 condensed consolidated 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 condensed consolidated 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, 2020, 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, 2019. The Company believes that its existing cash, cash equivalents and short-term investments will be sufficient to cover its cash flow requirements for at least the next twelve months from the issuance date of these condensed consolidated financial statements.

Principles of Consolidation

These condensed consolidated financial statements include the accounts of the Company and its wholly-owned subsidiaries. All intercompany accounts and transactions have been eliminated upon consolidation.

Use of Estimates

The preparation of financial statements in conformity with GAAP in the U.S. 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 contingencies in these condensed consolidated 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, 2019. During the three months ended March 31, 2020, there have been no changes to those policies.

Revenue Recognition

The Company sells ELZONRIS in the U.S. to one customer and one customer internationally through a title distribution channel. The U.S. customer subsequently resells ELZONRIS to a limited number of specialty distributors who, in turn, distribute ELZONRIS to specialty hospitals and the international customer subsequently resells and distributes ELZONRIS outside of the U.S. directly to hospitals in certain countries that participate in the early access program (EAP) in place as of March 31, 2020. For the three-months ended March 31, 2020, 82% of our product revenue, net was generated in the U.S with the remaining 18% achieved in Europe.

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

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

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

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

Government Contracts

The Company has entered into contracts (i) to participate in the Medicaid Drug Rebate Program and the Medicare Part D program, and (ii) to sell to the U.S. Department of Veterans Affairs, 340b entities and other government agencies ("Government Payors") so that ELZONRIS will be eligible for purchase by, in partial or full reimbursement from, such Government Payors. These reserves are recorded in the same period the revenue is recognized, resulting in a reduction of product revenue and accounts receivable (if the amount is payable to the Customer) or a current liability (if the amount is payable to a party other than a Customer). 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 purchased. To estimate sales with a right of return, the Company assesses, on a quarterly basis, the number of vials that are held in inventory throughout the distribution channel. Amounts for estimated product returns are established in the same period that the related gross revenue is recognized, resulting in a reduction of product revenue and the establishment of a current liability which is included as a component of accounts payable and accrued expenses.

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 accounts payable and accrued expenses.

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. The Company analyzes accounts that are past due for collectability and provides an allowance for receivables when collection becomes doubtful. No reserve has been recorded relating to an allowance for doubtful accounts at March 31, 2020.

Cost of Goods Sold

Cost of goods sold includes direct costs to manufacture commercial drug substance and drug product for ELZONRIS, as well as indirect costs including costs for packaging, shipping, insurance and quality assurance, idle capacity charges, write-offs for inventory that fails to meet specifications or is otherwise no longer suitable for commercial sale, and royalties due to the licensor of ELZONRIS related to the U.S. product sales recognized during the period.

Inventories, Net

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, 2020 were expensed to research and development prior to FDA approval of ELZONRIS on December 21, 2018.

The Company values its inventories at the lower of cost or estimated 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 goods sold. The determination of whether inventory costs will be realizable requires estimates by management. If actual market conditions are less favorable than projected by management, additional write-downs of inventory may be required which would be recorded as a cost of goods sold in the condensed consolidated statements of operations.

Foreign Currency

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

Accounting Standards Updates

In December 2019, the Financial Accounting Standards Board (FASB) issued ASU 2019-12, Simplifying the Accounting for Income Taxes, which simplifies the accounting for income taxes, eliminates certain exceptions within ASC Topic 740, Income Taxes, and clarifies certain aspects of the current guidance to promote consistency among reporting entities. Most amendments within the guidance are required to be applied on a prospective basis, while certain amendments must be applied on a retrospective or modified retrospective basis. ASU 2019-12 is effective for public entities for fiscal years, and interim periods within those fiscal years, beginning after December 15, 2020, with early adoption permitted. The Company adopted this standard on January 1, 2020. The Company adopted this guidance on January 1, 2020, and it had no impact on the Company's financial position, results of operations or 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 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. For available-for-sale debt securities with unrealized losses, this standard will require allowances to be recorded instead of reducing the amortized cost of the investment. ASU 2016-13 is effective for public entities, excluding entities eligible to be Small Reporting Companies, for fiscal years beginning after December 15, 2019, including interim periods within those fiscal years. In November 2019, the FASB issued No. 2019-10, *Financial Instruments—Credit Losses (Topic 326), Derivatives and Hedging (Topic 815), and Leases (Topic 842)*, which deferred the effective date of ASU 2016-13 for Small Reporting Companies for fiscal years beginning after December 15, 2022, including interim periods within those fiscal years, assuming the Company remains a Small Repoting Company. Early adoption is permitted. The Company is currently evaluating the impact of adopting ASU 2016-13 on the Company's financial statements.

Accounting standards updates adopted and/or issued, but not effective until after March 31, 2020 , are not expected to have a material effect on the Company's unaudited condensed consolidated financial position, annual results of operations and/or cash flows.

3. Liquidity and Capital Resources

As of March 31, 2020, the Company has approximately $151.5 million in cash, cash equivalents, and short-term investment securities. The Company primarily invests in highly liquid 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.

4. Prepaid Expenses and Other Current Assets

Prepaid expenses and other current assets consist of the following at March 31, 2020 and December 31, 2019:

	March 31, 2020	December 31, 2019
Prepaid third party vendor costs	$ 3,050,488	$ 3,105,088
Deposits	757,155	555,788
Prepaid insurance	4,154	140,356
Other receivable	465,942	657,895
Total	$ 4,277,739	$ 4,459,127

5. Property and Equipment, Net

Property and equipment, net, consist of the following at March 31, 2020 and December 31, 2019:

	March 31, 2020	December 31, 2019
Office furniture and fixtures	$ 537,341	$ 524,840
Manufacturing equipment	181,753	181,753
Leasehold improvements	82,694	82,694
Computer equipment	32,850	31,206
Property and equipment	834,638	820,493
Less accumulated depreciation and amortization	(643,380)	(629,335)
Property and equipment, net	$ 191,258	$ 191,158

Depreciation expense was $14,044 and $23,510 for the three-month periods ended March 31, 2020 and 2019, 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, 2020 and December 31, 2019:

	March 31, 2020			
	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, 2020
Assets:				
Fixed-income treasury portfolio	$ 122,292,549	$ —	$ —	$ 122,292,549
Cash and cash equivalents	29,230,953	—	—	29,230,953
Total assets at fair value	$ 151,523,502	$ —	$ —	$ 151,523,502

	December 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 December 31, 2019
Assets:				
Fixed-income treasury portfolio	$ 150,869,056	$ —	$ —	$ 150,869,056
Cash and cash equivalents	13,561,712	—	—	13,561,712
Total assets at fair value	$ 164,430,768	$ —	$ —	$ 164,430,768

The following is a summary of cash equivalents and available-for-sale investments held by the Company at March 31, 2020 and December 31, 2019:

	March 31, 2020			
	Amortized Cost	Gross Unrealized Gains*	Gross Unrealized Losses*	Estimated Fair Value
Cash:				
Cash from operating accounts	$ 5,212,245	$ —	$ —	$ 5,212,245
Cash equivalents:				
Money market funds	24,018,708	—	—	24,018,708
Total cash and cash equivalents	$ 29,230,953	$ —	$ —	$ 29,230,953
Short-term investments:				
Fixed-income treasury portfolio:				
Fannie Mae	5,058,610	32,225	—	5,090,835
Federal home loan bank	5,120,100	34,373	—	5,154,473
Freddie Mac	6,040,785	50,931	—	6,091,716
U.S. treasury securities	105,400,181	555,344	—	105,955,525
Total short-term investments	121,619,676	672,873	—	122,292,549
Total	$ 150,850,629	$ 672,873	$ —	$ 151,523,502

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

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

At March 31, 2020 and December 31, 2019, the remaining contractual maturities of available-for-sale investments classified as current on the balance sheet were less than 12 months.

There were no available-for-sale securities in a continuous unrealized loss position for greater than twelve months at March 31, 2020 and December 31, 2019. 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, 2020.

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 and short-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. Inventories, Net

Inventories, net, consists of the following:

	March 31, 2020
Raw materials	$ 6,116
Work-in-process	1,189,796
Finished goods	1,642,399
Inventory at cost	2,838,311
Inventory obsolescence reserves	(1,449,138)
Total Inventories, net	$ 1,389,173

Inventories, net, are related to our approved product, ELZONRIS. The Company recorded an additional $44,015 inventory obsolescence reserve during three-month period ended March 31, 2020, reflecting on hand ELZONRIS inventory that is projected to not sell through to the distribution channel prior to expiry.

8. Leases

The Company has leases for office facilities as well as for certain equipment. On March 3, 2020, the Company entered into a lease agreement to relocate our existing office space from the 11th floor at 750 Lexington Avenue, New York, New York, to the fourth floor in the same building. The lease will commence in the second quarter of 2020 and will expire in 2028 with a lease obligation of approximately $9.0 million. The Company expects to classify and recognize this new lease as an operating lease in the second quarter of 2020.

Operating lease 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, based on the Company's incremental borrowing rate at the commencement date. Lease expense is recognized as a single lease cost on a straight-line basis over the lease term.

During the three month periods ended March 31, 2020 and 2019, the Company recognized rent expense of $309,523 and 267,445, respectively, primarily related to our operating leases. Rent expense is included in selling, general and administrative expense in the condensed consolidated statements of operations.

Balance sheet information related to leases was as follows:	**March 31, 2020**
Operating Leases	
Operating lease right-of-use assets	$ 1,056,149
Operating lease liabilities - current portion	909,295
Operating lease liabilities	204,773
Total operating lease liabilities	$ 1,114,068
Weighted Average Remaining Lease Term — Operating leases	1.50 years
Weighted Average Discount Rate — Operating leases	7.49%

Future minimum lease payments under non-cancellable leases as of March 31, 2020 were as follows:

	Operating Leases
2020	$ 921,037
2021	110,248
2022	110,248
2023	27,562
Total future minimum lease payments	1,169,095
Less imputed interest	(55,027)
Present value of operating lease liabilities	$ 1,114,068

Supplemental cash flow information related to leases was as follows:

Cash paid for amounts included in the measurement of lease liabilities:	**March 31, 2020**
Operating cash flows from operating leases	$ 306,877

9. Accounts Payable and Accrued Expenses

Accounts payable and accrued expenses consist of the following at March 31, 2020 and December 31, 2019:

	March 31, 2020	December 31, 2019
Accrued research and development costs	$ 8,279,309	$ 9,012,182
Accrued commercial costs	7,846,508	4,674,204
Accrued compensation	1,883,512	5,598,551
Accrued sales deduction and allowance	1,289,020	1,360,033
Accrued general and administrative costs	1,419,759	708,042
Accrued legal	810,049	420,781
Accrued royalty	310,185	178,255
Total accounts payable and accrued expenses	$ 21,838,342	$ 21,952,048

10. Common Stock

As of March 31, 2020 and December 31, 2019, the Company was authorized to issue 83,750,000 shares of common stock.

Dividends on common stock will be paid when, and if, declared by the board of directors. Each holder of common stock is entitled to vote on all matters and is entitled to one vote for each share held. The Company will, at all times, reserve and keep available, out of its authorized but unissued shares of common stock, sufficient shares to affect the conversion of 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,	
	2020	2019
Balance at beginning of period	$ 28,171	$ (56,559)
Other comprehensive income before reclassification	570,713	25,074
Amounts reclassified from accumulated other comprehensive loss*	—	4,324
Total other comprehensive income	570,713	29,398
Balance at end of period	$ 598,884	$ (27,161)

*Amounts reclassified are included in other income in the Condensed Consolidated Statements of Operations.

12. Product revenue, Net

As of March 31, 2020, the Company's sole source of product revenue has been from sales of ELZONRIS. The following table summarizes activity in each of the product revenue allowance and reserve categories for the three months ended March 31, 2020:

	March 31, 2020				
	Government Rebates	Product Returns	Commercial Co-payment Assistance Programs	Distribution Fees	Total
Beginning balance at December 31, 2019	$ 578,115	$ 758,810	$ 129,862	$ 333,289	$ 1,800,076
Provision related to sales in the current year	960,428	15,970	—	277,196	1,253,594
Adjustments related to prior period sale	—	—	—	—	—
Credits and payments made	(876,793)	(171,010)	—	(308,800)	(1,356,603)
Ending balance at March 31, 2020	$ 661,750	$ 603,770	$ 129,862	$ 301,685	$ 1,697,067

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

The following table presents the Company's net revenue by major geographic region for the periods presented:

	Three Months Ended March 31,	
	2020	2019
United States	$ 7,318,026	$ 5,048,590
Europe	1,554,608	—
Total product revenue, net	$ 8,872,634	$ 5,048,590

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 position for all periods presented, diluted net loss per share is not presented since the common stock equivalents would have an anti-dilutive effect on the per share calculation.

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

	Three Months Ended March 31,	
	2020	2019
Basic and diluted net loss per common share calculation:		
Net loss	$ (21,293,505)	$ (27,407,266)
Basic and diluted weighted-average common shares	47,674,613	37,550,931
Basic and diluted net loss per share	$ (0.45)	$ (0.73)

The difference between basic and diluted weighted-average common shares generally results from the assumption that dilutive stock options outstanding were exercised and dilutive restricted stock has vested. For the three-month periods ended March 31, 2020 and 2019, 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,	
	2020	2019
Restricted stock awards	4,363,268	3,582,612
Restricted stock units	97,076	—
Options outstanding	2,722,695	3,535,018
Total	7,183,039	7,117,630

14. Grant Income

In October 2013, the Company entered into a contract with the the Leukemia and Lymphoma Society ("LLS") whereby LLS agreed to provide funding to the Company not to exceed $3.5 million. LLS is a national voluntary health organization that, among other activities, encourages and sponsors research relating to blood cancers to develop therapies to cure or mitigate these diseases. In consideration of funding by LLS and transfer to the Company of any rights LLS may have to any project inventions developed during the term of the agreement, the Company may be required to pay LLS a cash multiple on the LLS funding, less any amount the Company contributes to LLS to support the Company's preclinical and clinical development activities to bring ELZONRIS to commercialization. Through March 31, 2020, we have received the full $3.5 million based on milestones achieved. The Company is required to make a one-time payment upon regulatory approval and commercialization of ELZONRIS. Additionally, the Company is required to make payments based on achievement of specific sale-based commercial milestones. The total amount payable by the Company to LLS will not exceed three (3) times the amount of net funding received from LLS of $2.9 million. The Company may terminate the license for any or no reason upon 60 days advance written notice to LLS. If either party breaches a material obligation under the agreement and such obligation is not cured within a specified period of time following written notice from the other party, the nonbreaching party may terminate the agreement upon an additional written notice. As of March 31, 2020, we have paid $4.4 million pursuant to the agreement as funding for the education program to increase awareness of BPDCN. During the three months ended March 31, 2020, an additional $1.5 million liability was recorded in accounts payable and accrued expenses on the Company's consolidated balance sheet, as the Company met a new sales-based commercial milestone payable to LLS under this agreement within sixty days of achieving this milestone.

15. Income Taxes

The Company recorded $33,746 income tax expense related to income taxes due in foreign jurisdictions for the three-month period ended March 31, 2020 and $3,694 income tax benefit related to intraperiod tax allocations for the three-month period ended March 31, 2019. 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, the State of New York, Switzerland and the Netherlands. The Company currently has no ongoing audits.

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, 2020, the Company has recorded additional deferred tax assets, within the United States, which are fully offset by a valuation allowance. Realization of the deferred tax assets, within the United States, are 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.

Coronavirus Aid, Relief and Economic Security Act ("CARES Act")

In response to the COVID-19 pandemic, the Coronavirus Aid, Relief and Economic Security Act ("CARES Act") was signed into law on March 27, 2020. The CARES Act, among other things, includes tax provisions relating to refundable payroll tax credits, deferment of employer's social security payments, net operating loss utilization and carryback periods, modifications to the net interest deduction limitations and technical corrections to tax depreciation methods for qualified improvement property. At this time, the Company does not believe that the CARES Act will have a material impact on the Company's income tax provision for 2020. The Company will continue to evaluate the impact of the CARES Act on its financial position, results of operations and cash flows.

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. An amendment was also approved by the stockholders at the 2019 Annual Meeting to increase the authorized shares under the 2016 plan by an additional 2,500,000 shares.

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.

As of March 31, 2020, there were 426,210 shares of common stock available for future grants under the 2016 Plan.

The following table summarizes stock-based compensation related to the above plans by expense category for the three-month periods ended March 31, 2020 and 2019, respectively:

	Three Months Ended March 31,	
	2020	2019
Cost of goods sold	$ 55,000	$ —
Research and development	1,554,792	3,050,502
Selling, general and administrative	2,850,982	4,100,033
Total stock-based compensation recorded to income before taxes	4,460,774	7,150,535
Stock-based compensation expense capitalized in inventory	22,000	55,557
Total stock-based compensation expense	$ 4,482,774	$ 7,206,092

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 may be granted to directors 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, 2020 and 2019, respectively are as follows:

	Three Months Ended March 31,	
	2020	2019
Weighted-average volatility	64.81%	66.47%
Weighted-average risk-free interest rate	1.64%	2.59%
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, 2020, the Company issued 10,965 shares of the Company's common stock upon the exercise of outstanding stock options and received proceeds of approximately $24,233. As of March 31, 2020, there was approximately $3.7 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 2.23 years.

The following table summarizes the activity related to the Company's stock options for the three months ended March 31, 2020:

	Options	Weighted-Average Exercise Price	Weighted-Average Remaining Contractual Life	Aggregate Intrinsic Value
Outstanding at December 31, 2019	3,026,401	$ 10.39		
Options granted	9,000	6.97		
Options exercised	(10,965)	2.21		
Options forfeited	(301,741)	2.61		
Outstanding at March 31, 2020	2,722,695	$ 11.27	5.48	$ 892,783
Options exercisable at March 31, 2020	2,222,783	$ 10.67	4.84	$ 892,783

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, 2020 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, 2020. The intrinsic value of the Company's stock options changes based on the closing price of the Company's common stock.

Restricted Stock Awards

The Company grants restricted stock to its employees, directors, and non-employee consultants. Restricted stock is recorded as deferred compensation and charged against income on a straight-line basis over the vesting period, which ranges from immediate to four years in duration. If vesting of the award is based on a 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, 2020:

	Number of Shares	Weighted-Average Grant Date Fair Value Per Share
Outstanding at December 31, 2019	3,002,584	$ 11.37
Shares granted	2,123,554	6.86
Shares vested	(746,466)	10.14
Shares forfeited	(16,404)	15.11
Outstanding at March 31, 2020	4,363,268	$ 9.37

For the three-month period ended March 31, 2020, the Company granted 2,123,554 shares of restricted stock at a weighted-average grant date fair value of $6.86 per share amounting to approximately $14.6 million in total aggregate fair value. As of March 31, 2020, 4,363,268 shares remained unvested and there was approximately $35.8 million of unrecognized compensation cost related to restricted stock which is expected to be recognized over a remaining weighted-average period of approximately 1.91 years. The total fair value of restricted stock vested during the three-month periods ended March 31, 2020 and 2019 was approximately $7.6 million and $6.0 million, respectively.

In the event a modification is made to an equity award after the grant date, the Company records a change in stock-based compensation expense equal to the incremental fair value of the equity award immediately subsequent to the modification as compared to the fair value of the equity award immediately preceding the modification. During 2020, the Company modified certain outstanding equity award held by an employee. These modifications will result in incremental compensation cost of $0.2 million.

Restricted Stock Units

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

The following table summarizes the activity related to the Company's RSUs for the three months ended March 31, 2020:

	Number of Shares	Weighted-Average Grant Date Fair Value Per Unit
Outstanding at December 31, 2019	93,000	$ 12.71
Shares granted	26,076	6.88
Shares vested	—	—
Shares forfeited	(22,000)	14.88
Outstanding at March 31, 2020	97,076	$ 10.65

For the three months ended March 31, 2020, the Company granted 26,076 shares of RSUs at a weighted-average grant date fair value of $6.88 per share amounting to approximately $0.2 million in total aggregate fair value. As of March 31, 2020, 97,076 shares remained unvested and there was approximately $1.2 million of unrecognized compensation cost related to restricted stock units which is expected to be recognized over a remaining weighted-average period of approximately 3.24 years. There were no RSUs vestings during the three months ended March 31, 2020.

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 and $0.2 million of stock compensation expense related to the PSAs for three-month period ended March 31, 2020 and March 31, 2019, 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 $0.4 million and $2.5 million of stock compensation expense related to the PSAs for the three-month period ended March 31, 2020 and March 31, 2019, 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 $0.1 million and $0.2 million for the three-month periods ended March 31, 2020 and 2019, 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 $55,028 and $24,853 of compensation expense for the three months ended March 31, 2020 and 2019, 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, 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, 2020, the Company has paid or accrued $9.2 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 seven years totaling $38.7 million, with services to be rendered on some of these agreements through 2020. From inception through March 31, 2020, the Company has received and paid for services relating to these agreements in the amount of $33.2 million. In addition, the Company has a commercial supply agreement with a vendor in which the Company is required to manufacture at least one batch during 2020.

The Company has agreements in place with contract research organizations, or CROs, in connection with its clinical programs. The Company's total expenditures in the future would be approximately $0.7 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 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, 2020, the Company has received $3.5 million based on milestones achieved.

For the three-month period ended March 31, 2020, the Company recorded expense of approximately $1.5 million relating to the achievement of a post-approval milestone.

Contingencies

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

18. Subsequent Events

Agreement and Plan of Merger

On May 3, 2020, the Company entered into an Agreement and Plan of Merger (the "Merger Agreement") with Berlin-Chemie AG, a company formed under the laws of Germany ("Berlin-Chemie"), and Mercury Merger Sub, Inc., a Delaware corporation and a wholly owned subsidiary of Berlin-Chemie ("Purchaser"). A. Menarini - Industrie Farmaceutiche Riunite S.r.l, a company formed under the laws of Italy, is the ultimate parent of Berlin-Chemie and Purchaser (together, the "Menarini Group"). Purchaser will merge with and into Stemline and Stemline will continue as the surviving entity and become a private, wholly-owned subsidiary of Berlin-Chemie AG (the "Merger Transaction").

Pursuant to the terms and subject to the conditions of the Merger Agreement, Purchaser will commence a tender offer (the "Offer") no later than May 15, 2020 to acquire all of the outstanding shares of common stock of Stemline, $0.0001 par value per share (the "Shares"), at an offer price of (i) $11.50 per Share, net to the seller in cash, without interest (the "Cash Amount"), plus (ii) one contingent value right per Share (a "CVR"). Each CVR represents the right to receive (i) $1.00 in cash or (ii) for each Share subject to a stock option with an exercise price above $11.50 but below $12.50, the amount in cash equal to the excess of $12.50 over the per Share exercise price of such stock option (the "Milestone Payment"), which shall be payable upon the first sale by or on behalf of Stemline for use or consumption by the general public of ELZONRIS for the treatment of adult patients with BPDCN in the United Kingdom, France, Spain, Germany, or Italy after approval by the European Commission of a marketing authorization application in the European Union through the centralized procedure (the "Milestone"). If the Milestone is not achieved on or before December 31, 2021, the Milestone Payment will not be payable.

Purchaser's obligation to purchase the Shares tendered in the Offer is conditioned, among other things, upon the valid tender of a majority of the total number of Shares outstanding at the time of the expiration of the Offer and the expiration or termination of the waiting period under the Hart-Scott-Rodino Antitrust Improvements Act of 1976, as amended.

The Offer will initially expire at one minute after 11:59 p.m. Eastern Time 20 business days following the commencement of the Offer, unless otherwise agreed to in writing by Berlin-Chemie and Stemline.

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, 2019, 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, 2019, 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. ELZONRIS® (tagraxofusp-erzs) was approved by the U.S. Food and Drug Administration, or FDA, in December 2018 for the treatment of adult and pediatric patients, two years and older, with blastic plasmacytoid dendritic cell neoplasm, or BPDCN. ELZONRIS is the first treatment approved for BPDCN and the first approved CD123-directed therapy. In addition, we are also assessing ELZONRIS, as both single agent and in combination, in a variety of other indications including Phase 1 and 2 clinical trials in chronic myelomonocytic leukemia, or CMML, myelofibrosis, or MF, multiple myeloma, or MM, and acute myeloid leukemia, or AML, with additional trials planned including a CD123+ all-comers trial.

We are also advancing a pipeline of additional novel therapeutic candidates, including felezonexor (SL-801), an oral small molecule XPO-1 inhibitor currently in an ongoing Phase 1 trial of patients with advanced solid tumors, SL-901, a kinase inhibitor currently in investigational medicinal product dossier, or IMPD, enabling studies, and SL-1001, an oral small molecule RET (rearranged during transfection) kinase inhibitor currently in investigational new drug, or IND, directed studies intended for patients with a variety of genetically-defined malignancies.

Merger Agreement

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FDA-approved product

ELZONRIS

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

BPDCN, formerly known as blastic NK-cell lymphoma, is an aggressive, orphan hematologic malignancy with historically poor outcomes. BPDCN may present with features similar to, and can be mistaken for, certain diseases including AML, non-Hodgkin's lymphoma, or NHL, acute lymphoid leukemia, or ALL, myelodysplastic syndrome, or MDS, and CMML, and other malignancies including those with skin manifestations as well as certain or nonmalignant 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.

In January 2019, Stemline submitted a Marketing Authorization Application, or MAA, to the European Medicines Agency, or EMA, seeking approval of ELZONRIS for the treatment of adult patients with BPDCN. The application is being reviewed on a standard timeline under the centralized procedure, and we continue to interact with the EMA regarding the application. A scientific advisory group, or SAG, meeting to discuss clinical data of tagraxofusp in patients with BPDCN was held on March 5th, 2020. We are currently preparing our responses to the day 180 list of questions, and anticipate a possible oral examination in front of the Committee for Medicinal Products for Human Use, or CHMP, in mid-2020.

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

ELZONRIS – Market Expansion Efforts

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. Expression of CD123 has also been associated with poor prognosis and clinical outcomes in additional diseases including AML and CMML.

As such, we are seeking to broaden the commercial potential of ELZONRIS through ongoing clinical trials in additional indications including CMML, myelofibrosis, or MF, multiple myeloma, or MM, and acute myeloid leukemia, or AML, and potentially others.

Chronic Myelomonocytic Leukemia (CMML)

At the 2019 European Hematology Association, or EHA, and American Society of Clinical Oncology, or ASCO, annual conferences, we reported Phase 1/2 data from 23 patients with relapsed/refractory CMML who received ELZONRIS as a monotherapy in Stage 1 (lead-in, dose escalation stage) and Stage 2 (expansion stage). A new Stage 3a cohort is currently enrolling patients.

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

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

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

Myelofibrosis (MF)

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

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

The most common TRAEs, grade 3+, were anemia (14%), thrombocytopenia (7%) and fatigue (3%). There was also one case of CLS which was grade 3.

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

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

Multiple Myeloma (MM)

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

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

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

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

Acute Myeloid Leukemia (AML)

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

Additional trials and potential indications for ELZONRIS

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

Additional planned trials include ELZONRIS as monotherapy or in combination with other agents in a basket of CD123 + malignancies which may include ALL, hairy cell leukemia, Hodgkin's disease, and certain other lymphomas, as well as ELZONRIS trials in combination with other agents in patients with CMML and in trials in patients with CD123+ or BPDCN-like AML.

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

Clinical pipeline product candidates

SL-801

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

In September 2019, we provided an update at the European Society of Medical Oncology, or ESMO, Congress 2019 in Barcelona, Spain. Results from 52 heavily pre-treated solid tumor patients (~90% were third line or greater) with a wide spectrum of solid tumors, including gastrointestinal, breast, lung, neuroendocrine, ovarian, and others were presented. Schedule A was amended to Schedule B in an effort to improve tolerability while maintaining dose intensity. In Schedule B (n=7), one patient experienced grade 3 weakness. As such, the 75mg cohort was expanded and is being evaluated. The most common TRAEs were nausea (69%), vomiting (53%), fatigue (44%), decreased appetite (24%), and diarrhea (22%), and the most common TRAEs, grade 3 or higher, were nausea (9%), diarrhea and fatigue (4%) and vomiting (2%).

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

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

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

SL-1001

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

SL-901

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

SL-701

SL-701 is an immunotherapy designed to direct the immune system to attack targets present on brain cancer and other malignancies. SL-701 is comprised of several short synthetic peptides that correspond to epitopes of targets including IL-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.

SL-501

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

Financings

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

From inception through March 31, 2020, we have received net proceeds of $446.6 million from the sale of common stock.

We have never been profitable and our net loss from operations for the three months ended March 31, 2020 and 2019 was $21.3 million and $27.4 million, respectively. We expect to incur significant expenses. We expect our expenses to trend higher in connection with our ongoing activities, particularly as we advance our product candidates through preclinical activities and clinical trials to seek regulatory approval and, if approved, commercialize such product candidates. Accordingly, we may need additional financing to support our continuing operations. We will seek to fund our operations through public or private equity or debt financings or other sources. Adequate additional financing may not be available to us on acceptable terms, or at all. Our failure to raise capital as and when needed would have a negative impact on our financial condition and our ability to pursue our business strategy. We will need to generate significant revenues to achieve profitability, and we may never do so.

Litigation

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

Cost of Goods Sold

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

Research and Development Expenses

The following table shows our research and development expenses for the three-month periods ended March 31, 2020 and 2019, respectively:

	Three Months Ended March 31,	
	2020	**2019**
ELZONRIS	$ 5,184,440	$ 9,116,428
Other product candidates	1,394,204	1,722,290
Personnel expenses	4,125,879	5,550,846
Other expenses	838,793	564,258
Total research and development expenses	$ 11,543,316	$ 16,953,822

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

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

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

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

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

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

- the scope, enrollment, rate of progress and costs of our planned, as well as any additional, clinical trials and other research and development activities;
- timing and results of future clinical trials;
- the potential benefits of our product candidates over other therapies;
- the potential safety risks of our product 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 or product candidate could mean a significant change in the costs and timing associated with the development of that product or product candidate. For example, if the FDA or other regulatory authority were to require us to conduct clinical trials beyond those which we currently anticipate will be required for the completion of clinical development of a product candidate, we could be required to expend significant additional financial resources and time on the completion of clinical development. A similar result could occur if we experience significant delays in the progress of, including enrollment in, any clinical trials.

Selling, General and Administrative Expenses

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

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

Interest Income

Interest income consists of interest earned on our cash, cash equivalents, and short-term investments. 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.

Critical Accounting Policies and Estimates

To understand our consolidated financial statements, it is important to understand our critical accounting policies and estimates. We prepare our consolidated financial statements in accordance with generally accepted accounting principles, or GAAP. The preparation of financial statements also requires us to make estimates and assumptions that affect the reported amounts of assets, liabilities, costs and expenses and related disclosures. We base our estimates on historical experience and on various other assumptions that we believe to be reasonable under the circumstances. Actual results could differ significantly from the estimates made by our management. To the extent that there are differences between our estimates and actual results, our future 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, 2019. During the three months ended March 31, 2020, there have been no changes to those policies.

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 in the U.S. to our customer through its title distribution channel. The customer subsequently resells ELZONRIS to a limited number of specialty distributors who, in turn, distributes ELZONRIS to specialty hospitals. We recognize revenue on sales of ELZONRIS when our customer obtains control of the product, which occurs at a point in time (typically upon delivery). We also sell ELZONRIS to one customer internationally through a title distribution model. The international customer subsequently resells and distributes ELZONRIS outside of the U.S. directly to hospitals in certain countries that participate in the early access program (EAP) in place as of March 31, 2020. Product revenues are recorded at the wholesale acquisition costs, net of applicable reserves for variable consideration. Components of variable consideration include government rebates, product returns, commercial co-payment assistance programs, and distribution service fees. These reserves, as detailed below, are based on the amounts earned, or to be claimed on the related sales, and are classified as reductions of accounts receivable (if the amount is payable to the Customer and right of offset exists) or a current liability (if the amount is payable to a party other than a Customer). Overall, these reserves reflect our best estimates of the amount of consideration against product revenue that has been recognized. The amount of variable consideration which is included in the transaction price may be constrained and is included in the net sales price only to the extent that it is probable that a significant reversal in the amount of the cumulative revenue recognized under contracts will not occur in a future period. Our analyses contemplate the application of the constraint in accordance with ASC 606. Actual amounts of consideration ultimately received may differ from our estimates. If actual results in the future vary from our estimates, we will adjust these estimates, which will affect net product revenue and earnings in the period such variances become known.

Government Contracts

We entered into contracts (i) to participate in the Medicaid Drug Rebate Program and the Medicare Part D program, and (ii) to sell to the U.S. Department of Veterans Affairs, 340b entities, and other government agencies ("Government Payors") so that ELZONRIS will be eligible for purchase by, in partial or full reimbursement from, such Government Payors. These reserves are recorded in the same period the revenue is recognized, resulting in a reduction of product revenue and 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, 2020.

Inventories, Net

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, 2020 were expensed to research and development prior to FDA approval on December 21, 2018. We expect that our cost of goods sold as a percentage of net product revenue will increase during the second half of 2020 as future ELZONRIS units to be sold were manufactured after FDA approval and capitalized into inventory on the balance sheet.

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.

Foreign Currency

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

Recent Accounting Pronouncements

See Note 2, "Summary of Significant Accounting Policies" for our discussion about adopted and pending recent accounting standards.

Results of Operations

Comparison of Three Months Ended March 31, 2020 and 2019

Product revenue, net. 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, 2020, we recorded $8.9 million of product net revenue compared with $5.0 million for the quarter ended March 31, 2019, an increase of $3.9 million. The higher product net revenue resulted primarily from an increase in BPDCN patients treated with ELZONRIS. As the COVID-19 virus is a new phenomenon that we and the entire healthcare sector is dealing with, we believe it is still too early to predict the impact it will have on future net product revenue of ELZONRIS. COVID-19 could impact how we market and promote ELZONRIS and we are constantly re-evaluating our promotional tactics to adapt to the current marketplace.

Costs of goods sold. Cost of goods sold for the first quarter of 2020 was $0.6 million as compared to $0.1 million for the first quarter of 2019. The increase in cost of goods sold was primarily due to higher product net revenue during the quarter ended March 31, 2020 versus the prior year similar period. Additionally, the higher product costs were driven by increase stability costs incurred relating to drug product inventory.

Research and development expense. Research and development expense was $11.5 million for the quarter ended March 31, 2020, compared with $17.0 million for the quarter ended March 31, 2019, representing a decrease of $5.5 million. The majority of the lower costs were primarily driven by a $4.4 million expense recorded in the quarter ended March 31, 2019 relating to a milestone payment payable to the Leukemia and Lymphoma Society, following FDA approval of our BLA for ELZONRIS and first commercial sale. Additionally, the quarter ended March 31, 2019 included higher expenses related to non-cash stock-based compensation expense resulting from the vesting of performance-based equity awards and the manufacturing of ELZONRIS engineering batches for utilization in clinical trials.

Selling, general and administrative expense. Selling, general and administrative expense was $18.5 million for the quarter ended March 31, 2020, compared with $16.0 million for the quarter ended March 31, 2019, representing an increase of $2.5 million. The increase in costs were primarily attributable to ongoing U.S. launch expenses for ELZONRIS and pre-launch ELZONRIS-related costs in support of a potential regulatory approval and launch in the EU.

Interest income. Interest income was $0.6 million for the quarter ended March 31, 2020, compared with $0.5 million for the quarter ended March 31, 2019, representing an increase of $0.1 million. The increase was primarily due to higher average cash and investment balances during 2020 as compared to the prior year.

Liquidity and Capital Resources

Sources of Liquidity

As of March 31, 2020, our cash, cash equivalents and short-term investments totaled $151.5 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, 2020, we received net proceeds of $446.6 million from these offerings. We expect our operations to be partially funded in the future by cash inflows from revenues from ELZONRIS. We generated $8.9 million of net product revenues from ELZONRIS for the three months ended March 31, 2020. We have incurred losses and generated negative cash flows from operations since inception.

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,	
	2020	**2019**
Net cash used in operating activities	$ (13,773,474)	$ (22,137,027)
Net cash provided by (used in) investing activities	29,252,837	(49,629,151)
Net cash provided by financing activities	215,378	86,337,021
Exchange rate changes	(25,500)	—
Net increase in cash and cash equivalents	$ 15,669,241	$ 14,570,843

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, 2020 and 2019 resulted from research and development expenses as we continue our clinical trial activities relating to ELZONRIS, SL-801, and SL-701, as well as preclinical work related to SL-1001 and SL-901. Additional research and development costs also include CMC-related expenses for the manufacture of drug substance and drug product for 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, 2020. We also utilized a significant level of cash from our operating activities to support our commercial organization with selling and marketing activities relating to the launch of ELZONRIS in the U.S.

Investing activities. The net cash provided by and used in financing activities for the three months ended March 31, 2020 and 2019, respectively, reflects purchases and redemptions of short-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, 2020 resulted primarily from the issuance of common stock from the ESPP and exercise of stock options. 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.

Funding Requirements

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

- continue the ongoing clinical trials, and initiate additional clinical trials, of our product candidates;
- devise and implement our regulatory strategy, including for our regulatory filings in the U.S. and abroad;
- manufacture alternative formulations of ELZONRIS drug product;
- continue the research and development of ELZONRIS in additional indications and our other product candidates;
- seek to identify additional product candidates;
- acquire or in-license other products and technologies;
- seek marketing approvals for ELZONRIS in additional indications and 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 or future additional regulatory approvals;
- the scope, progress, results and costs of research and development, preclinical development, laboratory testing and clinical trials for our product candidates now or in the future;
- the extent to which we acquire or in-license other products and technologies;
- the costs, timing of regulatory preparation and outcome of regulatory review, in the U.S. or abroad, of our product or product candidates;
- income, if any, received from commercial sales of our product or product candidates, should our approved product be approved in additional indications or territories or any of our candidates, beyond our currently approved product, receive marketing approval;
- the cost of litigation with third parties, if any;
- the costs of preparing, filing and prosecuting patent applications, maintaining and enforcing our intellectual property rights and defending intellectual property-related claims; and
- our ability to establish any future collaboration arrangements on favorable terms, if at all.

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

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

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.

Income Taxes

Coronavirus Aid, Relief and Economic Security Act ("CARES Act")

In response to the COVID-19 pandemic, the Coronavirus Aid, Relief and Economic Security Act ("CARES Act") was signed into law on March 27, 2020. The CARES Act, among other things, includes tax provisions relating to refundable payroll tax credits, deferment of employer's social security payments, net operating loss utilization and carryback periods, modifications to the net interest deduction limitations and technical corrections to tax depreciation methods for qualified improvement property. At this time, we do not believe that the CARES Act will have a material impact on our income tax provision for 2020. We will continue to evaluate the impact of the CARES Act on our financial position, results of operations and cash flows.

Tax Loss Carryforwards

As of March 31, 2020, we had net operating losses of $307.4 million for federal and $311.2 million for state purposes, which are available to reduce future taxable income in the United States. We also had federal tax credits of approximately $39.0 million, which may be used to offset future tax liabilities within the United States. 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, 2020, we recorded a 100% valuation allowance against our net operating loss and tax credit carryforwards, within the United States, 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, and short-term investments of $151.5 million as of March 31, 2020 and $124.4 million as of March 31, 2019, 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, 2020 and March 31, 2019, 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, 2020, 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 additional current or future product candidates will be approved, commercialized, or successfully marketed in the future.

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

Before we generate any income from sales of our product candidates, clinical product candidates or future product candidates in the United States or elsewhere, we must complete preclinical and clinical development, conduct human subject research, submit clinical and manufacturing data to the U.S. Food and Drug Administration, or FDA, or foreign equivalent, qualify a third-party contract manufacturing organization, or CMO, satisfy the FDA or foreign equivalent that our CMO is capable of manufacturing the product in compliance with the FDA's current good manufacturing practices (CGMPs), submit a marketing application (e.g., Biologics License Application, or BLA, or foreign equivalent), or New Drug Application, or NDA, or foreign equivalent, receive regulatory approval from the FDA or a foreign regulatory authority, build a commercial organization, make substantial investments, and undertake significant marketing efforts ourselves or in partnership with others to ensure compliant marketing and market acceptance of any products we commercialize. We are not permitted to engage in unrestricted marketing or promotion of any of our product candidates before we receive regulatory approval from the FDA or comparable foreign regulatory authorities, and we may never receive such regulatory approval for our current or future product candidates.

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

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

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

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

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

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

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

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

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

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

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

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

Results of earlier clinical trials may not be predictive of the results of later-stage or subsequent clinical trials and results in the commercial setting may be different than clinical trial experience.

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

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

- As we optimize and scale-up production of our clinical product candidates, there may be manufacturing, formulation, fill-finish and other process and analytical changes that are part of the optimization and scale-up necessary for producing drug substance and drug product of a quality, quantity and stability sufficient for later stage clinical development and commercialization. Delays, including failures, in any of these steps, may delay initiation and completion of clinical trials, regulatory submissions, or commercial launch. We may also need to demonstrate comparability between newly manufactured drug substances and/or drug products relative to previously manufactured drug substances and/or drug products. Demonstrating comparability may cause us to incur additional costs or delay initiation or completion of our clinical trials, including the need or choice to initiate a dose escalation study, and, if unsuccessful, could require us to complete additional preclinical or clinical studies of our product candidates. Failure to demonstrate comparability could also result in delays in regulatory submissions or commercial launch. We are also developing a new lyophilized formulation of ELZONRIS. In the event that this formulation does not demonstrate comparability with the current liquid/frozen formulation, including from a pharmacokinetic, safety and/or efficacy perspective, the commercial success of ELZONRIS could be negatively impacted.

- We are, or may in the future be, treating patients with certain diseases or conditions that have not been previously treated with our product candidates. In these instances, we may choose to treat patients at several different doses and use multi-cycle dosing regimens to determine the optimal doses and schedules for both near-term and long-term safety and disease control in each indication. Use of our product candidates in new disease populations and at new dosing regimens could produce unforeseen adverse reactions and events that could impact the development and ability to obtain or maintain marketing approval for our product candidates.

- We may determine, based on safety and efficacy, that certain doses and regimens of our product candidates for particular indications are optimal for initial near-term therapy whereas the same, or other, doses and regimens are optimal for longer-term maintenance therapy.

- We are developing SL-701 as an injection administered under the skin, or subcutaneously, in our trials. Two previous investigator-sponsored trials of an earlier version of SL-701 used this method of delivery. Another previous investigator-sponsored trial of an earlier version of SL-701 used a different method of delivery, in which dendritic cells, which are a type of immune cell, were removed from the patient, exposed to immunogenic peptides, and then re-injected into or near a lymph node of the patient (intra/peri-nodally). Our plan continues with the subcutaneous injection method used in two of the previous studies and represents a change from one of the other previous studies.

- We manufactured and formulated SL-701 as a mixture of IL-13R2 mutant peptide, EphA2 peptide, a new survivin mutant peptide, and a tetanus toxoid peptide. An earlier version of this immunotherapy, which included IL-13R2 mutant and EphA2 peptides, was mixed with additional peptides in previous studies, including a different survivin peptide in some studies.

- In the initial stage of our SL-701 corporate-sponsored trial, we used granulocyte-macrophage-colony-stimulating factor, or GM-CSF, and imiquimod as the immunostimulants. In the second stage of our SL-701 trial, we used poly-ICLC as the immunostimulant, which was the immunostimulant used, along with an earlier version of SL-701, in the previous investigator-sponsored study but is not currently commercially available. If the poly-ICLC regimen is found to be superior, it would require successful approval and commercialization of poly-ICLC in addition to SL-701 to support product launch, which would entail a more complicated regulatory and commercialization strategy than required for a single product launch.

- In clinical trials, we have or are currently combining ELZONRIS with pomalidomide and dexamethasone in myeloma, ELZONRIS with a hypomethylating agent and potentially a Bcl-2 inhibitor and potentially certain chemotherapeutic agents in various indications including AML and BPDCN.

- In some of our current or future trials, we are, or may, combine our product candidates with each other or with other therapies such as chemotherapy, radiation, targeted therapy, or anti-angiogenic therapy which could result in unforeseen toxicities.

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

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

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

COVID-19 could impact our commercial efforts for ELZONRIS and could impact our other product candidates.

Our ability to successfully commercialize, market and sell ELZONRIS, may be impacted by the evolving COVID-19 pandemic, although we are currently unable to predict or quantify any such potential impact with any degree of certainty. To date, the pandemic has led to the implementation of various responses, including government-imposed quarantines, travel restrictions, changes in healthcare practice approaches, other public health safety measures, and closure of our offices. As a result of these responses, our marketing, sales, medical affairs, market access, and commercial efforts are happening virtually and the progress of these preparations may be impacted by the increased reliance on work-from-home arrangements for our employees, consultants, vendors, and potential customers. If the spread of COVID-19 and the social distancing measures taken by various governments continue, the commercialization of ELZONRIS and our product candidates, or efforts we may undertake for ELZONRIS and our product candidates, may be hindered by various factors, including challenges in hiring the employees necessary to support commercialization; delays in demand due to impacts on the healthcare system and overall economy; scarcity in inpatient capacity; delays in coverage decisions from Medicare and third-party payors; restrictions on our personal interactions with physicians, hospitals, payors, and other customers; interruptions or delays in our commercial supply chain; and increases in the number of uninsured or underinsured patients.

If any product candidate is approved but does not achieve an adequate level of acceptance by physicians, hospitals, healthcare payors and patients, we may not generate sufficient revenue from these products and we may not become or remain profitable, which would have a material adverse effect on our business.

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

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

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

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

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

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

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

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

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

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

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

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

Although we expect to focus a substantial amount of our efforts on the continued clinical testing and regulatory interactions for our clinical stage drug candidates, another key element of our strategy is to identify and test additional compounds. A portion of the preclinical research that we are conducting involves new and unproven drug discovery methods, as well as the preclinical testing of new compounds and potential new uses of existing compounds. The drug discovery that we are conducting using our StemScreen® platform technology may not be successful in identifying compounds that are useful in treating humans with cancer. Research programs designed to identify product candidates require substantial technical, financial and human resources, whether or not any product candidates suitable for approval and commercial marketing are ultimately identified. Even if our research programs may initially show promise in identifying potential product candidates, they may fail to yield product candidates for clinical development or commercialization for many reasons, including the following:

- the research methodology used may not be successful in identifying potential product candidates;
- competitors may develop alternatives that render our product candidates obsolete;
- a product candidate is subject to the ongoing collection of safety and efficacy data and may, on further study, be shown to have harmful side effects, be prone to serious adverse events, fail to continue to exhibit that characteristics that support the initial findings of safety and efficacy or to otherwise fail to meet applicable regulatory criteria;
- a product candidate may not be capable of being produced in commercial quantities at an acceptable cost, or at all; and
- a product candidate may not be accepted as safe and effective by regulatory authorities, patients, the medical community or third-party payors.

If we are unable to identify additional compounds for preclinical and clinical development, we may not have sufficient or any product income, which could result in significant harm to our financial position and adversely impact our stock price.

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

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

- different regulatory requirements for drug development and approvals and rules governing drug commercialization in foreign countries, including postmarket surveillance, monitoring and reporting;
- possible failure by us or our distributors to obtain appropriate licenses or regulatory approvals for the sale or use of our product candidates, if approved, in various countries;
- reduced or no protection over intellectual property rights;
- unexpected changes in tariffs, export and import restrictions, trade barriers, and regulatory requirements;
- economic weakness, including inflation, or political instability in particular foreign economies and markets;
- complexities associated with managing multiple payor-reimbursement regimes or self-pay systems;

- financial risks, such as longer payment cycles, difficulty enforcing contracts and collecting accounts receivable;
- compliance with tax, employment, immigration and labor laws for employees living or traveling abroad;
- foreign reimbursement, pricing, and insurance regimes;
- foreign taxes;
- foreign currency fluctuations, which could result in increased operating expenses and reduced revenue, and other obligations incident to doing business in another country;
- workforce uncertainty in countries where labor unrest is more common than in the U.S.;
- difficulties in managing foreign operations;
- compliance with the U.S. Foreign Corrupt Practices Act, the United Kingdom Bribery Act 2010, or similar antibribery and anticorruption laws in other jurisdictions as well as various regulations pertaining to data privacy, such as the European Union General Data Privacy Regulation;
- production shortages resulting from any events affecting raw material supply or manufacturing capabilities abroad; and
- business interruptions resulting from geopolitical actions, including war and terrorism, or natural disasters including earthquakes, typhoons, floods, and fires.

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

Any additional regulatory approvals that we or our potential strategic partners may receive for our product candidates may also be subject to limitations on the approved indication for use for which the product may be marketed or to the conditions of approval, may contain product contraindications, warnings, or precautions that limit the use of our product candidates or may contain requirements for potentially costly post-marketing testing, including Phase 4 clinical trials, and surveillance to monitor the safety and efficacy of our product candidates. In addition, with regard to ELZONRIS or any product candidates, should they become approved by the FDA, the manufacturing processes, testing, packaging, labeling, storage, distribution, post-market reporting, advertising, promotion and recordkeeping for the product will be subject to extensive and ongoing regulatory compliance requirements. These requirements include submissions of safety and other post-marketing information and reports, as well as continued compliance with CGMPs for commercial manufacturing and good clinical practices, or GCPs, for any clinical trials that we conduct post-approval. For example, we have several post-marketing commitments related to the FDA approval of ELZONRIS. In addition, there are now and may be in the future manufacturing, formulation, fill-finish and other process and analytical changes required by the FDA related to producing drug substance and drug product of a quality, quantity and stability sufficient for commercial supply. Changes, delays or failures in any of these steps may negatively affect disposition of manufactured batches of drug substance and/or drug product, and as a result, may require production of additional batches of drug substance and/or drug product. Issues that may arise with a product, including adverse events of unanticipated severity or frequency, or with our third-party manufacturers or manufacturing processes, or failure to comply with regulatory requirements, may result in, among other things:

- restrictions on the marketing or manufacturing of the product, withdrawal of the product from the market, or product recalls;
- warning letters, untitled letters, or holds on clinical trials;
- refusal by the FDA to approve pending applications or supplements to approved applications filed by us or our strategic partners, or suspension or revocation of product license approvals;
- product seizure or detention, or refusal to permit the import and export of products;
- investigations or inspections by government entities, including, but not limited to, FDA or foreign health authorities; and
- injunctions, fines, consent decrees, corporate integrity agreements, or the imposition of other civil penalties or criminal penalties.

We cannot predict the likelihood, nature or extent of government regulation that may arise from future legislation or administrative action, either in the United States or abroad. If we are slow or are unable to adapt to changes in existing requirements or the adoption of new requirements or policies, or if we are not able to maintain regulatory compliance, we may lose any marketing approval that we may have obtained and we may not achieve or sustain profitability, which would adversely affect our business.

The COVID-19 pandemic could have a material adverse effect on our clinical development program if the pandemic and associated government control measures continue.

The ongoing COVID-19 pandemic has presented substantial public health challenges and is impacting the global healthcare system, including the conduct of clinical trials in the U.S. and other parts of the world. As a result of the COVID-19 pandemic, we may encounter delays in our clinical trials. The majority of our clinical trials involve patients with cancer or those receiving ongoing immunosuppressive therapy who may be at higher risk of infection and are thus more likely to be subject to travel restrictions and self-quarantining. We have made efforts to allow patients currently enrolled in our ongoing clinical trials to continue unimpeded and have continued to allow new patients to enroll in our trials. We remain in close contact with clinical sites, CROs, and other third-party vendors, and have implemented measures to protect the health and safety of patients involved with our trials, and to preserve the integrity of our clinical data.

Further, we may not be able to complete our clinical trials that we initiated more recently and for which we have not yet completed enrollment in the time frame that we had previously planned. In addition, the pandemic may adversely affect our ability to conduct new trials. Some factors from the COVID-19 outbreak that may delay or otherwise adversely affect our clinical trial programs, as well as adversely impact our business generally, include:

- delays or difficulties in clinical site initiation, including difficulties in recruiting clinical sites, and delays enrolling patients in our clinical trials or increased rates of patients withdrawing from our clinical trials following enrollment as a result of contracting COVID-19, being forced to quarantine, or not otherwise being able to complete study assessments, particularly for older patients with a higher risk of contracting COVID-19;

- missed study visits or study procedures which could lead to an abundance of protocol deviations that have the potential to interfere with the interpretability of trial results;

- diversion of healthcare resources away from the conduct of clinical trials to focus on pandemic concerns, including clinical trial investigators and staff;

- limitations on travel, including limitations on domestic and international travel, and government-imposed quarantines that could interrupt key trial activities, such as clinical trial site initiations and monitoring;

- interruption of, or delays in receiving, supplies of our product candidates from our contract manufacturing organizations due to staffing shortages, or production slowdowns or stoppages; and

- disruptions and delays caused by potential workplace, laboratory and office closures and an increased reliance on employees working from home across the healthcare system.

To the extent the COVID-19 pandemic results in missed study visits or study procedures in our clinical trials, there could be an abundance of protocol deviations, which could impact the interpretability of the trial results. A significant number of deviations may call into question whether the execution of a clinical trial was consistent with the protocol.

We will continue to monitor the potential impact of COVID-19 on our clinical trial program, however, the full extent to which the COVID-19 pandemic may directly or indirectly impact the progress of our current and planned trials will depend on future developments that are highly uncertain and cannot be accurately predicted.

Risks Related to Commercialization of ELZONRIS and the Development and Commercialization of Our Product Candidates

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

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

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

- our inability to recruit, train, and retain adequate numbers of effective sales and marketing personnel;

- the inability of sales personnel to obtain access to physicians or effectively promote our approved product to physicians and other providers;

- the lack of complementary drug product to be offered by sales personnel, which may put us at a competitive disadvantage relative to companies with more extensive product lines;

- unforeseen costs and expenses associated with creating internal sales and marketing organizations;

- our inability to effectively build our manufacturing and commercial infrastructures to manufacture, market and sell our product candidates;

- our inability to build and staff, or enter into a partnership to support, an effective commercial distribution organization; and

- the addressable market for our product candidates may result in unsatisfactory income.

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

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

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

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

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

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

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

The regulations that govern marketing approvals, pricing and reimbursement for new drug products vary widely from country to country and are subject to changes in interpretation, application and new legislative proposals at any time. Some countries require the approval of the sale price of a drug before it can be marketed. In many countries, the pricing review period begins after marketing or product licensing approval is granted. In some foreign markets, prescription pharmaceutical pricing remains subject to continuing governmental control even after initial approval is granted. As a result, we might obtain marketing approval for a product in a particular country, but then be subject to price regulations that delay our commercial launch of the product, possibly for lengthy time periods, and negatively impact the income we are able to generate from the sale of the product in that country. Adverse pricing limitations may hinder our ability to recoup our investment in one or more product candidates, even if our product candidates obtain marketing approval.

Our ability to commercialize any products successfully also will depend in part on the extent to which reimbursement for these products and related treatments will be available from government health administration authorities, private health insurers and other organizations. Government authorities and third-party payors, such as private health insurers and health maintenance organizations, decide which medications they will cover and how much they will pay for them. A primary trend in the U.S. healthcare industry and elsewhere is cost containment. Government authorities and third-party payors have attempted to control costs by limiting coverage and the amount of reimbursement for particular medications. Increasingly, third-party payors are requiring that drug companies provide them with predetermined discounts from list prices and are challenging the prices charged for medical products. We cannot be sure that reimbursement will be available for any product that we commercialize and, if reimbursement is available, the level of reimbursement.

Reimbursement may impact the demand for, or the price of, any product candidate for which we obtain marketing approval. Obtaining reimbursement for our products may be particularly difficult because of the higher prices often associated with drugs administered under the supervision of a physician. If reimbursement is not available or is available only to limited levels, we may not be able to successfully commercialize any product candidate for which we obtain marketing approval.

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

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

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

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

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

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

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

Risks Related to Our Financial Position and Capital Requirements

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

We have incurred net losses from operations from our inception through March 31, 2020 of approximately $398.9 million. We do not know whether or when we will become profitable. Our losses have resulted principally from costs incurred in development and discovery activities. We anticipate that our operating losses will decrease over the next several years as we execute our strategy to commercialize ELZONRIS coupled with our plan to expand our discovery, research and development activities. We believe that our existing cash, cash equivalents, and investments, including the cash proceeds received from our follow-on public offering during the third quarter of 2019, will be sufficient to fund our operations and our capital expenditures for at least the next two years. If our cash is insufficient to meet future operating requirements, we will have to raise additional funds. If we are unable to obtain additional funds on terms favorable to us or at all, we may be required to cease or reduce our operating activities or sell or license to third-parties some or all of our intellectual property. If we raise additional funds by selling additional shares of our capital stock, the ownership interests of our stockholders will be diluted. If we need to raise additional funds through the sale or license of our intellectual property, we may be unable to do so on terms favorable to us, if at all. In addition, if we do not continue to meet our diligence obligations under our license agreements for our clinical drug candidates that we have in-licensed, we will lose our rights to develop and commercialize those clinical drug candidates.

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

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

Since our inception, most of our resources have been dedicated to the discovery, acquisition, preclinical and clinical development of our product candidates. We have expended and believe that we will continue to expend substantial resources for the development of our clinical product candidates and may expend additional resources on other product candidates and drug acquisition or discovery efforts. These expenditures will include costs associated with general administration, facilities, research and development, acquiring new technologies, manufacturing product and product candidates, conducting preclinical experiments, conducting clinical trials, preparing for and having regulatory interactions including applying for regulatory approvals, and commercializing ELZONRIS, as well as any product candidates that might receive approval for sale, as well as costs associated with operating as a public company.

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

Our future capital requirements depend on many factors, including:

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

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

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

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

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

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

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

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

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

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

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

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

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

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

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

Risks Related to Our Business and Industry

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

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

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

If we are unsuccessful in accomplishing these objectives, we may not be able to adequately commercialize our product(s) or product candidates, generate meaningful revenue, develop our product candidates, raise capital, expand our business, or continue our operations.

We face substantial competition, which may result in others discovering, developing or commercializing products before, or more successfully, than we do.

Our future success depends on our ability to demonstrate and maintain a competitive advantage with respect to the design, development, and commercialization of product candidates. Our competitors may succeed in developing competing products before we do for the same indications we are pursuing, obtaining regulatory approval or gaining acceptance for products or for the same markets that we plan to target. If we are not "first to market" with our product candidates, our competitive position could be compromised because it may be more difficult for us to obtain marketing approval for that product candidate and successfully market that product candidate as a competitor.

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

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

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

- design and develop products that address an unmet medical need or demonstrate a superior benefit/risk profile to other products in the market;
- conduct successful preclinical studies and clinical trials;
- attract qualified scientific, medical, sales, marketing and commercial personnel;
- obtain patent and/or other intellectual property protections for our processes and product candidates;
- obtain required regulatory approvals; and
- collaborate with others in the design, development and commercialization of new products.

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

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

Our success depends in part on our continued ability to attract, retain and motivate highly qualified management, clinical and scientific personnel. We are highly dependent upon our senior management as well as other employees, consultants and scientific and medical collaborators. As of March 16, 2020, we had 104 full-time employees, which may make us more reliant on our individual employees than companies with a greater number of employees. The loss of services of any of these individuals or one or more of our other members of senior management could delay or prevent the successful development of our product pipeline, completion of our ongoing and future clinical trials or the commercialization and successful marketing launch of our product candidates.

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

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

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

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

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

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

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

We face an inherent risk of product liability as a result of the clinical testing and commercial sale of our product candidates and products. For example, we may be sued if any product we develop allegedly causes or contributes to an injury or is found to be otherwise defective during product testing, clinical study, clinical use, manufacturing, marketing or sale. Any such product liability claims may include allegations of defects in manufacturing, defects in design, failure to warn of dangers inherent in the product, negligence, strict liability and a breach of warranties. Fraud-based claims, as well as claims made pursuant to state consumer protection acts, are also a possibility. If we cannot successfully defend ourselves against product liability claims, we may incur substantial liabilities or be required to limit commercialization of our product candidates. Even a successful defense would require significant financial and management resources. Regardless of the merits or eventual outcome, liability claims may result in:

- decreased demand for our product and product candidates that we may develop;
- injury to our reputation;
- withdrawal of clinical trial participants and inability to enroll future clinical trial participants;
- costs to defend the related litigation;
- a diversion of management's time and our resources;
- substantial monetary awards to trial participants or patients;
- product recalls, withdrawals or labeling, marketing or promotional restrictions;
- loss of income;
- the inability to commercialize our product candidates; and
- a decline in our stock price.

Our inability to obtain and retain sufficient product liability insurance at an acceptable cost to protect against potential product liability claims could prevent or inhibit the commercialization of products we develop. Although we maintain liability insurance, any claim that may be brought against us could result in a court judgment or settlement in an amount that is not covered, in whole or in part, by our insurance or that is in excess of the limits of our insurance coverage. Our insurance policies also have various exclusions, and we may be subject to a product liability claim for which we have no coverage. We will have to pay any amounts awarded by a court or negotiated in a settlement that exceed our coverage limitations or that are not covered by our insurance, and we may not have, or be able to obtain, sufficient capital to pay such amounts.

Our relationships with healthcare providers, physicians, and third-party payors in the United States and in foreign jurisdictions are subject to applicable anti-kickback, fraud and abuse and other healthcare laws and regulations, which could expose us to criminal, civil or administrative sanctions, contractual damages, potential exclusion from government-funded healthcare programs, reputational harm, and diminished profits and future earnings.

Healthcare providers, physicians and third-party payors in the United States and in foreign jurisdictions play a primary role in the recommendation and prescription of any product candidates for which we obtain marketing approval. Our future arrangements with third-party payors and customers may expose us to fraud and abuse and other healthcare laws and regulations that may constrain the business or financial arrangements and relationships through which we market, sell and distribute our products for which we obtain marketing approval. Restrictions under applicable federal, state and foreign healthcare laws and regulations include the following:

- the federal healthcare Anti-Kickback Statute prohibits, among other things, persons from knowingly and willfully soliciting, offering, receiving or providing remuneration, directly or indirectly, in cash or in kind, to induce or reward either the referral of an individual for, or the purchase, order or recommendation of, any good or service for which payment may be made under federal and state healthcare programs such as Medicare and Medicaid;
- the federal False Claims Act imposes civil penalties, against individuals or entities for knowingly presenting, or causing to be presented, to the federal government, claims for payment that are false or fraudulent or making a false statement to avoid, decrease or conceal an obligation to pay money to the federal government and also includes provisions allowing for private, civil whistleblower or "qui tam" actions;
- the federal Health Insurance Portability and Accountability Act of 1996, or HIPAA, as amended by the Health Information Technology for Economic and Clinical Health Act, or HITECH, imposes criminal and civil liability for executing a scheme to defraud any healthcare benefit program. HIPAA and HITECH also regulate the use and disclosure of identifiable health information by healthcare providers, health plans and healthcare clearinghouses, and impose obligations, including mandatory contractual terms, with respect to safeguarding the privacy, security and transmission of identifiable health information as well as requiring notification of regulatory breaches. HIPAA and HITECH violations may prompt civil and criminal enforcement actions as well as enforcement by state attorney generals;

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

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

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

- the U.S. Foreign Corrupt Practices Act, or FCPA, and other anti-corruption laws and regulations pertain to interactions with foreign government officials. The FCPA prohibits U.S. companies and their employees, officers, and representatives from paying, offering to pay, promising, or authorizing the payment of anything of value, directly or indirectly, to any foreign government official, which includes any officer, employee, political candidate or any person acting in an official capacity for or on behalf of any agency, instrumentality, department, subdivision, or other body of any national, state, or local government, for the purpose of influencing the foreign official in his or her official capacity, inducing the foreign official to do or omit to do an act in violation of his or her lawful duty, or to secure any improper advantage in order to obtain or retain business or to otherwise seek favorable treatment. In many countries in which we operate or sell our products, the healthcare professionals with whom we interact may be considered to be foreign government officials for purposes of the FCPA. The FCPA's Accounting Provisions separately require that publicly traded companies make and keep books, records and accounts, which in reasonable detail, accurately and fairly reflect the transactions and dispositions of the company's assets and devise and maintain a system of internal controls sufficient to assure management's control, authority, and responsibility over the company's assets;

- the European Union's General Data Protection Regulation and implementing laws in its member states govern the collection and processing of residents' personal data and, among other requirements, imposes certain consent and data access rights. Such laws may impact our ability to conduct clinical trials that involve personal data and engage in other activities that require the processing of personal data. Outside of the U.S. and the European Union, there are numerous other jurisdictions that have their own privacy and information security laws, and new laws and regulations are being considered and/or enacted globally, which may affect our ability to collect, process, and store their residents' personal data; and

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

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

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

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

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

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

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

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

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

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

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

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

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

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

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

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

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

We may not be successful in establishing and maintaining strategic partnerships, which could adversely affect our ability to develop and commercialize products.

We may seek to enter into strategic partnerships in the future, including alliances with other biotechnology or pharmaceutical companies, to enhance and accelerate the development and commercialization of our products in territories outside the United States. We face significant competition in seeking appropriate strategic partners and the negotiation process is time-consuming and complex. Moreover, we may not be successful in our efforts to establish a strategic partnership or other alternative arrangements for any future product candidates and programs because our research and development pipeline may be insufficient, our product candidates and programs may be deemed to be at too early a stage of development for collaborative effort, and/or third-parties may not view our product candidates and programs as having the requisite potential to demonstrate safety and efficacy. Even if we are successful in our efforts to establish strategic partnerships, the terms that we agree upon may not be favorable to us, and we may not be able to maintain such strategic partnerships if, for example, development or approval of a product candidate is delayed or sales of an approved product are disappointing.

If we ultimately determine that entering into strategic partnerships is in our best interest but either fail to enter into, are delayed in entering into, or fail to maintain such strategic partnerships:

- the development of certain of our current or future product candidates may be terminated or delayed;
- our cash expenditures related to development of certain of our current or future product candidates would increase significantly and we may need to seek additional financing;
- we may be required to hire additional employees or otherwise develop expertise, such as sales and marketing expertise, for which we have not budgeted;
- we may bear all of the risk related to the development of any such product candidates;
- the competitiveness of any product candidate that is commercialized could be reduced; and
- with respect to our platform technology, StemScreen®, we may not realize its potential as a means of identifying and validating new cancer therapies.

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

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

We also expect to rely upon third parties to produce and test drug substance and drug product required for the clinical trials and commercial supply of our product and product candidates. If we are unable to arrange for third-party manufacturing sources, or to do so on commercially reasonable terms or on a timely basis, we may not be able to complete the development of such other product candidates or market those that are approved. Reliance on third-party manufacturers entails risks to which we would not be subject if we manufactured product candidates ourselves, including reliance on the third-party for regulatory compliance and quality assurance, the possibility of breach of the manufacturing agreement by the third-party because of factors beyond our control (including a failure to synthesize and manufacture our product candidates in accordance with our product specifications), and the possibility of termination or nonrenewal of the agreement by the third-party, based on its own business priorities, at a time that is costly or damaging to us. We will be dependent on the ability of these third-party manufacturers to produce adequate supplies of drug product to support our clinical development programs, our supply needs for ELZONRIS, and future commercialization of any product candidates for which we may receive regulatory approval. In addition, the FDA and other regulatory authorities require that our products and product candidates be manufactured according to CGMPs, and similar standards for products manufactured for markets outside the U.S. Any failure by our third-party manufacturers to comply with CGMPs or failure to scale up manufacturing processes, including any failure to deliver sufficient quantities of product or product candidates of acceptable quality in a timely manner, could lead to a delay in, or failure to obtain, regulatory approval for trial initiation or marketing of any of our product candidates or commercial products. In addition, such failures could be the basis for action by the FDA to withdraw product approvals previously granted to us and for other regulatory action, which could result in or lead to recall, seizure, import alerts, fines, imposition of operating restrictions, total or partial suspension of production, injunctions, consent decrees, or civil or criminal sanctions.

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

We rely on our third-party manufacturers to purchase from third-party suppliers the materials necessary to produce our products, and product candidates for our clinical studies. There are a small number of suppliers for certain capital equipment and materials that we use to manufacture and test our drugs. Such suppliers may not sell these materials to our third-party manufacturers at the times we need them or on commercially reasonable terms. We do not have control over the process or timing of the acquisition of these materials by our third-party manufacturers. Moreover, we currently do not have any agreements for the commercial production of these materials. Although we generally do not begin a clinical trial unless we believe we have a sufficient supply of a product candidate to complete the clinical trial, any significant delay in the supply of a product candidate or the material components thereof for a clinical trial, including an ongoing clinical trial, due to the need to replace a third-party manufacturer, could considerably delay completion of our clinical studies, product testing and potential regulatory approval of our product candidates. If our third-party manufacturers or we are unable to purchase these materials after regulatory approval has been obtained for our product candidates, the commercial launch of our product candidates would be delayed or there would be a shortage in supply, which would impair our ability to generate revenues from the sale of our products or product candidates.

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

The third parties upon whom we rely for the manufacturing, and supply of our drug product, drug substance, starting materials and intermediates for drug substances used to manufacture ELZONRIS and our product candidates are our sole source of supply, and the loss or disruption of any of these suppliers, including as a result of the COVID-19 pandemic, could significantly harm our business.

Our ability to successfully develop our drug candidates, supply our drug candidates for clinical trials and to ultimately supply our commercial drugs, including ELZONRIS, in quantities sufficient to meet the market demand, depends in part on our ability to obtain the appropriate substances for these drugs in accordance with regulatory requirements and in sufficient quantities for clinical testing and commercialization. It is expected that many of our manufacturing partners will be sole source suppliers for the foreseeable future. Various raw materials, components, and testing services required for our products may also be single sourced. We are not certain that our single-source suppliers will be able to supply sufficient quantities of their products or on the timelines necessary to meet our needs, either because of our limited experience with those suppliers, our relative importance as a customer to those suppliers, public health emergencies such as the COVID-19 pandemic or natural disasters that may cause those suppliers to stop work for a period of time. If any of our suppliers ceases its operations for any reason or is unable or unwilling to supply the materials necessary for our drug product and product candidates in sufficient quantities or on the timelines necessary to meet our needs, it could significantly and adversely affect our business, the supply of our product candidates and our financial condition. In addition, if our current or future supply of any of our product candidates should fail to meet specifications during its stability program there could be a significant interruption of our supply of product, which would adversely affect the clinical development and commercialization of the product.

Although COVID-19 has not had a material adverse effect on our supply chain to date, no assurance can be given that it will not in the future if the situation persists or worsens. Many states and countries continue to be, or may become, subject to government-imposed quarantines and travel restrictions due to the COVID-19 pandemic. While all of our suppliers have been classified as "essential services", with no reduced operations at manufacturing and research locations to date, there is no guarantee that this will remain the case in perpetuity, and despite essential services classification, the rate of spread of COVID-19 could negatively impact staff availability and result in time-limited shutdowns. We are working closely with our contract manufacturer to ensure availability of sufficient commercial supply.

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

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

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

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

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

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

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

Our global commercialization strategy for certain of our product or product candidates may depend on our ability to enter into agreements with collaborators to obtain assistance and funding for the development and commercialization of these product or product candidates. Supporting diligence activities conducted by potential collaborators and negotiating the financial and other terms of a collaboration agreement are long and complex processes with uncertain results. Even if we are successful in entering into one or more collaboration agreements, collaborations may involve greater uncertainty for us, as we have less control over certain aspects of our collaborative programs than we do over our proprietary development and commercialization programs. We may determine that continuing to collaborate under the terms provided is not in our best interest, and we may terminate such collaboration. Our collaborators could delay or terminate their agreements, and our product or product candidates subject to collaborative arrangements may never be successfully commercialized.

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

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

Risks Related to Our Intellectual Property Rights

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

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

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

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

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

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

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

Failure to obtain patents directed to all approved uses of ELZONRIS may enable a competitor to market ELZONRIS for such approved but unpatented indication(s), which could lead to price erosion for sales of ELZONRIS. With respect to SL-701, although we have licensed an issued U.S. patent directed to the composition of matter for the mutant immunogenic IL-13Rα2 peptide, as well as issued patents in Europe, Japan, Australia, and Mexico directed to uses of the SL-701 composition, we currently do not have any composition of matter patent protection for SL-701. We have filed U.S. and foreign patent applications directed to methods of use of a new survivin mutant peptide for use in SL-701. The U.S. patent application directed to certain uses of a new survivin mutant peptide for use in SL-701 for treating brain cancer has been issued by the USPTO as U.S. 10,485,858 and patents have been issued for certain uses of the SL-701 composition in Europe and Japan. While we have a non-exclusive license to issued U.S. patents directed to methods of use for the EphA2 peptide, we currently do not have any composition of matter patent protection, although we do have rights to foreign pending patent applications that seek to cover certain uses of this peptide. With respect to SL-801 and SL-901, we have licensed composition of matter patents issued in the U.S. and abroad directed to the SL-801 compound and SL-901. With respect to SL-1001, we have filed U.S. and foreign patent applications directed to the SL-1001 composition, and these applications are pending Our inability to obtain adequate patent protection for our product candidates or platform technology could adversely affect our business.

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

If we were to initiate legal proceedings against a third-party to enforce a patent covering one of our products or product candidates, the defendant could counterclaim that our patent is invalid and/or unenforceable. In patent litigation in the United States, defendant counterclaims alleging invalidity and/or unenforceability are commonplace. Grounds for a validity challenge could be an alleged failure to meet any of several statutory requirements, for example, lack of patentable subject matter, novelty, obviousness, written description or non-enablement.

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

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

We cannot guarantee that our product or product candidates, the use of our product or product candidates, or our platform technology, StemScreen®, do not infringe third-party patents or other intellectual property. Third parties might allege that we are infringing their patent rights or that we have misappropriated their trade secrets. Such third parties might resort to litigation against us. The basis of such claims that our product candidates or platform technology infringe the rights of third parties could also adversely affect our business. Regardless of the outcome of any litigation, defending the litigation may be expensive, time-consuming and distracting to management. For example, we are aware of third-party patents with certain claims that may be directed to some of our product candidates, including one of the peptides used in SL-701 and structures which may be related to SL-1001. We may need to seek a license with respect to one or more of these third-party patents in order to commercialize our products. No assurance can be given that any such licenses will be available, or that they will be available on reasonable or commercially acceptable terms or at all. Failure to obtain any required licenses could restrict our ability to commercialize our products in certain territories or subject us to patent infringement litigation, could result in us having to cease commercialization of our products and/or subject us to money damages in such territories.

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

In order to avoid or settle potential claims with respect to any patent rights of third parties, we may choose or be required to seek a license from a third-party and be required to pay license fees or royalties or both. These licenses may not be available on expected, reasonable, or acceptable terms, or at all. Even if we or any future strategic partners were able to obtain a license, the rights may be non-exclusive, which could result in our competitors gaining access to the same intellectual property.

Ultimately, we could be prevented from commercializing one or more of our products or product candidates, or be forced to cease some aspect of our business operations, if, as a result of actual or threatened patent infringement claims, we are unable to enter into licenses on acceptable terms. Even if we were to ultimately prevail, or to settle at an early stage, such litigation could burden us with substantial unanticipated costs. In addition, litigation or threatened litigation could result in significant demands on the time and attention of our management team, distracting them from the pursuit of other company business.

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

If third parties successfully assert intellectual property rights against us, we might be barred from using certain aspects of our platform technology or barred from developing and commercializing certain products. Prohibitions against using certain technologies, or prohibitions against commercializing certain products, could be imposed by a court or by a settlement agreement between a patent owner and us. In addition, if we are unsuccessful in defending against allegations of patent infringement or misappropriation of trade secrets, we may be forced to pay substantial damage awards to the plaintiff. There is inevitable uncertainty in any litigation, including intellectual property litigation. There can be no assurance that we would prevail in any intellectual property litigation, even if the case against us is weak or flawed. If litigation leads to an outcome unfavorable to us, we may be required to obtain a license from the patent owner, in order to continue our research and development programs or to market our product. It is possible that the necessary licenses will not be available to us on commercially acceptable terms, or at all. This could limit our research and development activities, our ability to commercialize certain products, or both.

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

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

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

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

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

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

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

In addition, in spite of our best efforts, our licensors might conclude that we have materially breached our license agreements and might therefore seek to terminate the license agreements, thereby removing our ability to obtain regulatory approval and to market products covered by these license agreements. Our licensors may also seek to terminate the license agreements if we fail to satisfy our diligence obligations and/or meet specified milestones or upon insolvency. From time to time, we have had to request extensions of our development obligations contained in some of our license agreements, and we may need to seek further extensions in the future.

Although we have obtained such extensions in the past, there can be no assurance that our licensors will continue to extend the development timelines or other milestones contained in our license agreements. If these in-licenses are terminated, or if the underlying patents fail to provide the intended market exclusivity, we could lose our rights to develop and commercialize ELZONRIS and the product candidates governed by the licenses, and competitors would have the freedom to seek regulatory approval of, and to market, products identical to ours. This could have a material adverse effect on our competitive business position and our business prospects.

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

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

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

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

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

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

- others may be able to make compounds that are the same as or similar to our product or product candidates but that are not covered by the claims of the patents that we own or have exclusively licensed;
- we or our licensors or any future strategic partners might not have been the first to make the inventions covered by the issued patent or pending patent application that we own or have exclusively licensed;
- we or our licensors or any future strategic partners might not have been the first to file patent applications covering certain of our inventions;
- others may independently develop similar or alternative technologies or duplicate any of our technologies without infringing our intellectual property rights;
- it is possible that our pending patent applications will not lead to issued patents;
- it is possible that our applications for patent term extension for ELZONRIS pursuant to the Hatch Waxman Act will not result in added patent term, or may result in a shorter patent term extension than we applied for or that is available under the Hatch Waxman Act;
- the scope of our issued patents may not extend to competitive products developed or produced by others;
- issued patents that we own or have exclusively licensed may not provide us with any competitive advantages, or may be held invalid or unenforceable, as a result of legal challenges by our competitors;
- our competitors might conduct research and development activities in countries where we do not have patent rights or where the patent rights that we do not have are not comparable to those afforded in the United States, or where the applicable laws provide a safe harbor exemption from infringement liability for certain research purposes, and then use the information learned from such activities to develop competitive products for sale in our major commercial markets;
- we may not develop additional proprietary technologies that are patentable; and
- the intellectual property rights of others may have an adverse effect on our business.

Risks Related to Our Common Stock

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

The market price of our common stock may be highly volatile, and could be subject to wide fluctuations in response to various factors, some of which are beyond our control. Since our initial public offering which occurred in January 2013, the price of our common stock has ranged from $3.21 per share to $47.25 per share. The stock market in general and the market for biopharmaceutical companies in particular have experienced extreme volatility that has often been unrelated to the operating performance of particular companies. The market price for our common stock may be influenced by many factors, including:

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

The possibility of the economy's return to recessionary conditions and the possibility of further turmoil or volatility in the financial markets would likely have an adverse effect on our business, financial position, and results of operations.

The economy in the United States and globally has experienced volatility in recent years and may continue to experience such volatility for the foreseeable future. There can be no assurance that economic conditions will not worsen. Unfavorable or uncertain economic conditions can be caused by declines in economic growth, business activity, or investor or business confidence, limitations on the availability or increases in the cost of credit and capital, the timing and impact of changing governmental policies, natural disasters, epidemics / pandemics, such as coronavirus disease 2019 ("COVID-19"), terrorist attacks, acts of war, or a combination of these or other factors. A worsening of business and economic conditions could have adverse effects on our business, including substantial fluctuations in the market price of our common stock, which could decline below current levels.

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 43.4% 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 affected at a duly called stockholder meeting and prohibit actions by our stockholders by written consent;

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

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

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

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

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

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

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

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

Risks Related to our Proposed Merger with Berlin-Chemie AG

If the proposed merger is not completed, our business could be materially and adversely affected and our stock price could decline.

On May 3, 2020, we entered into an Agreement and Plan of Merger (the "Merger Agreement") with Berlin-Chemie AG, a company formed under the laws of Germany ("Berlin-Chemie"), and Mercury Merger Sub, Inc., a Delaware corporation and a wholly owned subsidiary of Berlin-Chemie ("Purchaser"). A. Menarini - Industrie Farmaceutiche Riunite S.r.l, a company formed under the laws of Italy, is the ultimate parent of Berlin-Chemie and Purchaser (together, the "Menarini Group"). Purchaser will merge with and into Stemline and Stemline will continue as the surviving entity and become a private, wholly-owned subsidiary of Berlin-Chemie AG (the "Merger Transaction").

Purchaser will commence a tender offer (the "Offer") no later than May 15, 2020 to acquire all of the outstanding shares of common stock of Stemline, $0.0001 par value per share (the "Shares"), at an offer price of (i) $11.50 per Share, plus (ii) one contingent value right per Share (a "CVR"). Each CVR represents the right to receive (i) the $1.00 in cash or (ii) for each Share subject to a stock option with an exercise price above $11.50 but below $12.50, the amount in cash equal to the excess of $12.50 over the per Share exercise price of such stock option (the "Milestone Payment"), which shall be payable upon the first sale by or on behalf of Stemline for use or consumption by the general public of ELZONRIS for the treatment of adult patients with BDPCN in the United Kingdom, France, Spain, Germany, or Italy after approval by the European Commission of a marketing authorization application in the European Union through the centralized procedure (the "Milestone"). If the Milestone is not achieved on or before December 31, 2021, the Milestone Payment will not be payable.

Consummation of the Merger Transaction is conditioned upon Purchaser purchasing Shares tendered in the Offer which is subject to the satisfaction or waiver of a number of conditions set forth in Annex I to the Merger Agreement, including (i) Stemline shall have validly tendered Shares representing one more Share than 50% of the total number of Shares outstanding at the time of the expiration of the Offer; and (ii) the expiration or termination of the waiting period under the Hart-Scott-Rodino Antitrust Improvements Act of 1976, as amended (the "HSR Act"). Therefore, the Merger Transaction may not be completed or may not be completed as quickly as expected. If the Merger Agreement is terminated, the market price of our ordinary shares will likely decline, as we believe that our market price reflects an assumption that the Merger Transaction will be completed. In addition, our share price may be adversely affected as a result of the fact that we have incurred and will continue to incur significant expenses related to the Merger Transaction that will not be recovered if the Merger Transaction is not completed. If the Merger Agreement is terminated under certain circumstances, we may be obligated to pay Purchaser a termination fee of $25.4 million. As a consequence of the failure of the Merger Transaction to be completed, as well as of some or all of these potential effects of the termination of the Merger Agreement, our business could be materially and adversely affected.

The fact that there is a merger pending could have an adverse effect on our business and results of operations.

While the Merger Transaction is pending, it creates uncertainty about our future. We are subject to a number of risks that may adversely affect our business and results of operations, including:

- the diversion of management and employee attention may detract from our ability to obtain regulatory approval for and, if approved, to successfully commercialize ELZONRIS in the European Union;
- continuing to incur significant expenses related to the Merger Transaction;
- the Merger Agreement restricting us from engaging in advantageous business activities outside of our ordinary course of business without Purchaser's consent; and
- being unable to respond effectively to competitive pressures, industry developments and future opportunities.

If the Merger Transaction occurs, our shareholders will not be able to participate in any upside to our business other than through the CVRs; if the required approval and commercialization milestone under the CVRs is not achieved, shareholders may not realize any value from the CVRs.

If the Merger Transaction occurs, our stockholders will receive one CVR per Share, which will represent the right to receive the Milestone Payment upon the first sale of ELZONRIS by or on behalf of Stemline for use or consumption by the general public for the treatment of adult patients with BPDCN in the United Kingdom, France, Spain, Germany, or Italy after approval by the European Commission of a marketing authorization application in the European Union through the centralized procedure. However, if such Milestone is not achieved on or on prior to December 31, 2021, the Milestone Payment will not be payable to stockholders and they will not receive any consideration for the CVRs they hold. Even if our business following the merger performs well, our current shareholders will not receive any additional consideration or be able to share in the increased value of our business by virtue of being equity owners.

In addition, following the Merger Transaction, our stockholders will no longer hold Shares in Stemline and they will not receive any equity of Purchaser or any entity within the Menarini Group. Consequently, our current stockholders will not be able to realize any increase in value of our business by virtue of being equity owners.

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
2.1	Agreement and Plan of Merger, dated May 4, 2020, among Stemline Therapeutics, Inc., Berlin-Chemie AG and Mercury Merger Sub, Inc., filed as Exhibit 2.1 to Form 8-K on May 4, 2020 (File No. 001-35619) and incorporated herein by reference.
3.1	Amendment to Stemline's Amended and Restated Bylaws, dated May 4, 2020, filed as Exhibit 3.1 to Form 8-K on May 4, 2020 (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 11, 2020.
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 11, 2020.
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 11, 2020.
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 11, 2020.
101	Interactive data files pursuant to Rule 405 of Regulation S-T: (i) Condensed Consolidated Balance Sheets, (ii) Condensed Consolidated Statements of Operations, (iii) Condensed Consolidated Statements of Comprehensive Loss, (iv) Condensed Consolidated Statements of Stockholders' Equity, (v) Condensed Consolidated Statements of Cash Flows, and (vi) the Notes to the Unaudited Condensed Consolidated 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 11, 2020

STEMLINE THERAPEUTICS, INC.

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

Date: May 11, 2020

By: /s/ David G. Gionco
David G. Gionco
Senior 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 11, 2020

/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 11, 2020

/s/ David G. Gionco
David G. Gionco
Senior Vice President of Finance and
Chief 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, 2020 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 11, 2020

/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, 2020 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 11, 2020

/s/ David G. Gionco
David G. Gionco
Senior Vice President of Finance and
Chief Accounting Officer