UNITED STATES
SECURITIES AND EXCHANGE COMMISSION
Washington, D.C. 20549

Form 10-Q

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

For the quarterly period ended September 30, 2020

OR

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

For the transition period from _____ to _____

Commission File Number: 001-31326

ELOXX PHARMACEUTICALS, INC.

(Exact name of registrant as specified in its charter)

Delaware	**84-1368850**
(State or other jurisdiction of incorporation or organization)	**(I.R.S. Employer Identification Number)**

950 Winter Street
Waltham, Massachusetts 02451
(Address of principal executive offices) (Zip Code)

781-577-5300
(Registrant's telephone number, including area code)

Securities registered pursuant to Section 12(b) of the Securities Exchange Act of 1934:

Title of each class	Trading Symbol(s)	Name of each exchange on which registered
Common Stock, $0.01 par value per share	ELOX	The Nasdaq Global 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 ☐

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

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

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

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

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

On November 2, 2020, the registrant had 40,152,883 shares of common stock, $0.01 par value per share, outstanding.

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Special Note Regarding Forward-Looking Statements

Eloxx Pharmaceuticals, Inc., together with its subsidiaries, is collectively referred to herein as "we," "our," "us," "Eloxx" or the "Company". *Hyperlinks and web addresses are provided as a convenience and for informational purposes only. Eloxx bears no responsibility for the security or content of external websites.*

This Quarterly Report on Form 10-Q, or this Report, and the other documents we have filed with the U.S. Securities and Exchange Commission that are incorporated herein by reference, contain forward-looking statements that involve substantial risks and uncertainties. All statements, other than statements of historical facts, including statements regarding our strategy, future operations, future financial position, future revenues, projected costs, prospects, plans and objectives of management, are forward-looking statements. The words "anticipate," "believe," "estimate," "expect," "intend," "may," "plan," "predict," "project," "target," "potential," "will," "would," "could," "should," "continue," and similar expressions are intended to identify forward-looking statements, although not all forward-looking statements contain these identifying words. We may not actually achieve the plans, intentions or expectations disclosed in our forward-looking statements, and you should not place undue reliance on our forward-looking statements. Actual results or events could differ materially from the plans, intentions and expectations disclosed in the forward-looking statements we make. In particular, you should consider the numerous risks described in the "Risk Factors" section in this Report.

Although we believe the expectations reflected in the forward-looking statements are reasonable, we cannot guarantee future results, level of activity, performance or achievements. You should not rely upon forward-looking statements as predictions of future events. Unless required by law, we will not undertake and we specifically disclaim any obligation to release publicly the result of any revisions which may be made to any forward-looking statements to reflect events or circumstances after the date of such statements or to reflect the occurrence of events, whether or not anticipated. In that respect, we wish to caution readers not to place undue reliance on any such forward-looking statements, which speak only as of the date they are made.

This Report and the other documents incorporated herein by reference include 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 and disclaim responsibility for its content.

The following are some risks and uncertainties, among others, that could cause actual results to differ materially from those expressed or implied by forward-looking statements in this Report:

- risks related to our ability to progress any product candidates in preclinical or clinical trials;
- the uncertainty of clinical trial results and the fact that positive results from preclinical studies are not always indicative of positive clinical results;
- risks related to the scope, rate and progress of our preclinical studies and clinical trials and other research and development activities;
- risks related to the competition for patient enrollment from drug candidates in development;
- the impact of the recent global outbreak of COVID-19 on our clinical trials, operations, vendors, suppliers and employees;
- risks related to our ability to obtain the capital necessary to fund our operations;
- risks relating to the cost of filing, prosecuting, defending and enforcing any patent claims and other intellectual property rights;
- risks related to our ability to obtain adequate financing in the future through product licensing, public or private equity or debt financing or otherwise;
- general business conditions, regulatory environment, competition and market for our products;
- business abilities and judgment of personnel, and the availability of qualified personnel; and
- risks related to the potentially significant, unexpected costs and liabilities arising with respect to the historic Sevion business and operations.

Item 1. Condensed Consolidated Financial Information

ELOXX PHARMACEUTICALS, INC. AND SUBSIDIARIES

UNAUDITED CONDENSED CONSOLIDATED BALANCE SHEETS
(in thousands, except share and per share data)

	September 30, 2020	December 31, 2019
ASSETS		
Current assets:		
Cash and cash equivalents	$ 30,592	$ 22,493
Marketable securities	—	33,783
Restricted cash	52	43
Prepaid expenses and other current assets	1,568	1,390
Total current assets	32,212	57,709
Property and equipment, net	149	201
Operating lease right-of-use asset	551	924
Other long-term assets	30	113
Total assets	$ 32,942	$ 58,947
LIABILITIES AND STOCKHOLDERS' EQUITY		
Current liabilities:		
Accounts payable	$ 638	$ 1,871
Accrued expenses	3,091	4,655
Current portion of long-term debt	4,917	4,336
Advances from collaboration partners	805	403
Current portion of operating lease liability	496	499
Taxes payable	38	43
Total current liabilities	9,985	11,807
Long-term debt	7,823	10,502
Operating lease liability	56	425
Total liabilities	17,864	22,734
Commitments and contingencies		
Stockholders' equity:		
Preferred stock, $0.01 par value per share, 5,000,000 shares authorized, no shares issued or outstanding as of September 30, 2020 and December 31, 2019	—	—
Common stock, $0.01 par value per share, 500,000,000 shares authorized, 40,343,181 and 40,186,469 shares issued, and 40,150,530 and 40,030,763 shares outstanding as of September 30, 2020 and December 31, 2019, respectively	403	402
Common stock in treasury, at cost, 192,651 and 155,706 shares as of September 30, 2020 and December 31, 2019, respectively	(1,825)	(1,703)
Additional paid-in capital	181,969	174,515
Accumulated other comprehensive income	—	18
Accumulated deficit	(165,469)	(137,019)
Total stockholders' equity	15,078	36,213
Total liabilities and stockholders' equity	$ 32,942	$ 58,947

See accompanying notes to unaudited condensed consolidated financial statements

ELOXX PHARMACEUTICALS, INC. AND SUBSIDIARIES

UNAUDITED CONDENSED CONSOLIDATED STATEMENTS OF OPERATIONS AND COMPREHENSIVE LOSS
(in thousands, except share and per share data)

	Three Months Ended September 30,		Nine Months Ended September 30,	
	2020	2019	2020	2019
Operating expenses:				
Research and development	$ 3,231	$ 6,801	$ 11,308	$ 20,160
General and administrative	3,065	5,978	12,347	18,907
Restructuring charges	—	—	3,994	—
Total operating expenses	6,296	12,779	27,649	39,067
Loss from operations	(6,296)	(12,779)	(27,649)	(39,067)
Other expense, net	321	96	801	174
Net loss	$ (6,617)	$ (12,875)	$ (28,450)	$ (39,241)
Net loss per share, basic and diluted	$ (0.16)	$ (0.32)	$ (0.71)	$ (1.05)
Weighted average number of shares of common stock used in computing net loss per share, basic and diluted	40,142,178	39,944,324	40,115,351	37,394,310
Comprehensive loss:				
Net loss	$ (6,617)	$ (12,875)	$ (28,450)	$ (39,241)
Other comprehensive income (loss):				
Change in unrealized gain (loss) on available-for-sale securities	(13)	(12)	(18)	12
Comprehensive loss	$ (6,630)	$ (12,887)	$ (28,468)	$ (39,229)

See accompanying notes to unaudited condensed consolidated financial statements

ELOXX PHARMACEUTICALS, INC. AND SUBSIDIARIES

UNAUDITED CONDENSED CONSOLIDATED STATEMENTS OF CASH FLOWS
(in thousands)

	Nine Months Ended September 30,	
	2020	**2019**
Cash flows from operating activities:		
Net loss	$ (28,450)	$ (39,241)
Adjustments to reconcile net loss to net cash used in operating activities:		
Stock-based compensation	7,385	8,631
Depreciation	52	68
Amortization of operating lease right-of-use asset	373	350
Amortization of debt discount	438	378
Amortization, net, of premiums and discounts on investments	15	(228)
Changes in operating assets and liabilities:		
Prepaid expenses and other assets	(137)	(150)
Advances from collaboration partners	—	403
Accounts payable	(1,233)	940
Accrued expenses	(1,564)	(856)
Operating lease liabilities	(372)	(350)
Taxes payable	(5)	(79)
Net cash used in operating activities	(23,498)	(30,134)
Cash flows from investing activities:		
Purchases of marketable securities	—	(56,041)
Proceeds from maturities of marketable securities	33,750	13,500
Purchase of property and equipment	—	(40)
Cash received (paid) for long-term deposits	42	(22)
Net cash provided by (used in) investing activities	33,792	(42,603)
Cash flows from financing activities:		
Proceeds from underwritten public offering, net of issuance costs	—	32,169
Proceeds from debt financing obligation	797	15,000
Payment of debt issuance costs	—	(276)
Repayment of term loan principal	(3,333)	—
Proceeds from exercises of stock options	70	148
Proceeds from sale of common stock under at-the-market sales agreement	—	455
Payment for settlement of taxes upon vesting of restricted stock units	(122)	(1,215)
Proceeds from advances from collaboration partners	402	—
Net cash (used in) provided by financing activities	(2,186)	46,281
Increase (decrease) in cash, cash equivalents and restricted cash	8,108	(26,456)
Cash, cash equivalents and restricted cash at the beginning of the period	22,536	48,651
Cash, cash equivalents and restricted cash at the end of the period	$ 30,644	$ 22,195
Reconciliation of cash, cash equivalents and restricted cash to condensed consolidated balance sheets:		
Cash and cash equivalents	$ 30,592	$ 22,155
Restricted cash	52	40
Total cash, cash equivalents and restricted cash	$ 30,644	$ 22,195
Supplemental disclosure of cash flow activities:		
Cash paid for interest	$ 665	$ 703
Cash paid for income taxes	$ 5	$ 79
Supplemental disclosure of non-cash financing activities:		
Non-cash acquisition of treasury stock	$ —	$ 46
Non-cash issuance of common stock upon exercise of warrants	$ —	$ 178
Fair value of warrants issued in connection with long-term debt	$ —	$ 421

See accompanying notes to unaudited condensed consolidated financial statements

ELOXX PHARMACEUTICALS, INC. AND SUBSIDIARIES

UNAUDITED CONDENSED CONSOLIDATED STATEMENTS OF STOCKHOLDERS' EQUITY
(in thousands, except share data)

	Common stock				Treasury stock			
	Shares	Amount	Additional paid-in capital	Accumulated other comprehensive income	Shares	Amount	Accumulated deficit	Total stockholders' equity
Balance at December 31, 2019	40,030,763	$ 402	$ 174,515	$ 18	(155,706)	$ (1,703)	$ (137,019)	$ 36,213
Exercise of stock options	10,636	—	64	—	—	—	—	64
Vesting of restricted stock units	84,055	1	(1)	—	(34,874)	(116)	—	(116)
Stock-based compensation expense	—	—	3,995	—	—	—	—	3,995
Change in unrealized gain (loss) on investments	—	—	—	47	—	—	—	47
Net loss	—	—	—	—	—	—	(13,946)	(13,946)
Balance at March 31, 2020	40,125,454	$ 403	$ 178,573	$ 65	(190,580)	$ (1,819)	$ (150,965)	$ 26,257
Vesting of restricted stock units	9,836	—	—	—	(1,036)	(3)	—	(3)
Stock-based compensation expense	—	—	1,976	—	—	—	—	1,976
Change in unrealized gain (loss) on investments	—	—	—	(52)	—	—	—	(52)
Net loss	—	—	—	—	—	—	(7,887)	(7,887)
Balance at June 30, 2020	40,135,290	$ 403	$ 180,549	$ 13	(191,616)	$ (1,822)	$ (158,852)	$ 20,291
Exercise of stock options	5,401	—	5	—	—	—	—	5
Vesting of restricted stock units	9,839	—	—	—	(1,035)	(3)	—	(3)
Stock-based compensation expense	—	—	1,415	—	—	—	—	1,415
Change in unrealized gain (loss) on investments	—	—	—	(13)	—	—	—	(13)
Net loss	—	—	—	—	—	—	(6,617)	(6,617)
Balance at September 30, 2020	40,150,530	$ 403	$ 181,969	$ —	(192,651)	$ (1,825)	$ (165,469)	$ 15,078

ELOXX PHARMACEUTICALS, INC. AND SUBSIDIARIES

UNAUDITED CONDENSED CONSOLIDATED STATEMENTS OF STOCKHOLDERS' EQUITY
(in thousands, except share data)

	Common stock				Treasury stock			
	Shares	Amount	Additional paid-in capital	Accumulated other comprehensive income	Shares	Amount	Accumulated deficit	Total stockholders' equity
Balance at December 31, 2018	35,860,114	$ 360	$ 129,825	$ —	(91,423)	$ (1,129)	$ (86,145)	$ 42,911
Exercise of stock options	25,000	—	25	—	—	—	—	25
Issuance of common stock under at-the-market sales agreement	35,362	1	454	—	—	—	—	455
Issuance of warrants	—	—	421	—	—	—	—	421
Vesting of restricted stock units	25,132	—	—	—	(10,467)	(121)	—	(121)
Stock-based compensation expense	—	—	2,658	—	—	—	—	2,658
Change in unrealized gain (loss) on investments	—	—	—	1	—	—	—	1
Net loss	—	—	—	—	—	—	(11,917)	(11,917)
Balance at March 31, 2019	35,945,608	$ 361	$ 133,383	$ 1	(101,890)	$ (1,250)	$ (98,062)	$ 34,433
Exercise of stock options	36,790	—	90	—	—	—	—	90
Issuance of common stock upon exercise of warrants	44,814	—	178	—	(14,893)	(178)	—	—
Vesting of restricted stock units	54,122	3	(3)	—	(11,914)	(111)	—	(111)
Issuance of common stock upon public offering	3,833,334	38	32,184	—	—	—	—	32,222
Stock-based compensation expense	—	—	3,016	—	—	—	—	3,016
Change in unrealized gain (loss) on investments	—	—	—	23	—	—	—	23
Net loss	—	—	—	—	—	—	(14,449)	(14,449)
Balance at June 30, 2019	39,914,668	$ 402	$ 168,848	$ 24	(128,697)	$ (1,539)	$ (112,511)	$ 55,224
Exercise of stock options	29,537	—	28	—	—	—	—	28
Vesting of restricted stock units	33,449	2	—	—	(10,835)	(46)	—	(44)
Equity financing issuance costs	—	—	(50)	—	—	—	—	(50)
Stock-based compensation expense	—	—	2,957	—	—	—	—	2,957
Change in unrealized gain (loss) on investments	—	—	—	(12)	—	—	—	(12)
Net loss	—	—	—	—	—	—	(12,875)	(12,875)
Balance at September 30, 2019	39,977,654	$ 404	$ 171,783	$ 12	(139,532)	$ (1,585)	$ (125,386)	$ 45,228

See accompanying notes to unaudited condensed consolidated financial statements

1. Nature of the Business

Eloxx Pharmaceuticals, Inc., together with its subsidiaries (collectively "Eloxx" or the "Company"), is a clinical-stage biopharmaceutical company developing novel ribonucleic acid (RNA)-modulating drug candidates, each designed to be a eukaryotic ribosomal selective glycoside (ERSG), formulated to treat rare and ultra-rare premature stop codon diseases. Premature stop codons are point mutations that disrupt the stability of the impacted messenger RNA (mRNA) and the protein synthesis from that mRNA. As a consequence, patients with premature stop codon diseases have reduced levels of, or no, protein from a gene whose product performs an essential function. This type of mutation accounts for some of the most severe phenotypes across genetic diseases. Nonsense mutations have been identified in over 1,800 rare and ultra-rare diseases. Read-through therapeutic development is focused on increasing functional protein synthesis by enabling the cytoplasmic ribosome to read through premature stop codons to produce full-length proteins. As opposed to a typical gene therapy approach of targeting a single, unique mutation in a target disease, this small molecule strategy enables targeting an entire class of mutations across the rare disease landscape. The small molecule approach has the potential to address a range of different premature stop codons in a single gene since the ERSG compounds are targeted to the ribosomes. ELX-02, the Company's lead investigational drug product candidate, is a small molecule designed to restore production of full-length functional proteins. ELX-02 is in clinical development for systemic administration for cystic fibrosis. ELX-02 is an investigational drug that has not been approved by any global regulatory body. The Company is also conducting investigational new drug (IND) enabling preclinical studies of ERSG compounds for autosomal dominant polycystic kidney disease (ADPKD) and in rare inherited retinal disorders (IRDs) by intravitreal administration with an initial focus on Usher Syndrome.

The Company's preclinical candidate pool consists of a library of novel ERSG drug candidates identified based on read-through potential and cytoplasmic ribosomal selectivity. The Company is headquartered in Waltham, Massachusetts, with additional offices in Morristown, New Jersey and Rehovot, Israel.

The Company's research and development strategy is to target rare or ultra-rare diseases where a high unmet medical need exists, a nonsense mutation-bearing patient population is established, preclinical read-through can be established in predictive personalized medicine models, and a defined path through Orphan Drug development, regulatory approval, patient access and commercialization is identified. The Company believes patient advocacy is an important element of patient focused drug development and seeks opportunities to collaborate with patient advocacy groups throughout the discovery and development process. The Company's current clinical program for its lead investigational drug product candidate, ELX-02, includes Phase 2 studies in cystic fibrosis in Europe, Israel and the United States. On March 25, 2020, the Company announced that enrollment in its Phase 2 clinical trials for ELX-02 in cystic fibrosis had been temporarily paused in response to the COVID-19 pandemic in order to avoid unnecessary exposure in at-risk populations, to maintain the integrity of study data and to support global healthcare providers in their commitment to ensure patient safety. On June 17, 2020, the Company announced that enrollment in its Phase 2 clinical trial in cystic fibrosis had been resumed in Israel and Europe, and on August 12, 2020, the Company announced that enrollment in its Phase 2 clinical trial in cystic fibrosis had been resumed in the U.S.

The extent and severity of the impact of the current global health crisis on the Company's business and clinical trials will be determined largely by the ability of patients and prospective patients in its clinical trials to access trial sites, the ability of personnel from clinical research organizations ("CROs") to oversee the administration of the Company's drug in accordance with trial protocols and the Company's ability to monitor and communicate effectively with CROs, staff at clinical trial sites and principal investigators. In addition, the impact of the COVID-19 pandemic on the operations of the U.S. Food and Drug Administration (the "FDA") and other health authorities may delay potential advancement of the Company's product candidates. The Company cannot reasonably estimate the extent to which these potential disruptions may materially impact its consolidated results of operations or financial position.

During 2019, the Company announced that the Cystic Fibrosis Foundation (the "CF Foundation") is providing funding for a portion of the U.S. Phase 2 cystic fibrosis clinical trial. The Company has since formed a joint program advisory group with the CF Foundation focused on the development of ELX-02 for cystic fibrosis. The Cystic Fibrosis Therapeutics Development Network ("TDN") has sanctioned the Phase 2 study protocol, which is being conducted at TDN member sites. On August 4, 2020, the Company announced that the FDA granted orphan drug designation for ELX-02 for the treatment of cystic fibrosis. The FDA's Office of Orphan Drug Products grants orphan status to support the development of medicines for underserved patient populations, or rare disorders, that affect fewer than 200,000 people in the U.S. Orphan drug designation qualifies Eloxx for certain benefits, including seven years of market exclusivity upon regulatory approval (if received), exemption from FDA application fees, tax credits on qualified U.S. clinical trials and eligibility for grant funding opportunities that can be used for clinical trial costs. ELX-02 had previously been granted orphan medicinal product designation for the treatment of cystic fibrosis by the European Medicines Agency.

Liquidity

The Company has a history of net losses and negative cash flows from operating activities since inception, and as of September 30, 2020, had an accumulated deficit of $(165.5) million. The Company expects to continue to incur net losses and use cash in its operations for the foreseeable future. To date, the Company has not generated revenue from the sale of any product or service and does not expect to generate significant revenue unless and until it obtains marketing approval for and commercializes one or more of its product candidates currently in development. Successful transition to profitable operations is dependent upon achieving a level of revenue adequate to support the Company's cost structure.

The Company has financed its operations primarily from the sale of equity securities and to a lesser extent, loans and grants. The Company may never achieve profitability, and unless and until it does, the Company will continue to need to raise additional capital to fund its operations. As discussed in Note 14, on February 24, 2020, the Company's Board of Directors approved a leadership and organizational realignment, aimed at supporting the Company's efforts to improve operating performance, and concentrate development efforts on the Company's core programs. The Company believes that its cash and cash equivalents of $30.6 million at September 30, 2020 will enable it to meet anticipated cash needs required to maintain its current and planned operations through at least the next 12 months from the issuance of the financial statements for the quarter ended September 30, 2020. Management intends to fund future operations through private or public debt or equity financing transactions and may seek additional capital through arrangements with strategic partners or from other sources. If the Company is unable to obtain adequate financing, it will evaluate options which may include reducing or deferring operating expenses, which may have a material adverse effect on the Company's operations and future prospects.

2. Basis of Presentation and Significant Accounting Policies

The Company has prepared the accompanying unaudited interim condensed consolidated financial statements in accordance with accounting principles generally accepted in the United States of America ("U.S. GAAP") as found in the Accounting Standards Codification ("ASC") and Accounting Standards Updates ("ASUs") promulgated by the Financial Accounting Standards Board ("FASB"). The Company has reclassified certain items from the prior year's condensed consolidated financial statements to conform to the current year's presentation. Certain information and footnote disclosures normally included in the Company's annual financial statements have been condensed or omitted, as permitted by such rules and regulations. These interim financial statements, in the opinion of management, reflect all normal recurring adjustments necessary for a fair presentation of the Company's financial position, results of operations, and cash flows for the interim periods ended September 30, 2020 and 2019.

The results of operations for the interim periods are not necessarily indicative of the results of operations to be expected for the full year. These interim financial statements should be read in conjunction with the audited financial statements as of and for the year ended December 31, 2019, and the notes thereto, which are included in the Company's 2019 Annual Report on Form 10-K filed with the U.S. Securities and Exchange Commission (the "SEC") on March 6, 2020.

The significant accounting policies used in the preparation of these condensed consolidated financial statements are consistent with those described in the Company's audited financial statements as of and for the year ended December 31, 2019, and the notes thereto, in the Company's 2019 Annual Report on Form 10-K, except as follows. Due to its adoption of ASU No. 2016-13 (further described below under the heading *"Recent Accounting Pronouncements – Adopted"*) on January 1, 2020, the Company has updated its accounting policy for marketable securities.

Marketable Securities

The Company classifies all investment instruments with an original maturity date, when purchased, in excess of three months but less than one year as current marketable securities. Marketable securities are classified as available-for-sale and are carried at fair value. The Company periodically assesses its portfolio of securities to determine whether to record any estimated allowances for credit losses in the statement of operations. This assessment includes considering whether the Company intends to sell a security, whether it is more likely than not that the Company will have to sell a security before recovery of its amortized cost, and whether a decline in a security's fair value below its amortized cost is credit-related or non-credit-related. The Company records non-credit-related unrealized gains and losses on available-for-sale securities in accumulated other comprehensive income, which is a separate component of stockholders' equity on its consolidated balance sheet. Gains or losses realized upon sales of available-for-sale securities are recorded in other income. The cost of securities sold is based on the specific identification method. To date, the Company has recorded no allowances for credit losses, and no realized gains or losses upon sales of securities.

Recent Accounting Pronouncements – Adopted

In June 2016, the FASB issued ASU No. 2016-13, *Measurement of Credit Losses on Financial Instruments*. This standard requires that for most financial assets, losses must be based on an expected loss approach which includes estimates of losses over the life of exposure that considers historical, current and forecasted information. Expanded disclosures related to the methods used to estimate the losses as well as a specific disaggregation of balances for financial assets are also required. The Company adopted the new standard on January 1, 2020 and adoption did not have a material impact on the consolidated financial statements.

In August 2018, the FASB issued ASU No. 2018-13, *Fair Value Measurement (Topic 820): Disclosure Framework-Changes to the Disclosure Requirements for Fair Value Measurement*, which modifies the disclosure requirements for certain fair value measurements. The Company adopted the new standard on January 1, 2020 and adoption did not have a material impact on the consolidated financial statements.

3. Prepaid Expenses and Other Current Assets

Prepaid expenses and other current assets as of September 30, 2020 and December 31, 2019 consisted of the following (in thousands):

	September 30, 2020	December 31, 2019
Research and development	$ 743	$ 448
Other	534	699
Insurance	256	217
VAT receivables	35	26
Total	$ 1,568	$ 1,390

4. Property and Equipment

Property and equipment, net as of September 30, 2020 and December 31, 2019 consisted of the following (in thousands):

	September 30, 2020	December 31, 2019
Computers and software	$ 124	$ 166
Office furniture and equipment	164	164
Leasehold improvements	158	158
	446	488
Less: Accumulated depreciation	(297)	(287)
Property and equipment, net	$ 149	$ 201

Depreciation expense was $16 thousand and $18 thousand for the three months ended September 30, 2020 and 2019 and $52 thousand and $68 thousand for the nine months ended September 30, 2020 and 2019, respectively.

5. Accrued Expenses

Accrued expenses as of September 30, 2020 and December 31, 2019 consisted of the following (in thousands):

	September 30, 2020	December 31, 2019
Research and development expenses	$ 990	$ 1,560
Payroll, bonus and other employee-related expenses	1,028	2,200
Restructuring	577	—
Professional services	414	664
Other	22	137
Interest on debt	60	94
Total	$ 3,091	$ 4,655

6. Debt

Term Loan

On January 30, 2019, the Company entered into a Loan and Security Agreement (the "SVB Loan Agreement") in the amount of $15.0 million with Silicon Valley Bank ("SVB") and WestRiver Innovation Lending Fund VIII, L.P. ("WestRiver", and together with SVB, the "Lenders").

Outstanding principal on the loan accrues interest at a floating rate equal to the greater of (i) 5.25% per annum and (ii) the sum of 2.5% plus the prime rate, as published in the Wall Street Journal. Interest payments are payable monthly following the funding of the loan. On September 30, 2020, the interest rate was 5.75%. The Company commenced making payments on the outstanding principal balance of the loan on February 1, 2020, which is payable in 36 equal monthly installments. Amounts outstanding under the loan are due and payable on January 1, 2023.

In conjunction with the initial loan advance, the Company issued warrants (the "Warrants") to SVB and WestRiver to purchase an aggregate of 40,834 shares of the Company's common stock at a warrant exercise price of $11.02 (subject to certain adjustments), which price was calculated using the 10-day average bid price of the Company's common stock prior to the date of the SVB Loan Agreement.

The Company may prepay the outstanding principal balance of the loans advanced by SVB in whole but not in part, subject to a prepayment fee ranging from 1% to 3% of any amount prepaid, depending upon when the prepayment occurs. The Company will also pay a final payment fee equal to 6% of the total loans advanced, due upon the earlier of maturity or termination of the SVB Loan Agreement.

Under the terms of the SVB Loan Agreement, the Company granted first priority liens and security interests in substantially all of the Company's assets (excluding all of its intellectual property, which is subject to a negative pledge) and a pledge by the Company of the shares of one of its wholly-owned subsidiaries as collateral for the obligations thereunder. The SVB Loan Agreement also contains representations and warranties by the Company and SVB and indemnification provisions in favor of SVB and customary covenants (including limitations on other indebtedness, liens, acquisitions, investments and dividends, but no financial covenants), and events of default (including payment defaults, breaches of covenants following any applicable cure period, a material impairment in the perfection or priority of SVB's security interest in the collateral, and events relating to bankruptcy or insolvency).

As of September 30, 2020, the carrying value of the outstanding loan consists of $11.7 million in principal less the unamortized debt discount of approximately $0.6 million. The debt issuance costs, the valuation of the Warrants, and the final maturity payment of $0.9 million, have been recorded as a debt discount which are being accreted to interest expense through the maturity date of the loan. Interest expense relating to the loan for the three months ended September 30, 2020 and 2019 was $0.4 million and $0.4 million, respectively, and for the nine months ended September 30, 2020 and 2019 was $1.1 million and $1.2 million, respectively. Interest expense is calculated using the effective interest method and is inclusive of non-cash amortization of the debt discount. At September 30, 2020, the effective interest rate was 10.85%.

PPP Loan

In April 2020, the Company entered into a loan agreement with SVB under the U.S. Small Business Administration (the "SBA") Paycheck Protection Program (the "PPP") pursuant to the Coronavirus Aid, Relief and Economic Security Act of 2020 (the "CARES Act") and received loan proceeds of $0.8 million (the "PPP Loan"). The Company expects to use the loan proceeds for payroll and other covered costs in accordance with the relevant terms and conditions of the CARES Act. The Company issued a promissory note for the PPP Loan with a maturity date of April 21, 2022 and an interest rate of 1.0% per annum. Monthly payments of principal and interest will be due beginning on September 21, 2021, although interest accrues from the issuance date. The Company may prepay the PPP Loan without penalty or premium, and the promissory note provides for customary events of default. A PPP loan may be partially or entirely forgiven based on employee retention for the 24-week period starting on the loan date through October 2020, and the use of loan proceeds for payroll or other specified costs during the same period. Forgiveness is also based on the employer maintaining or restoring headcount and maintaining salary levels. Forgiveness is reduced if headcount declines or if salaries decrease. Any loan forgiveness will be made subject to SVB approval in accordance with SBA requirements.

The Company's scheduled future principal payments for the long-term debt are as follows (in thousands):

	September 30, 2020
Remainder of 2020	$ 1,250
2021	5,398
2022	5,398
2023	417
Total future principal payments	12,463
Less: unamortized discount	(623)
Carrying value of long-term debt	11,840
Less: current portion	(4,917)
Add: Final fee due at maturity in 2023	900
Long-term portion	$ 7,823

7. Legal and Other Contingencies

From time to time, the Company may become involved in various lawsuits and legal proceedings, which arise in the ordinary course of business. The Company is currently unaware of any material pending legal proceedings to which it is a party or of which its property is the subject. However, the Company may at times in the future become involved in litigation in the ordinary course of business, which may include actions related to or based on its intellectual property and its use, customer claims, product liability claims, employment practices and employee complaints and other events arising out of its operations. When appropriate in management's estimation, the Company will record adequate reserves in its financial statements for pending litigation. Litigation is subject to inherent uncertainties, and an adverse result in any such matters could adversely impact its reputation, operations, and its financial operating results or overall financial condition. Additionally, any litigation to which the Company may become subject could also require significant involvement of its senior management and may divert management's attention from the Company's business and operations.

The Company accounts for its contingent liabilities in accordance with ASC Topic 450, "Contingencies". A provision is recorded when it is both probable that a liability has been incurred and the amount of the loss can be reasonably estimated. With respect to legal matters, provisions are reviewed and adjusted to reflect the impact of negotiations, estimated settlements, legal rulings, advice of legal counsel and other information and events pertaining to a particular matter. For the periods ended September 30, 2020 and 2019, the Company was not a party to any litigation that is reasonably possible to have a material adverse effect on the Company's business, financial position, results of operations or cash flows. Legal costs incurred in connection with loss contingencies are expensed as incurred.

8. Stockholders' Equity

Warrants

In connection with the January 2019 issuance of debt, the Company granted warrants to purchase 40,834 shares of common stock and recorded a charge in additional paid-in-capital in the amount of $0.4 million reflecting the fair value of the warrants on the date of issuance.

Transactions related to warrants to purchase the Company's common stock during the nine months ended September 30, 2020, were as follows:

	Shares	Weighted average exercise price	Weighted average remaining contractual life (years)
Warrants outstanding at December 31, 2019	323,894	$ 4.32	3.74
Granted	—		
Exercised	—		
Forfeited	—		
Warrants outstanding at September 30, 2020	323,894	$ 4.32	2.99
Warrants exercisable at September 30, 2020	323,894	$ 4.32	2.99

9. Stock-Based Compensation

Summary of Option Activity

Transactions related to stock options awarded to employees and directors during the nine months ended September 30, 2020 were as follows:

	Shares	Weighted average exercise price	Weighted average remaining contractual life (years)	Aggregate intrinsic value
Options outstanding at December 31, 2019	4,737,670	$ 11.17	8.54	$ 3,629,073
Granted	1,400,010	3.52		
Exercised	(16,037)	4.31		
Forfeited	(1,166,026)	10.03		
Options outstanding at September 30, 2020	4,955,617	$ 10.05	6.78	$ 723,236
Options exercisable at September 30, 2020	2,672,234	$ 12.44	4.78	$ 689,501

The aggregate intrinsic value represents the total intrinsic value (the difference between the fair value of the Company's common stock as of September 30, 2020 and the exercise price, multiplied by the number of in-the-money options) that would have been received by the option holders had all option holders exercised their options on September 30, 2020. This amount is impacted by changes in the fair value of the Company's common stock.

Summary of Restricted Stock Unit Activity

Transactions related to restricted stock units awarded to employees during the nine months ended September 30, 2020 were as follows:

	Shares	Weighted average grant date fair value per share
Unvested at December 31, 2019	463,945	$ 9.45
Granted	300,000	3.59
Vested	(140,675)	10.19
Forfeited	(266,977)	8.65
Unvested at September 30, 2020	356,293	$ 5.06

Stock-based Compensation Expense

Stock-based compensation relates to stock options granted to employees, non-employee directors and non-employees, time-based restricted stock units granted to employees and performance-based stock options and restricted stock units granted to an employee. On February 24, 2020, the Company's Board of Directors approved a leadership and organizational realignment, which accelerated the vesting of certain awards, resulting in additional stock-based compensation of $2.1 million recorded in restructuring charges. The total equity-based compensation expense related to all of the Company's equity-based awards was recognized as follows (in thousands):

	Three Months Ended September 30,		Nine Months Ended September 30,	
	2020	2019	2020	2019
Research and development	$ 285	$ 718	$ 766	$ 1,987
General and administrative	1,129	2,239	4,495	6,644
Restructuring charges	—	—	2,124	—
Total stock-based compensation expense	$ 1,414	$ 2,957	$ 7,385	$ 8,631

10. Marketable Securities

Below is a summary of cash, cash equivalents and marketable securities at September 30, 2020 (in thousands):

	Amortized Cost	Unrealized Gains	Unrealized Losses	Fair Value
Cash and cash equivalents	$ 30,592	$ —	$ —	$ 30,592
Marketable securities - U.S. treasuries	—	—	—	—
Total cash, cash equivalents and marketable securities	$ 30,592	$ —	$ —	$ 30,592

Below is a summary of cash, cash equivalents and marketable securities at December 31, 2019 (in thousands):

	Amortized Cost	Unrealized Gains	Unrealized Losses	Fair Value
Cash and cash equivalents	$ 22,493	$ —	$ —	$ 22,493
Marketable securities - U.S. treasuries	33,765	19	(1)	33,783
Total cash, cash equivalents and marketable securities	$ 56,258	$ 19	$ (1)	$ 56,276

As of September 30, 2020 and December 31, 2019, no credit losses were identified related to the cash equivalents or marketable securities.

11. Fair Value of Financial Instruments

At September 30, 2020 and December 31, 2019, the Company's financial assets valued based on Level 1 inputs consisted of cash, cash equivalents and marketable securities (U.S. treasuries). During the three and nine months ended September 30, 2020, the Company did not have any transfers of financial assets between Level 2 and 3.

Some assets and liabilities are required to be recorded at fair value on a recurring basis, while other assets and liabilities are recorded at fair value on a nonrecurring basis. The carrying amounts of current financial instruments, which include accounts payable, accrued expenses, lease obligation liability and debt, approximate their fair values due to the short-term nature of these instruments.

12. Other Expense, Net

Other expense, net consisted of the following (in thousands):

	Three Months Ended September 30,		Nine Months Ended September 30,	
	2020	2019	2020	2019
Interest and other income	$ (23)	$ (272)	$ (336)	$ (766)
Foreign currency exchange losses (gains), net	11	28	32	(3)
Investment income, net	6	(106)	15	(240)
Interest and other expense	327	446	1,090	1,183
Total other expense, net	$ 321	$ 96	$ 801	$ 174

13. Net Loss Per Share

The loss and the weighted average number of shares used in computing basic and diluted net loss per share for the periods, are as follows (amounts in thousands, except share and per share data):

	Three Months Ended September 30,		Nine Months Ended September 30,	
	2020	2019	2020	2019
Numerator:				
Net loss	$ (6,617)	$ (12,875)	$ (28,450)	$ (39,241)
Denominator:				
Weighted average number of shares of common stock used in computing net loss per share, basic and diluted	40,142,178	39,944,324	40,115,351	37,394,310
Net loss per share, basic and diluted	$ (0.16)	$ (0.32)	$ (0.71)	$ (1.05)

The following potentially dilutive securities have been excluded from the computation of diluted weighted average shares outstanding as their effect would be anti-dilutive:

	Nine Months Ended September 30,	
	2020	2019
Options to purchase common stock	4,955,617	4,797,040
Restricted stock units	356,293	508,228
Warrants	323,894	323,894
Total potential common stock equivalents	5,635,804	5,629,162

14. Restructuring

On February 24, 2020, the Company's Board of Directors approved a leadership and organizational realignment aimed at supporting its efforts to improve operating performance and concentrate development efforts on its core programs. The organizational realignment reduced managerial layers and consolidated roles across the organization, resulting in the elimination of 13 full-time positions during the first quarter of 2020. This resulted in a one-time charge of $4.0 million, including $2.1 million in stock-based compensation expense, with the severance portion being paid out over a one-year period. The accrued charges and associated payments that occurred for the nine months ended September 30, 2020, are as follows (amounts in thousands):

	Beginning Balance	Additions	Deductions	Ending Balance
Severance and related costs	$ —	$ 1,705	$ (1,128)	$ 577
Contract termination costs	—	165	(165)	—
Total restructuring charges	$ —	$ 1,870	$ (1,293)	$ 577

Item 2. Management's Discussion and Analysis of Financial Condition and Results of Operations

The following information should be read in conjunction with the consolidated financial statements and related notes thereto included in this Quarterly Report on Form 10-Q (this "Report").

Except for the historical information contained in this Report, the matters discussed herein may be deemed to be forward-looking statements that involve risks and uncertainties. We make such forward-looking statements pursuant to the safe harbor provisions of the Private Securities Litigation Reform Act of 1995 and other federal securities laws. In this Report, words such as "may," "expect," "anticipate," "estimate," "intend," and similar expressions (as well as other words or expressions referencing future events, conditions or circumstances) are intended to identify forward-looking statements.

Our actual results and the timing of certain events may differ materially from the results discussed, projected, anticipated, or indicated in any forward-looking statements. 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 Report. 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 Report, they may not be predictive of results or developments in future periods.

The following information and any forward-looking statements should be considered in light of factors discussed elsewhere in this Report, including those risks identified under Item 1A., Risk Factors. In many instances, dollar amounts contained in the narrative descriptions in the following section of this Report are stated in approximate values, pursuant to generally accepted rounding conventions. We caution readers not to place undue reliance on any forward-looking statements made by us, which speak only as of the date they are made. We disclaim any obligation, except as specifically required by law and the rules of the U.S. Securities and Exchange Commission (the "SEC"), to publicly update or revise any such statements to reflect any change in our expectations or in events, conditions or circumstances on which any such statements may be based, or that may affect the likelihood that actual results will differ from those set forth in the forward-looking statements.

Company Overview

We are a clinical-stage biopharmaceutical company developing novel ribonucleic acid (RNA)-modulating drug candidates, each designed to be a eukaryotic ribosomal selective glycoside (ERSG), formulated to treat rare and ultra-rare premature stop codon diseases. Premature stop codons are point mutations that disrupt the stability of the impacted messenger RNA (mRNA) and the protein synthesis from that mRNA. As a consequence, patients with premature stop codon diseases have reduced levels of, or no, protein from a gene whose product performs an essential function. This type of mutation accounts for some of the most severe phenotypes across genetic diseases. Nonsense mutations have been identified in over 1,800 rare and ultra-rare diseases. Read-through therapeutic development is focused on increasing functional protein synthesis by enabling the cytoplasmic ribosome to read through premature stop codons to produce full-length proteins. As opposed to a typical gene therapy approach of targeting a single, unique mutation in a target disease, this small molecule strategy enables targeting an entire class of mutations across the rare disease landscape. Our small molecule approach has the potential to address a range of different premature stop codons in a single gene since our ERSG compounds are targeted to the ribosomes. ELX-02, our lead investigational drug product candidate, is a small molecule designed to restore production of full-length functional proteins. ELX-02 is in clinical development for systemic administration for cystic fibrosis. ELX-02 is an investigational drug that has not been approved by any global regulatory body. We are also conducting IND-enabling preclinical studies of ERSG compounds for autosomal dominant polycystic kidney disease (ADPKD) and in rare inherited retinal disorders (IRDs) by intravitreal administration with an initial focus on Usher Syndrome. Our preclinical candidate pool consists of a library of novel ERSG drug candidates identified based on read-through potential and cytoplasmic ribosomal selectivity. We hold worldwide development and commercialization rights to ELX-02 and other novel compounds in our read-through library, for all indications, in all territories, under a license from the Technion Research and Development Foundation Ltd. ("TRDF").

During 2019, we advanced our clinical program for ELX-02 into Phase 2 studies in cystic fibrosis and nephropathic cystinosis. We also completed a renal impairment study with ELX-02 in subjects with mild, moderate, and severe renal impairment. The results from the renal impairment study provided support for both continuing our clinical development programs and evaluating the suitability of our ERSG library for development in additional renal diseases, including ADPKD.

Our research and development strategy targets rare or ultra-rare diseases where a high unmet medical need exists, a nonsense mutation-bearing patient population is established, preclinical read-through can be established in predictive personalized medicine models, and a defined path through Orphan Drug development, regulatory approval, patient access and commercialization is identified. We believe patient advocacy is an important element of patient focused drug development, and we seek opportunities to collaborate with patient advocacy groups throughout the discovery and development process.

Our current clinical program for our lead investigational drug product candidate, ELX-02, includes Phase 2 studies in cystic fibrosis.

We intend to be the global leader in the application of the science of translational read-through and the associated pathway of nonsense mediated decay (NMD). We believe that expanding our expertise across these basic science areas of mRNA regulation, ribosomal function, and protein translation forms a solid foundation to support our discovery and development activities. Our ERSG compounds modulate the activity of the ribosome, a ribonucleoprotein complex of RNAs and proteins responsible for protein production (a process also known as translation). These novel small molecule ERSG compounds are designed to allow the ribosome to read-through a nonsense mutation in mRNA (which is transcribed from the DNA sequence), to restore the translation process to produce full-length, functional proteins and increase the amount of mRNA that would otherwise be degraded as part of a phenomenon called nonsense mediated mRNA decay. As our ERSG compounds target the general mechanism for protein production in the cell, we believe they have the potential to treat numerous genetic diseases where nonsense mutations have impaired gene function. Since nonsense mutations may occur at different positions within a given gene, a potential advantage of the small molecule ERSG approach is being able to use one molecule to address a range of mutations within a given disease state. Our subcutaneously injected ERSG molecules have the potential to be self-administered for systemic disease and to be active across many of the body's tissues.

We believe that our library of related novel small molecules holds the potential to be disease-modifying therapies that may change the course of numerous genetic diseases and improve the lives of patients. Our early preclinical data in animal models of nonsense mutations suggests that drug product candidates from our read-through compound ERSG library may have potential beneficial effects for each of the following diseases: cystic fibrosis, nephropathic cystinosis, ADPKD, a variety of IRDs (including Usher Syndrome), primary ciliary dyskinesia, mucopolysaccharidosis type 1, Duchenne muscular dystrophy and Rett syndrome, and have demonstrated the potential for beneficial effects in multiple organs such as the brain, eye, kidney, lungs, muscles and others. Of the novel compounds in our ERSG Library, approximately 30 compounds have been selected, based on read-through activity, for continued preclinical research and we anticipate additional compounds advancing toward Investigational New Drug (IND) filings.

Our scientific manuscript titled "ELX-02 generates protein via premature stop codon read-through without inducing native stop codon read-through protein" was published in the August 2020 issue of the *Journal of Pharmacology and Experimental Therapeutics* (JPET). This manuscript demonstrates that while ELX-02 mediates read-through of premature stop codons, the fidelity of native stop codons found at the end of healthy transcripts is maintained. This indicates that translation integrity is preserved with target-therapeutic exposure of ELX-02, consistent with the favorable tolerability profile across our preclinical and clinical data sets.

Currently, the clinical programs for our lead investigational drug candidate, ELX-02, are focused on development for cystic fibrosis patients with diagnosed nonsense mutations. We have completed a Phase 1 single ascending dose (SAD) trial at sites in Israel and Belgium, a multiple ascending dose (MAD) trial in Belgium and the United States, and a renal impairment study in the United States with subjects having mild, moderate and severe renal impairment. The results of the SAD study were published in *Clinical Pharmacology in Drug Development* in January 2019. The results from the MAD study were presented in 2019 at both the European Cystic Fibrosis Society clinical meeting and the North American Cystic Fibrosis Conference (NACFC). Additionally, the results from the renal impairment study were presented at the 2019 American Society of Nephrology (ASN) Kidney Week in November 2019.

Our scientific review written by Professor Eitan Kerem, M.D., Senior Attending Physician at the Hadassah CF Center in Jerusalem, Israel and Senior Medical Consultant to Eloxx, titled "ELX-02: an investigational read-through agent for the treatment of nonsense mutation-related genetic disease" was published in October 2020 by the *Expert Opinion on Investigational Drugs Journal*. This manuscript details the development of ELX-02 for the restoration of functional protein in nonsense-mediated disease in support of our ongoing Phase 2 trials.

Our Phase 2 cystinosis trial involved two sequential cohorts with three escalating doses in three patients per cohort. The first cohort enrolled three homozygous W138X patients ages 23 to 38, with prior kidney transplants and varying degrees of renal insufficiency. In January 2020, we announced positive data from the first cohort of the Phase 2 study of ELX-02 in the treatment of patients with nonsense mutation-mediated nephropathic cystinosis. The results of the first cohort met the primary safety endpoint and the reductions in white blood cell (WBC) cystine provided a clear indication of biologic activity in these patients at nominal doses > 0.5 mg/kg/day. Following review of the safety and pharmacokinetic data by an independent Safety Review Committee (SRC), the SRC approved progressing to the second cohort that would enable enrolling patients ages 12 and older. Due to study design limitations, patients across all dose groups had elevated and uncontrolled pretreatment WBC cystine levels which made it difficult to fully evaluate ELX-02-mediated WBC cystine reductions. Therefore, we have discontinued this study and will not proceed with the second cohort as contemplated in the original protocol. We will continue to review these data with a panel of scientific and clinical experts to determine appropriate modifications for a possible new study design.

The clear indications of biologic activity in this study provide human clinical proof of concept for ELX-02 and de-risk other clinical applications of our ERSG library using this dosage range. These encouraging results also provide a basis for expansion to studies of additional kidney diseases caused by nonsense mutations, such as ADPKD.

Our Phase 2 cystic fibrosis clinical trial program for ELX-02 is being conducted at leading global investigator sites in Europe, Israel and the United States. On March 25, 2020, we announced that enrollment in these trials had been paused temporarily in response to the global COVID-19 pandemic in order to avoid unnecessary exposure in at-risk populations, to maintain the integrity of our study data and to support global healthcare providers in their commitment to ensure patient safety. On June 17, 2020, we announced that enrollment in our Phase 2 clinical trial in cystic fibrosis had been resumed in Israel and Europe, and on August 12, 2020, we announced that enrollment in our Phase 2 clinical trial in cystic fibrosis has been resumed in the U.S. The COVID-19 pandemic continues to evolve, and we continue to work closely with our clinical sites and investigators. We are also evaluating additional clinical sites in other countries where patient enrollment may be feasible. We remain committed to completing enrollment in these Phase 2 proof of concept clinical trials and reporting top line data in the first half of 2021, which is contingent on no further disruptions due to the COVID-19 pandemic. Several planned Safety Review Committee meetings have occurred and allowed dose escalation up to the top dose level with no drug-related serious adverse events reported to date. We have had multiple patients progressing through the four-dose escalation range. In the U.S., the Cystic Fibrosis Foundation ("CF Foundation") is providing funding for a portion of the trial and we have formed a joint program advisory group with the CF Foundation focused on the development of ELX-02 for cystic fibrosis. The Cystic Fibrosis Therapeutics Development Network ("TDN") has sanctioned the Phase 2 study protocol, which is being conducted at TDN member sites. Additional information about our clinical trials can be found at www.ClinicalTrials.gov (Identifiers: NCT04126473 and NCT04135495).

Professor Eitan Kerem, M.D., former Head of the Division of Pediatrics, Children's Hospital, Hadassah Medical Center in Israel, has joined Eloxx as a Senior Medical Consultant. For the U.S. trial, Dr. Ahmet Uluer, Director of the Adult Cystic Fibrosis Program at the Boston Children's Hospital/Brigham and Women's Hospital CF Center, is the lead study investigator. The protocols have been sanctioned by the TDN in the U.S. and the European Cystic Fibrosis Society Clinical Trial Network (which has given our European/Israel trial a "high priority" ranking). During October 2019, we completed an interim CMC review meeting with the U.S. Food and Drug Administration (the "FDA") and we have gained alignment with the agency on our manufacturing formulation and process, which we believe will be suitable for our expected drug supply needs through completion of our pivotal trials. The in-person European Cystic Fibrosis Society conference in Lyon, France scheduled for June 2020 was cancelled, and we withdrew our abstract. We presented data from two scientific abstracts at the North American Cystic Fibrosis Virtual Conference (NACFC). The two abstracts were also showcased in the NACFC virtual poster gallery and electronically published as a supplement to Pediatric Pulmonology. The live sessions and discussions took place through October 23, 2020. These virtual posters are available to registered attendees on the NACFC online conference platform. The preclinical study results demonstrate ELX-02's selectivity for read-through of premature stop codons versus native stop codons and its ability to restore production of functional *CFTR* in patient-derived organoids.

We believe there is a significant unmet medical need in the treatment of cystic fibrosis patients carrying nonsense mutations on one or both alleles of the CFTR gene. Cystic fibrosis is the most prevalent genetic disease in the western world and there are no currently approved therapies that target the impairment associated with Class 1 CFTR mutations. We believe that nonsense mutations may impact a similar proportion of patients diagnosed with cystinosis. Given the high proportion of pediatric patients in many rare orphan diseases, we intend to apply for relevant Orphan Drug incentives in the U.S. and Europe, including the Rare Pediatric Disease Priority Review Voucher in the U.S. Currently, the European Medicines Agency (the "EMA") has designated ELX-02 as an orphan medicine for the treatment of cystic fibrosis and mucopolysaccharidosis type I (MPS I). The FDA had previously granted orphan drug designation to ELX-02 for the treatment of nephropathic cystinosis, MPS I, and Rett syndrome, and on August 4, 2020, we announced that the FDA has granted orphan drug designation for ELX-02 for the treatment of cystic fibrosis. The FDA's Office of Orphan Drug Products grants orphan status to support the development of medicines for underserved patient populations, or rare disorders, that affect fewer than 200,000 people in the U.S. Orphan drug designation qualifies Eloxx for certain benefits, including seven years of market exclusivity upon regulatory approval (if received), exemption from FDA application fees, tax credits on qualified U.S. clinical trials and eligibility for grant funding opportunities that can be used for clinical trial costs.

We are also evaluating the suitability of our ERSG library for development in rare renal diseases associated with nonsense mutations, such as ADPKD. ADPKD is a relatively common inherited genetic kidney disease occurring in between one in 400 and one in 1,000 patients and is the fourth leading cause of end-stage renal disease in the United States. Over 25% of the primary genetic changes that cause ADPKD are nonsense mutations, where a premature stop codon in the gene leads to a truncated, often unstable, protein. We have evaluated the three most relevant ADPKD nonsense mutations in an *in vitro* read-through assay and have demonstrated significant levels of read-through for ELX-02 and several library compounds, which is the first step in our preclinical development toward an IND.

We continue to progress our ERSG pipeline in IRDs, another area of high unmet medical need, that are associated with vision loss and blindness. There are over 300 IRDs associated with nonsense mutations. We recently reported on a critical milestone demonstrating that several of our library compounds successfully reach retinal disorder-relevant tissue layers and can restore protein production in an animal model. These data support that our ERSG compounds are suitable for reaching and promoting read-through in target cells within the retina. We had planned to present these data at the Association for Research in Vision and Ophthalmology (ARVO) Annual Meeting in May 2020, but the meeting was cancelled due to the global COVID-19 pandemic. As an alternative, we submitted a recorded video presentation which became available on ARVO's website May 6, 2020. Our IRD research also includes exploring multiple sustained release formulation technologies, and *in vitro* release rates achieved to date have been consistent with our target release profile of one to three months. Our scientific manuscript titled "Intravitreal administration of small molecule read-through agents demonstrate functional activity in a nonsense mutation mouse model" was published in October 2020 by the *Journal of Experimental Eye Research*. This manuscript demonstrates that multiple small molecules in our ERSG library mediate dose-dependent read-through at the back of the eye after a single intravitreal injection. Collectively, these manuscripts demonstrate the wide-ranging potential of our small molecule read-through approach to rare genetic disorders mediated by nonsense mutations; from targeted delivery for inherited retinal disorders to systemic delivery for multi-system disorders like cystic fibrosis.

On February 24, 2020, our Board of Directors approved a leadership and organizational realignment aimed at supporting our efforts to improve operating performance and concentrate development efforts on our core programs. The organizational realignment reduced managerial layers and consolidated roles across the organization, resulting in the elimination of 13 full-time positions during the first quarter of 2020. We incurred a resulting one-time pre-tax charge of $4.0 million during the first quarter of 2020.

COVID-19

The outbreak of COVID-19 and the preventative or protective actions that we, our employees, consultants, suppliers, contract research organizations (CROs), and other partners or governments may take may significantly disrupt our business operations. We are diligently working to ensure that we can operate with minimal disruption, and to mitigate the impact of the pandemic on our employees' health and safety and that of the patients and healthcare professionals in our clinical trials. However, given the significant uncertainty regarding the ongoing impact of the COVID-19 outbreak, there remains a risk that we or our employees, contractors, suppliers, and other partners may be prevented from conducting business activities for indefinite periods of time, including due to a substantial percentage of personnel contracting the virus or due to shutdowns that may be requested or mandated by governmental authorities. Given the interconnectivity of the global economy and the possible rate of future global transmission of the virus, the full extent to which the pandemic could affect the global economy is unknown and its impact may extend beyond the areas which are currently known by us to be affected.

Our management and Board of Directors are focused on the operational challenges resulting from the COVID-19 pandemic. To date, the pandemic has not had a material adverse impact on our financial condition, and we have not had to lay off or furlough any employees. Operations have continued even though our clinical trials were temporarily paused. Both Phase 2 clinical trials have now resumed. We are evaluating various alternatives to remain flexible and adapt to changing circumstances that may arise in the near and long term. We continue to monitor our operations, states of affairs in the regions in which we and our business partners operate and conduct research and clinical trial activities, and applicable government recommendations. As a result, we have made modifications to our normal operations, including restrictions on business travel and meetings, permitting employees to work remotely and the implementation of COVID-19 workplace safety guidelines to screen employees and office visitors for COVID-19 symptoms upon entering our offices. We have also implemented one-way traffic flows, social-distanced workspaces, additional cleaning requirements and mandatory face coverings for common spaces and provided components of Personal Protective Equipment (PPE) for all employees working out of our various office locations. Notwithstanding these measures, the COVID-19 pandemic could affect the health and availability of our workforce as well as those of the third parties we rely on. If members of our management and other key personnel in critical functions across our organization are unable to perform their duties or have limited availability due to COVID-19, we may not be able to execute on our business strategy and our operations may be adversely impacted. We may also experience limitations in employee resources, including due to illness of employees or their families or the desire of employees to avoid contact with individuals or large groups of people. In addition, we have experienced and will continue to experience disruptions to our business operations resulting from quarantines, self-isolations and travel and other restrictions on our employees which may impact their ability to perform their job responsibilities.

The extent and severity of the impact of the current global health crisis on our business and clinical trials will be determined largely by the ability of patients and prospective patients in our clinical trials to access trial sites, the ability of personnel from our CROs to oversee the administration of our drug in accordance with trial protocols and our ability to monitor and communicate effectively with our CROs, staff at clinical trial sites and principal investigators. In addition, the

impact of the COVID-19 pandemic on the operations of the FDA and other health authorities may delay potential advancement of our product candidates.

Critical Accounting Policies and Use of Estimates

Our management's discussion and analysis of financial condition and results of operations is based on our unaudited condensed consolidated financial statements, which have been prepared in accordance with accounting principles generally accepted in the United States, or U.S. GAAP. The preparation of these financial statements requires us to make estimates and assumptions that affect the reported amounts of assets and liabilities and the disclosure of contingent assets and liabilities at the date of the condensed consolidated financial statements, as well as the reported expense during the reporting periods. We monitor and analyze these items for changes in facts and circumstances, and material changes in these estimates could occur in the future. We base our estimates on historical experience and on various other factors that we believe are reasonable under the circumstances, the results of which form the basis for making judgments about the carrying value of assets and liabilities that are not readily apparent from other sources. Changes in estimates are reflected in reported results for the period in which they become known. Actual results may differ materially from these estimates under different assumptions or conditions.

The critical accounting policies that we believe impact significant judgments and estimates used in the preparation of our financial statements presented in this Report are described in "Management's Discussion and Analysis of Financial Condition and Results of Operations" in our 2019 Annual Report on Form 10-K. There have been no material changes to our critical accounting policies through September 30, 2020, from those discussed in our Annual Report on Form 10-K filed with the SEC on March 6, 2020.

Results of Operations

The following table summarizes our results of operations for each of the periods presented (in thousands):

	Three Months Ended September 30,		Change		Nine Months Ended September 30,		Change	
	2020	2019	$	%	2020	2019	$	%
Operating expenses:								
Research and development, net	$ 3,231	$ 6,801	$ (3,570)	(52) %	$ 11,308	$ 20,160	$ (8,852)	(44) %
General and administrative	3,065	5,978	(2,913)	(49) %	12,347	18,907	(6,560)	(35) %
Restructuring charges	—	—	—	—	3,994	—	3,994	—
Total operating expenses	6,296	12,779	(6,483)	(51) %	27,649	39,067	(11,418)	(29) %
Loss from operations	(6,296)	(12,779)	6,483	(51) %	(27,649)	(39,067)	11,418	(29) %
Other expense, net	321	96	225	234 %	801	174	627	360 %
Net loss	$ (6,617)	$ (12,875)	$ 6,258	(49) %	$ (28,450)	$ (39,241)	$ 10,791	(27) %

Research and development expense

Research and development expenses were $3.2 million for the three months ended September 30, 2020 compared to $6.8 million for the same period in 2019, a decrease of $3.6 million. The decrease in research and development expenses was primarily related to $2.7 million in reduced fees incurred for subcontractors, consultants and advisors in connection with ongoing clinical trials and research and development activities, due to delays in clinical trial enrollment resulting from the COVID-19 pandemic, a $0.4 million decrease in stock-based compensation and a $0.5 million decrease in salaries and other personnel-related costs due to lower headcount during the 2020 period. Research and development expenses for the three months ended September 30, 2020 and 2019 included non-cash stock-based compensation expense totaling $0.3 million and $0.7 million, respectively.

Research and development expenses were $11.3 million for the nine months ended September 30, 2020 compared to $20.2 million for the same period in 2019, a decrease of $8.9 million. The decrease in research and development expenses was primarily related to $7.2 million in reduced fees incurred for subcontractors, consultants and advisors in connection with ongoing clinical trials and research and development activities, due to delays in clinical trial enrollment resulting from the COVID-19 pandemic, a $1.2 million decrease in stock based compensation and a $0.4 million decrease in salaries and other personnel-related costs due to lower headcount during the 2020 period. Research and development expenses for the nine months ended September 30, 2020 and 2019 included non-cash stock-based compensation expense totaling $0.8 million and $2.0 million, respectively.

General and administrative expenses

General and administrative expenses were $3.1 million for the three months ended September 30, 2020, compared to $6.0 million for the same period in 2019, a decrease of $2.9 million. The decrease in general and administrative expenses was primarily due to decreases of $0.8 million in personnel and related costs due to a reduction in headcount and related salaries for the 2020 period, $1.1 million in stock-based compensation and $1.0 million in professional services and other infrastructure-related costs. General and administrative expenses for the three months ended September 30, 2020 and 2019 included non-cash stock-based compensation expense totaling $1.1 million and $2.2 million, respectively.

General and administrative expenses were $12.3 million for the nine months ended September 30, 2020, compared to $18.9 million for the same period in 2019, a decrease of $6.6 million. The decrease in general and administrative expenses was primarily due to decreases of $1.7 million in personnel and related costs due to a reduction in headcount and related salaries for a portion of the 2020 period, $2.2 million in stock-based compensation and $2.7 million in professional services and other infrastructure-related costs. General and administrative expenses for the nine months ended September 30, 2020 and 2019 included non-cash stock-based compensation expense totaling $4.5 million and $6.6 million, respectively.

Restructuring charges

Restructuring charges of $4.0 million for the nine months ended September 30, 2020 resulted from the leadership and organizational realignment during the first quarter of 2020. The total included $1.9 million related to contract termination and employee separation costs (primarily severance and benefits) and $2.1 million of non-cash stock compensation, relating to accelerated vesting of executive stock awards. There were no similar charges during the three months ended September 30, 2020.

Other expense, net

We recorded $0.3 million in other expense, net for the three months ended September 30, 2020, compared to $0.1 million for the same period in 2019. The increase in other expense, net was primarily due to lower interest income.

We recorded $0.8 million in other expense, net for the nine months ended September 30, 2020, compared to $0.2 million for the same period in 2019. The increase in other expense, net was primarily due to lower interest income.

Liquidity and Capital Resources

Liquidity is the ability of a company to generate funds to support its current and future operations, satisfy its obligations, and otherwise operate on an ongoing basis. Significant factors in the management of liquidity are funds generated by operations, levels of accounts receivable and accounts payable and capital expenditures. To date, we have not generated revenue from sales of any product or service.

Although the impact of the COVID-19 pandemic on clinical operations and trial enrollment cannot fully be determined, we believe that our cash and cash equivalents of $30.6 million at September 30, 2020, will enable us to meet the anticipated cash needs required to reach top line Phase 2 data in cystic fibrosis and maintain our current and planned operations through at least the next 12 months from the issuance of this Report. Since our inception, we have incurred significant operating losses. Our net losses were $(28.5) million for the nine months ended September 30, 2020, and $(50.9) million for the year ended December 31, 2019. As of September 30, 2020, we had an accumulated deficit of $(165.5) million. To date, we have financed our operations primarily through equity capital investments, and to a lesser extent, from loans and grants. We have devoted substantially all of our financial resources and efforts to research and development. We expect that it may be several years, if ever, before we receive regulatory approval and have a product candidate ready for commercialization. We expect to continue to incur significant expenses and operating losses for the foreseeable future. A successful transition to profitable operations is dependent upon achieving a level of revenue adequate to support our cost structure. Our net losses may fluctuate significantly from quarter to quarter and year to year. We anticipate that our expenses may increase if, and as, we:

- advance ELX-02 and/or other product candidates further into clinical development;
- experience additional delays in enrollment and completion of our clinical trials due to the COVID-19 pandemic or otherwise;
- continue the preclinical development of our research programs and advance candidates into clinical trials;
- pursue regulatory authorization to conduct clinical trials of additional product candidates;

- seek marketing approvals for our product candidates;
- establish a sales, marketing and distribution infrastructure to commercialize any product candidates for which we obtain marketing approval;
- maintain, expand and protect our intellectual property portfolio;
- hire additional clinical, regulatory, management and scientific personnel;
- add operational, financial and management information systems and personnel;
- acquire or in-license other product candidates and technologies; and
- operate as a public company.

We may never achieve profitability, and unless and until we do, we will continue to need to raise additional cash to fund our operations. On February 24, 2020, our Board of Directors approved a leadership and organizational re-alignment, which is expected to achieve annual cost savings of approximately $4.9 million primarily related to salaries and benefits, with anticipated fiscal year 2020 savings of approximately $2.3 million, net of severance costs. Our cash, cash equivalents, and marketable securities are highly liquid investments with original maturities of one year or less at the date of purchase and consist of cash in operating accounts and secured investments, primarily U.S. treasuries.

Management intends to fund future operations through private or public debt or equity financing transactions and may seek additional capital through arrangements with strategic partners or from other sources. If we are unable to obtain adequate financing, we will evaluate alternatives which may include reducing or deferring operating expenses, which may have a material adverse effect on our operations and future prospects.

Principal Financing Activities

On January 30, 2019, we entered into a Loan and Security Agreement (the "Loan Agreement") with Silicon Valley Bank ("SVB"), and WestRiver Innovation Lending Fund VIII, L.P. (together with SVB, the "Lenders"). Pursuant to the terms and conditions of the Loan Agreement, the Lenders extended a term loan to us of $15.0 million.

Outstanding principal on the loan accrues interest at a floating rate equal to the greater of (i) 5.25% per annum and (ii) the sum of 2.5% plus the prime rate, as published in the Wall Street Journal. Interest payments are payable monthly following the funding of the loan. On September 30, 2020, the interest rate was 5.75%. We commenced making payments on the outstanding principal balance of the loan on February 1, 2020, which is payable in 36 equal monthly installments. Amounts outstanding under the loan are due and payable on January 1, 2023.

In conjunction with the initial loan advance, we issued warrants (the "Warrants") to the Lenders to purchase an aggregate of 40,834 shares of our common stock at a warrant exercise price of $11.02 (subject to certain adjustments), which price was calculated using the 10-day average bid price of our common stock prior to the date of the Loan Agreement.

We may prepay the outstanding principal balance of the loans advanced by the Lenders in whole but not in part, subject to a prepayment fee ranging from 1% to 3% of any amount prepaid, depending upon when the prepayment occurs. We will also pay a final payment fee equal to 6% of the total loans advanced, due upon the earlier of maturity or termination of the Loan Agreement.

Under the terms of the Loan Agreement, we granted first priority liens and security interests in substantially all of our assets (excluding all of its intellectual property, which is subject to a negative pledge) and a pledge by us of the shares of one of our wholly-owned subsidiaries as collateral for the obligations thereunder. The Loan Agreement also contains representations and warranties by us and the Lenders and indemnification provisions in favor of the Lenders and customary covenants (including limitations on other indebtedness, liens, acquisitions, investments and dividends, but no financial covenants), and events of default (including payment defaults, breaches of covenants following any applicable cure period, a material impairment in the perfection or priority of the Lenders' security interest in the collateral, and events relating to bankruptcy or insolvency).

In April 2020, we entered into a loan agreement with SVB under the U.S. Small Business Administration (the "SBA") Paycheck Protection Program (the "PPP") pursuant to the Coronavirus Aid, Relief and Economic Security Act of 2020 (the "CARES Act") and received loan proceeds of $0.8 million (the "PPP Loan"). We expect to use the loan proceeds for payroll

and other covered costs in accordance with the relevant terms and conditions of the CARES Act. We issued a promissory note for the PPP Loan with a maturity date of April 21, 2022 and an interest rate of 1.0% per annum. Monthly payments of principal and interest will be due beginning on September 21, 2021, although interest accrues from the issuance date. We may prepay the PPP Loan without penalty or premium, and the promissory note provides for customary events of default. A PPP loan may be partially or entirely forgiven based on employee retention for the 24-week period starting on the loan date through October 2020, and the use of loan proceeds for payroll or other specified costs during the same period. Forgiveness is also based on the employer maintaining or restoring headcount and maintaining salary levels. Forgiveness is reduced if headcount declines or if salaries decrease. Any loan forgiveness will be made subject to SVB approval in accordance with SBA requirements.

On June 24, 2019, we completed an underwritten public offering of 3,833,334 shares of common stock at the public offering price of $9.00 per share and received gross proceeds of approximately $34.5 million, before deducting underwriting discounts and commissions of $2.1 million and offering expenses of $0.2 million.

Cash Flows

The following table summarizes our sources and uses of cash for each of the periods presented (in thousands):

	Nine Months Ended September 30,	
	2020	2019
Net cash used in operating activities	$ (23,498)	$ (30,134)
Net cash provided by (used in) investing activities	33,792	(42,603)
Net cash (used in) provided by financing activities	(2,186)	46,281

Our operating activities used cash of $23.5 million and $30.1 million during the nine months ended September 30, 2020 and 2019, respectively. For the nine months ended September 30, 2020, net cash used in operating activities resulted primarily from our net loss of $(28.5) million and total changes in working capital of $3.4 million partially offset by total non-cash charges of $8.3 million. Non-cash charges primarily related to $7.4 million of stock-based compensation, $0.5 million of amortization of lease assets, and $0.4 million of debt discount amortization. Changes in working capital were primarily related to decreases of $1.7 million in accrued expenses, $1.2 million in accounts payable, $0.3 million in operating lease liabilities, and an increase of $0.1 million in prepaid expenses and other assets. For the nine months ended September 30, 2019, net cash used in operating activities resulted primarily from our net loss of $(39.2) million partially offset by total non-cash charges of $9.2 million and total changes in working capital of $0.1 million. Non-cash charges primarily related to $8.6 million of stock-based compensation, $0.3 million of amortization of lease assets, $0.4 million of debt discount amortization and $0.1 million of depreciation expense, offset by $0.2 million of discount amortization on our investments. Changes in working capital were primarily related to an increase in prepaid expenses and other current assets of $0.2 million and advances from collaboration partners of $0.4 million related to the achievement of a milestone in connection with the CF Foundation funding commitment for our cystic fibrosis development program in the U.S.

Our investing activities provided cash of $33.8 million and used cash of $42.6 million during the nine months ended September 30, 2020 and 2019, respectively. For the nine months ended September 30, 2020, cash provided by investing activities was primarily related to $33.8 million of proceeds from maturities of marketable securities. For the nine months ended September 30, 2019, cash used in investing activities consisted primarily of $56.0 million in purchases of marketable securities, offset by $13.5 million of proceeds from maturities of marketable securities.

Our financing activities used cash of $2.2 million during the nine months ended September 30, 2020 and provided cash of $46.3 million during the nine months ended September 30, 2019. For the nine months ended September 30, 2020, net cash used in financing activities consisted primarily of $3.3 million in term loan principal repayments, offset by $0.8 million received from the PPP Loan and $0.4 million in advances received from collaboration partners. For the nine months ended September 30, 2019, net cash provided by financing activities resulted primarily from net proceeds of $32.7 million from sales of common stock and the issuance of debt of $15.0 million in January 2019 offset by $1.2 million of taxes paid upon the vesting of restricted stock units and $0.3 million of debt issuance costs.

Equity Sales Agreement

In November 2018, we entered into an Equity Distribution Agreement (the "Agreement") with Citigroup Global Markets Inc. and Cantor Fitzgerald & Co. (collectively, the "Sales Agents"), pursuant to which we may sell and issue shares of our common stock up to an aggregate of $50 million through the Sales Agents. The shares were offered pursuant to a registered shelf offering. For the year ended December 31, 2018, under the Agreement, we sold 201,100 shares of common stock and received net proceeds of $2.2 million. In January 2019, we sold 35,362 shares of common stock and received net proceeds of $0.7 million.

Off-Balance Sheet Arrangements

During the periods presented, we did not have, and we do not have, any off-balance sheet arrangements, as such term is defined under Item 303 of Regulation S-K, that have or are reasonably likely to have a current or future effect on our financial condition, changes in financial condition, revenue or expense, results of operations, liquidity, capital expenditures or capital resources that is material to investors.

Item 3. Quantitative and Qualitative Disclosures about Market Risk

Not applicable to a "smaller reporting company", as defined in Item 10(f)(1) of SEC Regulation S-K.

Item 4. Controls and Procedures

Management's Evaluation of Disclosure Controls and Procedures

We maintain disclosure controls and procedures that are designed to ensure that information required to be disclosed in the reports that we file or submit under the Securities and Exchange Act of 1934 (the "Exchange Act") is (1) recorded, processed, summarized, and reported within the time periods specified in the SEC's rules and forms and (2) accumulated and communicated to our management, including our principal executive officer and principal financial officer, to allow timely decisions regarding required disclosure.

As of September 30, 2020, our management, under the supervision of and with the participation of our principal executive officer and principal financial officer, evaluated the effectiveness of the design and operation of our disclosure controls and procedures (as defined in Rules 13a-15(e) and 15d-15(e) under the Exchange Act). Our 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. Our principal executive officer and principal financial officer have concluded based upon the evaluation described above that, as of September 30, 2020, our disclosure controls and procedures were effective in ensuring that material information relating to the Company, including its consolidated subsidiaries, required to be disclosed by the Company in the reports that it files or submits under the Exchange Act is recorded, processed, summarized, and reported within the time period specified in the SEC's rules and forms, including ensuring that such material information is accumulated and communicated to our management, including our principal executive officer and principal financial officer, as appropriate, to allow timely decisions regarding required disclosures.

Changes in Internal Control over Financial Reporting

During the quarter ended September 30, 2020, there have been no changes in our internal control over financial reporting, as such term is defined in Rules 13a-15(f) and 15(d)-15(f) promulgated under the Securities Exchange Act of 1934, that have materially affected, or are reasonably likely to materially affect, our internal control over financial reporting.

PART II. OTHER INFORMATION

Item 1. Legal Proceedings

From time to time, we may become involved in various lawsuits and legal proceedings, which arise in the ordinary course of business. We are currently unaware of any material pending legal proceedings to which we are party or of which our property is the subject. However, we may at times in the future become involved in litigation in the ordinary course of business, which may include actions related to or based on our intellectual property and its use, customer claims, product liability claims, employment practices and employee complaints and other events arising out of our operations. When appropriate in management's estimation, we will record adequate reserves in our financial statements for pending litigation. Litigation is subject to inherent uncertainties, and an adverse result in any such matters could adversely impact our reputation, operations, and our financial operating results or overall financial condition. Additionally, any litigation to which we may become subject could also require significant involvement of our senior management and may divert management's attention from our business and operations.

Item 1A. Risk Factors

Investing in our common stock involves a high degree of risk. You should carefully consider the risks and uncertainties described below, together with all other information in this Report, before you decide to purchase our common stock. If any of the possible adverse events described below actually occurs, we may be unable to conduct our business as currently planned and our financial condition and operating results could be harmed. In addition, the trading price of our common stock could decline due to the occurrence of any of the events described below, and you may lose all or part of your investment. Additional risks that we currently do not know about, or that we currently believe immaterial, may also impair our business.

Risks Related to Drug Discovery, Development, Regulatory Approval and Commercialization

We depend heavily on the success of our lead product candidate, ELX-02. If ELX-02 fails during development or suffers any material delays, it may adversely impact the commercial viability of ELX-02 and our business.

We currently have no products approved for sale. To date, we have invested substantial efforts and financial resources in the research and development of ELX-02, which is currently our only product candidate in clinical development. We have increased investment in our preclinical candidate portfolio but have yet to advance other molecules into clinical development.

Our ability to achieve and sustain profitability depends on obtaining regulatory approvals for, and successfully commercializing ELX-02 and any future product candidates, either alone or with third parties. Before obtaining regulatory approval for the commercial distribution of our therapeutic product candidates, we or a collaborator must conduct extensive preclinical studies and clinical trials to demonstrate the safety and efficacy in humans of our product candidates. The clinical trials, manufacturing and marketing of ELX-02, and any future product candidates, will be subject to extensive and rigorous review and regulation by numerous governmental authorities in the U.S., the EU and other jurisdictions where we intend to test and, if approved, market our current and future product candidates. Before obtaining regulatory approvals for the commercial sale of any product candidate, we must demonstrate through preclinical studies and clinical trials that the product candidate is safe and effective for use in each target indication, and potentially in specific patient populations, including the pediatric population. This process can take many years and may include post-marketing studies and surveillance, which would require the expenditure of substantial resources. Of the large number of drugs in development for approval in the U.S. and the EU, only a small percentage successfully complete the FDA or EMA regulatory approval processes and are commercialized. Accordingly, even if we are able to obtain the requisite financing to continue to fund our research, development and clinical programs, we cannot assure you that ELX-02 or any of our future product candidates will be successfully developed or commercialized.

Preclinical studies and clinical trials are expensive, difficult to design and implement, can take many years to complete and are uncertain as to outcome. The start or end of a clinical trial is often delayed or halted due to changing regulatory requirements, manufacturing challenges, required clinical trial administrative actions, slower than anticipated patient enrollment, changing standards of care, availability or prevalence of use of a comparative therapeutic or required prior or combination therapy, clinical outcomes or financial constraints. For instance, delays or difficulties in patient enrollment or difficulties in retaining trial participants can result in increased costs, longer development times or termination of a clinical trial. Clinical trials of a new product candidate require the enrollment of a sufficient number of patients, including patients who are suffering from the disease the product candidate is intended to treat and who meet other eligibility criteria. Rates of patient enrollment are affected by many factors, including the size of the patient population, the eligibility criteria for the clinical trial, the age and condition of the patients, the stage and severity of disease, the nature of the protocol, the proximity of patients to clinical sites and the availability of effective treatments for the relevant disease.

The results of preclinical studies and early clinical trials of our product candidates may not be predictive of the results of later-stage clinical trials. Product candidates in later stages of clinical development may fail to show the desired safety and efficacy traits despite having progressed through preclinical studies and initial clinical trials. A number of companies in the biopharmaceutical industry have suffered significant setbacks in advanced clinical trials due to lack of efficacy or safety profiles, notwithstanding promising results in earlier trials. Accordingly, we, or any development partners, may ultimately be unable to provide regulatory agencies with satisfactory data on clinical safety and efficacy sufficient to obtain approval for any indication.

Further, we may experience delays in clinical trials of our product candidates. We do not know whether ongoing clinical trials will be completed on schedule or at all, or whether planned clinical trials will begin on time, need to be redesigned, enroll patients on time or be completed on schedule, if at all. Clinical trials can be delayed for a variety of reasons, including delays related to:

- obtaining regulatory approval to commence a trial;
- reaching agreement on acceptable terms with prospective contract research organizations, or CROs, and clinical trial sites, the terms of which can be subject to extensive negotiation and may vary significantly among different CROs and clinical trial sites;
- obtaining institutional review board, or IRB, approval at each clinical trial site;
- recruiting suitable patients to participate in a trial;
- having patients complete a trial or return for post-treatment follow-up;
- clinical trial sites deviating from trial protocol or dropping out of a trial;
- adding new clinical trial sites;
- unforeseen factors beyond our control, including public health concerns such as the COVID-19 pandemic; or
- manufacturing sufficient quantities of product candidate for use in clinical trials.

Patient enrollment, a significant factor in the timing of clinical trials, is affected by many factors including the size and nature of the patient population, the proximity of patients to clinical sites, the eligibility criteria for the trial, the design of the clinical trial, competing clinical trials and clinicians' and patients' perceptions as to the potential advantages of the drug being studied in relation to other available therapies, including any new drugs that may be approved for the indications we are investigating. Furthermore, we rely on third parties, such as CROs and clinical trial sites, to ensure the proper and timely conduct of our clinical trials and while we have agreements governing their committed activities, we may have limited influence over their actual performance.

On March 25, 2020, we announced that enrollment in our clinical trials had been paused temporarily in response to the COVID-19 pandemic in order to avoid unnecessary exposure in at-risk populations, to maintain the integrity of our study data and to support global healthcare providers in their commitment to ensure patient safety. On June 17, 2020, we announced that enrollment in our Phase 2 clinical trial in cystic fibrosis had resumed in Israel and Europe, and on August 12, 2020, we announced that enrollment in our Phase 2 clinical trial in cystic fibrosis had resumed in the U.S. COVID-19 is continuing to evolve and we continue to work closely with our clinical trial sites and investigators. While we remain committed to completing enrollment in these Phase 2 proof of concept clinical trials and reporting top line data in the first half of 2021, we cannot provide assurances as to when this will be accomplished or whether we will incur significant additional costs, expend additional resources or be subject to additional regulatory requirements, including COVID-19 related disruptions, any of which may have a material adverse impact on our financial condition and results of operations.

We could encounter delays if prescribing physicians encounter unresolved ethical issues associated with enrolling patients in clinical trials of our product candidates in lieu of prescribing existing treatments that have established safety and efficacy profiles. Further, a clinical trial may be suspended or terminated by us, our collaborators, the IRB of the institutions in which such trials are being conducted, the Data Safety Monitoring Board (“DSMB”) for such trial, or by the FDA or other regulatory authorities due to a number of factors, including:

- failure to conduct the clinical trial in accordance with regulatory requirements or our clinical protocols;
- inspection of the clinical trial operations or trial site by the FDA or other regulatory authorities resulting in the imposition of a clinical hold;

- unforeseen safety issues or adverse side effects;
- failure to demonstrate a benefit from using a drug;
- changes in governmental regulations or administrative actions; or
- lack of adequate funding to continue the clinical trial.

In addition, significant adverse events with respect to individuals who are not enrolled in any of our clinical trials but who receive our drug candidate under our compassionate use policy (typically under a single-patient investigational new drug application ("IND") administered by the individual's treating physician) may result in a partial or full clinical hold on our ongoing clinical trials. A clinical hold may result in the inability to enroll new patients in our studies until the hold is removed and may make it more difficult to enroll patients thereafter. Additionally, a clinical hold may also result in, among other things, protocol redesign, changes in eligibility criteria and increased costs, any of which could adversely affect our projected development timelines and jeopardize successful completion of our clinical programs.

If we experience delays in the completion of any clinical trial of our product candidates, the commercial prospects of our product candidates may be impaired and our ability to generate product revenues from such product candidates may be delayed. In addition, any delays in completing our clinical trials may increase our costs, slow down our product development and approval process and may jeopardize our ability to commence product sales and generate revenues. Any of these occurrences may have an adverse impact on our business, financial condition and prospects. Further, 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.

We and our collaborating partners may be subject, directly or indirectly, to federal and state healthcare fraud and abuse and false claims laws and regulations. If we or our collaborating partners are unable to comply, or have not fully complied, with such laws, we could face substantial penalties.

All marketing activities associated with product candidates that are approved for sale in the U.S., if any, will be, directly or indirectly through our customers, subject to numerous federal and state laws governing the marketing and promotion of pharmaceutical products in the U.S., including, without limitation, the federal Anti-Kickback Statute, the federal False Claims Act and the Health Insurance Portability and Accountability Act ("HIPAA"). These laws may adversely impact, among other things, our proposed sales, marketing and education programs.

The federal Anti-Kickback Statute prohibits persons from knowingly and willfully soliciting, receiving, offering or paying remuneration, directly or indirectly, to induce either the referral of an individual, or the furnishing, recommending, or arranging for a good or service, for which payment may be made under a federal healthcare program, such as the Medicare and Medicaid programs. The term "remuneration" has been broadly interpreted to include anything of value, including for example, gifts, discounts, the furnishing of supplies or equipment, credit arrangements, payments of cash, waivers of co-payments and deductibles, ownership interests and providing anything at less than its fair market value. The reach of the Anti-Kickback Statute was also broadened by the Patient Protection and Affordable Care Act, as amended by the Health Care and Education Affordability Reconciliation Act, or the PPACA, which, among other things, amends the intent requirement of the federal Anti-Kickback Statute and the applicable criminal healthcare fraud statutes. Pursuant to the 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, PPACA 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 (discussed below) or the civil monetary penalties statute, which imposes penalties against any person who is determined to have presented or caused to be presented a claim to a federal health program that the person knows or should know is for an item or service that was not provided as claimed or is false or fraudulent. Penalties for violations of the federal Anti-Kickback Statute include criminal penalties and civil sanctions such as fines, imprisonment and possible exclusion from Medicare, Medicaid and other state or federal healthcare programs. Many states have also 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.

The federal False Claims Act imposes liability on any person who, among other things, knowingly presents, or causes to be presented, a false or fraudulent claim for payment by a federal healthcare program. The "qui tam" provisions of the False Claims Act allow a private individual to bring civil actions on behalf of the federal government alleging that the defendant has submitted a false claim to the federal government, and to share in any monetary recovery. In addition, various states have enacted false claims laws analogous to the False Claims Act. Many of these state laws apply where a claim is

submitted to any third-party payer and not merely a federal healthcare program. When an entity is determined to have violated the False Claims Act, it may be required to pay up to three times the actual damages sustained by the government, plus civil penalties up to approximately $22,000 for each separate false claim.

The HIPAA created several new federal crimes, including health care fraud, and false statements relating to health care matters. The health care fraud statute prohibits knowingly and willfully executing a scheme to defraud any health care benefit program, including private third-party payors. The false statements statute prohibits knowingly and willfully falsifying, concealing or covering up a material fact or making any materially false, fictitious or fraudulent statement in connection with the delivery of or payment for health care benefits, items or services.

We are unable to predict whether we could be subject to actions under any of these or other fraud and abuse laws, or the impact of such actions. Moreover, to the extent that any of our product candidates will be sold in a foreign country, if approved for marketing, we and our current or future collaborators may be subject to similar foreign laws and regulations. If we or any of our current or future collaborators are found to be in violation of any of the laws described above and other applicable state and federal fraud and abuse laws, we may be subject to penalties, including civil and criminal penalties, damages, fines, exclusion from government healthcare reimbursement programs and the curtailment or restructuring or our operations, any of which could have a material adverse effect on our business, results of operations and financial condition.

Positive results from preclinical or in vitro and in vivo testing of ELX-02 are not necessarily predictive of the results of future clinical trials of ELX-02. If we cannot achieve positive results in our clinical trials for ELX-02, we may be unable to successfully develop, obtain regulatory approval for and commercialize ELX-02.

Positive results from our preclinical testing of ELX-02 in vitro and in vivo may not necessarily be predictive of the results from our ongoing and planned clinical trials in humans. Many companies in the pharmaceutical and biotechnology industries have suffered significant setbacks in clinical trials after achieving positive results in preclinical and in vitro and in vivo studies, and we, or the third parties whose product candidates we expect to be co-administered with ELX-02, may face similar setbacks. Preclinical and clinical data are often susceptible to varying interpretations and analyses, and the FDA or EMA or other regulatory agencies may require changes to our protocols or other aspects of our clinical trials or require additional studies. Additionally, many companies that believed their product candidates performed satisfactorily in preclinical studies and clinical trials nonetheless failed to obtain FDA or EMA approval. If we fail to secure positive results from our clinical trials of ELX-02 or regulatory agencies require us to undertake significant additional studies as a result of our data, the development timeline, regulatory approval and commercialization prospects for our lead product candidate, and, correspondingly, our business and financial prospects, would be materially adversely affected, which may result in termination of development activities, the inability to raise additional needed capital and/or a precipitous decline in our stock price, as well as impair our ability to enter into collaboration arrangements or damage existing strategic partnerships.

Our product candidates, including ELX-02, may cause adverse events or have other properties that could delay or prevent their regulatory approval or limit the scope of any approved label or market acceptance.

Undesirable side effects caused by our product candidates, such as ELX-02, could cause us or regulatory authorities to interrupt, delay or halt clinical trials and could result in the denial of regulatory approval by the FDA or other comparable foreign regulatory authorities. It is possible that, during the course of the clinical development of ELX-02 or other product candidates, results of our clinical trials (or significant adverse events experienced by individuals receiving drug under our compassionate use policy) could reveal an unacceptable severity and prevalence of side effects. For example, in preclinical testing of ELX-02, we observed renal toxicities in the animals we tested following administration of this compound at doses in excess of the doses we expect to administer in our clinical trials. As a result of this or any other side effects, our clinical trials could be suspended or terminated or not even allowed to commence, and the FDA or comparable foreign regulatory authorities could order us to cease further development, or deny approval, of our product candidates for any or all targeted indications. The drug-related side effects could affect patient recruitment or the ability of enrolled patients to complete the trial or result in potential product liability claims.

Additionally, if one or more of our product candidates receive marketing approval, and we or others later identify undesirable side effects caused by such products, a number of potentially significant negative consequences could result, including:

- regulatory authorities may withdraw approvals of such product or impose restrictions on its distribution in the form of a new or modified risk evaluation and mitigation strategy;
- regulatory authorities may require additional labeling, such as additional warnings or contraindications, which may negatively impact sales;

- we may be required to change the way the product is administered or to conduct additional clinical studies;
- we could be sued and held liable for harm caused to patients; and
- our reputation may suffer.

Any of these events could prevent us from achieving or maintaining market acceptance of the particular product candidate, if approved, and could significantly harm our business, results of operations and prospects.

Our clinical trials may be costly, lengthy, time-consuming and difficult to design and implement, may result in unforeseen costs and could be delayed or terminated, which may have a material adverse effect on our business, results of operations and financial condition.

For human trials, patients must be recruited, and each product candidate must be tested at various doses and formulations for each clinical indication. In addition, to ensure safety and effectiveness, the effect of drugs often must be studied over a long period of time, especially for the chronic genetic diseases that we will be studying. Many of our programs focus on diseases with small patient populations, making patient recruitment and enrollment difficult. Insufficient patient enrollment in our clinical trials could delay or cause us to abandon a product development program. We may decide to abandon development of a product candidate or a study at any time due to unfavorable results, or we may have to spend considerable resources repeating clinical trials or conducting additional trials, either of which would increase costs and delay any revenue from those product candidates, if any.

Failure or delay in the commencement or completion of our clinical trials may be caused by several factors, including:

- slower than expected rates of patient recruitment, particularly with respect to trials of rare diseases such as nonsense mutation cystic fibrosis;
- determination of dosing levels and corresponding effect analysis;
- unforeseen safety issues;
- lack of effectiveness during clinical trials;
- inability to monitor patients adequately during or after treatment;
- inability or unwillingness of medical investigators and IRBs to follow our clinical protocols;
- unforeseen factors beyond our control, including public health concerns such as the COVID-19 pandemic; and
- lack of sufficient funding to finance the clinical trials.

We may find it difficult to recruit and enroll patients in our clinical trials, which could cause significant delays in the completion of such trials or may cause us to abandon one or more clinical trials.

Some of the diseases that our product candidates are intended to treat are rare and ultra-rare and we expect only a subset of the patients with these diseases will be eligible for our clinical trials. Because ELX-02 targets small populations and patient numbers have not been determined definitively, we must be able to identify patients in order to complete our development programs, secure regulatory approval and commercialize ELX-02 successfully.

In addition, the protocol for our clinical trials generally mandates that a patient cannot be involved in more than one clinical trial for the same indication. Therefore, subjects that participate in ongoing clinical trials for products that are competitive with our product candidates are not eligible to participate in our clinical trials. We cannot guarantee that any of our programs will identify a sufficient number of patients to complete clinical development, pursue regulatory approval and market our product candidates, if approved. The combined number of patients in the U.S., Japan and Europe and elsewhere may turn out to be lower than expected, may not be otherwise amenable to treatment with ELX-02, or new patients may become increasingly difficult to identify, all of which would adversely affect our results of operations and our business. An inability to recruit and enroll a sufficient number of patients for any of our current or future clinical trials would result in significant delays or may require us to abandon one or more clinical trials altogether, which could impact our ability to develop our product candidates and may have a material adverse effect on our business, results of operations and financial condition. On March 25, 2020, we announced that enrollment in our clinical trials had been paused temporarily in response to the COVID-19 pandemic in order to avoid unnecessary exposure in at-risk populations, to maintain the integrity of our study data and to support global healthcare providers in their commitment to ensure patient safety. On June 17, 2020, we

announced that enrollment in our Phase 2 clinical trial in cystic fibrosis had resumed in Israel and Europe, and on August 12, 2020, we announced that enrollment in our Phase 2 clinical trial in cystic fibrosis had resumed in the U.S. COVID-19 is continuing to evolve and we continue to work closely with our clinical trial sites and investigators to ensure that patient enrollment will continue as quickly as is feasible in a safe environment for our patients. We are also evaluating additional clinical sites in other countries where patient enrollment may be feasible. Additionally, significant additional costs as a result of this delay in enrollment or failure to complete enrollment in accordance with our objectives may have a material adverse impact on our financial condition and results of operations.

Because our clinical trials depend upon third-party researchers, scientists and consultants, the results of our clinical trials and such research activities are subject to delays and other risks that are, to a certain extent, beyond our control, which could impair our clinical development programs and our competitive position.

We depend on independent investigators, consultants, researchers, medical experts, collaborators, chemists, toxicologists and a small number of medical institutions and third-party contract research organizations to assist with our research efforts and conduct our preclinical and clinical trials and related activities. These collaborators, scientists, consultants and other third parties have provided, and we expect that they will continue to provide, valuable advice and services regarding our clinical development programs and product candidates. These collaborators, scientists, consultants and other third parties are not our employees, may have other commitments that would limit their future availability to us and typically will not enter into non-compete agreements with us. We cannot control the amount or timing of resources that they devote to our preclinical and or clinical development programs and they may not assign as great a priority to our preclinical or clinical development programs or pursue them as diligently as we would if we were undertaking such programs directly. If outside collaborators fail to devote sufficient time and resources to our preclinical and clinical development programs, or if their performance is substandard, the authorization of INDs and clinical trial applications ("CTAs") and the approval of anticipated new drug applications ("NDAs") and other marketing applications, and our introduction of new drugs, if any, may be delayed or impeded, which could impair our clinical development programs and would have a material adverse effect on our business and results of operations. These collaborators may also have relationships with other commercial entities, some of whom may compete with us and we may be unable to prevent them from establishing competing businesses or developing competing products. The extent to which the COVID-19 pandemic and municipalities' efforts to combat it through temporary quarantines, containment zones and limitations on travel, as well as other restrictions, may create business disruptions within the organizations of our third-party researchers, scientists and consultants, as well as CROs, clinical trial sites and patient assistance groups, that result in the unavailability of personnel needed to successfully conduct and complete our clinical trials, may have a material adverse impact on our business and financial condition.

We are subject to extensive governmental regulation including the requirements of the FDA and comparable foreign regulatory authorities for development and approval of our product candidates before they can be marketed.

We, our product candidates, our suppliers, our contract manufacturers, our contract testing laboratories and our clinical trial sites and clinical trial researchers are subject to extensive regulation by the FDA and comparable foreign regulatory authorities. Failure to comply with applicable requirements of the FDA or comparable foreign regulatory authorities could result in, among other things, any of the following actions:

- warning letters;
- fines and other monetary penalties;
- unanticipated expenditures;
- holds on the initiation or continuation of clinical trials;
- delays in the FDA's or other foreign regulatory authorities' approving, or the refusal of any regulatory authority to approve, any product candidate;
- product recall or seizure;
- interruption of manufacturing or clinical trials;
- operating restrictions;
- injunctions; and
- criminal prosecutions.

In addition to the approval requirements, other numerous and pervasive regulatory requirements apply, both before and after approval of our product candidates, to us, our product candidates, and our suppliers, contract manufacturers, and contract laboratories, and our clinical trial sites and clinical trial researchers including requirements related to testing, manufacturing, quality control, labeling, advertising, promotion, distribution, exporting product materials, reporting to the FDA of certain adverse experiences associated with use of the product candidate, and obtaining additional approvals for certain modifications to the product candidate or its labeling or claims following approval, if any.

We also are subject to inspection by the FDA and comparable foreign regulatory authorities, to determine our compliance with regulatory requirements, as are our suppliers, contract manufacturers, contract testing laboratories, and our clinical trial sites and clinical researchers, and there can be no assurance that the FDA or any other comparable foreign regulatory authority will not identify compliance issues that may disrupt production or distribution, or require substantial resources to correct. We may be required to make modifications to our manufacturing operations in response to these inspections, which may require significant resources and may have a material adverse effect upon our business, results of operations and financial condition.

The approval process for any product candidate may also be delayed by changes in government regulation, future legislation or administrative action or changes in policy of the FDA and comparable foreign regulatory authorities that occur prior to or during their respective regulatory reviews of such product candidate. Delays in obtaining regulatory approvals with respect to any product candidate may:

- delay commercialization of, and our ability to derive product revenue from, such product candidate;
- delay any regulatory-related milestone payments payable under outstanding collaboration agreements;
- require us to perform costly procedures with respect to such product candidate; or
- otherwise diminish any competitive advantages that we may have with respect to such product candidate.

We may not obtain the necessary FDA, EMA or other worldwide regulatory approvals to commercialize our product candidates in a timely manner, if at all, which would have a material adverse effect on our business, results of operations and financial condition.

We need FDA approval to commercialize our product candidates in the U.S., EMA approval to commercialize our product candidates in the EU and approvals from other foreign regulatory authorities to commercialize our product candidates elsewhere in the world. In order to obtain FDA approval of any of our product candidates, we must submit to the FDA an NDA demonstrating that the product candidate is safe for humans and effective for its intended use. This demonstration requires significant research and animal tests, which are referred to as preclinical studies, as well as human tests, which are referred to as clinical trials. In the EU, we must submit a Marketing Authorization Application, or MAA, to the EMA. Satisfaction of the regulatory requirements of the FDA, the EMA and other foreign regulatory authorities typically takes many years, depends upon the type, complexity and novelty of the product candidate and requires substantial resources for research, development and testing. Even if we comply with all the requests of regulatory authorities, they may ultimately reject any marketing applications that we file for our product candidates, or we might not obtain regulatory clearance in a timely manner if at all. Companies in the pharmaceutical and biotechnology industries have suffered significant setbacks in advanced or late-stage clinical trials, even after obtaining promising earlier trial results or preliminary findings or other comparable results for such clinical trials. Further, even if favorable testing data is generated during the clinical trials of a product candidate, the applicable regulatory authority may not accept or approve the marketing application filed by a pharmaceutical or biotechnology company for the product candidate. Failure to obtain approval of the FDA, EMA or comparable foreign regulatory authorities of any of our product candidates in a timely manner, if at all, will severely undermine our business, financial condition and results of operation by reducing our potential marketable products and our ability to generate corresponding product revenue.

Our research and clinical efforts may not result in drugs that the FDA, EMA or other foreign regulatory authorities consider safe for humans and effective for indicated uses, which would have a material adverse effect on our business, results of operations and financial condition. After clinical trials are completed for any product candidate, if at all, the FDA, EMA and other foreign regulatory authorities have substantial discretion in the drug approval process of the product candidate in their respective jurisdictions and may require us to conduct additional clinical testing or perform post-marketing studies, which would cause us to incur additional costs. Incurring such costs may have a material adverse effect on our business, results of operations and financial condition.

If we are unable to establish sales and marketing capabilities or enter into agreements with third parties to market and sell any of our product candidates that obtain regulatory approval, we may be unable to generate any revenue.

We have no experience selling and marketing our product candidates or any other products. To successfully commercialize any products that may result from our clinical development programs and obtain regulatory approval, we will need to develop these capabilities, either on our own or with the assistance of others. We may seek to enter into collaborations with other entities to utilize their marketing and distribution capabilities, but we may be unable to do so on favorable terms, if at all. If any future collaborative partners do not commit sufficient resources to commercialize our future products, if any, and we are unable to develop the necessary marketing capabilities on our own, we will be unable to generate sufficient product revenue to sustain our business. We will be competing with many companies that currently have extensive and well-funded marketing and sales operations. Without an internal team or the support of a third party to perform marketing and sales functions, we may be unable to compete successfully against these more established companies or successfully commercialize any of our product candidates.

Even though we have received orphan drug designation from the FDA for ELX-02 for the treatment of cystic fibrosis, cystinosis, MPS I, and Rett syndrome, we may not be able to obtain orphan drug marketing exclusivity for ELX-02 or any of our other potential product candidates for other indications.

Regulatory authorities in some jurisdictions, including the U.S. and the EU, may designate drugs for relatively small patient populations as orphan drugs. Under the Orphan Drug Act of 1983, the FDA may designate a drug as an orphan drug if it is intended to treat a rare disease or condition, which is generally defined as a patient population of fewer than 200,000 individuals annually in the U.S. Similarly, in Europe, a medicinal product may receive orphan designation under Article 3 of Regulation (EC) 141/2000. This applies to products that are intended for a life-threatening or chronically debilitating condition and either the condition affects no more than five in 10,000 persons in the EU when the application is made or the product, without the benefits derived from orphan status, would unlikely generate sufficient return in the EU to justify the necessary investment. Moreover, in order to obtain orphan designation in the EU, it is necessary to demonstrate that there exists no satisfactory method of diagnosis, prevention or treatment of the condition authorized for marketing in the EU, or if such a method exists, that the product will be of significant benefit to those affected by the condition.

The FDA has granted orphan drug designation for ELX-02 for the treatment of cystic fibrosis, MPS I and Rett syndrome. We may seek orphan drug designation for our other product candidates, and with respect to other indications. Generally, if a drug with an orphan drug designation subsequently receives the first FDA marketing approval for the indication for which it has such designation, the drug is entitled to a period of marketing exclusivity, which precludes the FDA from approving another marketing application for the same drug for the same indication for that time period. The applicable period is seven years in the U.S. Orphan drug exclusivity may be lost if the FDA or EMA determines that the request for designation was materially defective or if the manufacturer is unable to assure sufficient quantity of the drug to meet the needs of patients with the rare disease or condition.

Even if we obtain orphan drug exclusivity for a product candidate, that exclusivity may not effectively protect the candidate from competition because different drugs can be approved for the same condition. Even after an orphan drug is approved, the applicable regulatory authority can subsequently approve another drug for the same condition if it concludes that the later drug is clinically superior in that it is shown to be safer, more effective or makes a major contribution to patient care. Similarly, if our competitors are able to obtain orphan product exclusivity for their products in the same indications for which we are developing our product candidates, we may not be able to have our products approved by the applicable regulatory authority for a significant period of time.

Developments by competitors may render our products or technologies obsolete or non-competitive which would have a material adverse effect on our business, results of operations and financial condition.

We compete with fully integrated biopharmaceutical companies and smaller biopharmaceutical companies that are collaborating with larger pharmaceutical companies, academic institutions, government agencies and other public and private research organizations. Our product candidates will have to compete with existing therapies and potential therapies under development by our competitors. In addition, our commercial opportunities may be reduced or eliminated if our competitors develop and market products that are less expensive, more effective or safer than our product candidates. Other companies have product candidates in various stages of preclinical or clinical development to treat diseases for which we are also seeking to develop product candidates. Some of these potential competing drugs are further advanced in development than our product candidates and may be commercialized earlier. Even if we are successful in developing effective drugs, our products may not compete successfully with products produced by our competitors.

Most of our competitors, either alone or together with their collaborative partners, operate larger research and development programs, staff and facilities, and have substantially greater financial resources than we do, as well as significantly greater experience in:

- developing drugs;
- undertaking preclinical testing and human clinical trials;
- obtaining marketing approvals from the FDA and other regulatory authorities;
- formulating and manufacturing drugs; and
- launching, marketing and selling drugs.

These organizations also compete with us to attract qualified personnel, for acquisitions and joint venture candidates and for other collaborations.

Efforts to compete and the pursuit of activities of our competitors may impose unanticipated costs on our business, which would have a material adverse effect on our business, results of operations and financial condition.

If we are unable to develop and commercialize our product candidates, our business will be adversely affected.

A key element of our strategy is to develop and commercialize a portfolio of new products. We seek to do so through our internal research programs and strategic collaborations for the development of new products. Research programs to identify new product candidates require substantial technical, financial and human resources, whether or not any product candidates are ultimately identified. Our research programs may initially show promise in identifying potential product candidates, yet fail to yield product candidates for clinical development for many reasons, including:

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

Any failure to develop or commercialize any of our product candidates may have a material adverse effect on our business, results of operations and financial condition.

Risks Related to Our Financial Position and Need for Additional Capital

We have incurred significant 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 maintain profitability.

We have a history of net losses and negative cash flows from operating activities since inception, and as of September 30, 2020, had an accumulated deficit of $(165.5) million. Historically, we have financed our operations primarily through equity capital investments, and to a lesser extent from loans and grants. We have devoted substantially all of our financial resources and efforts to research and development. We expect that it will be several years, if ever, before we receive regulatory approval and have a product candidate ready for commercialization. We expect to continue to incur significant expenses and operating losses for the foreseeable future. Our net losses may fluctuate significantly from quarter to quarter and year to year. We anticipate that our expenses will increase substantially if and as we:

- advance ELX-02 and/or other product candidates further into clinical development;
- continue to experience delays in enrollment and completion of our clinical trials due to the COVID-19 pandemic or otherwise;
- continue the preclinical development of our research programs and advance candidates into clinical trials;
- pursue regulatory authorization to conduct clinical trials of additional product candidates;
- seek marketing approvals for our product candidates;

- establish a sales, marketing and distribution infrastructure to commercialize any product candidates for which we obtain marketing approval;
- maintain, expand and protect our intellectual property portfolio;
- hire additional clinical, regulatory, management and scientific personnel;
- add operational, financial and management information systems and personnel;
- acquire or in-license other product candidates and technologies; and
- operate as a public company.

We have never generated any revenue from product sales and may never be profitable. To become and remain profitable, we and our collaborators must develop and eventually commercialize one or more product candidates with significant market potential. This will require us to be successful in a range of challenging activities, including completing preclinical studies and clinical trials of our product candidates, obtaining marketing approval for these product candidates, manufacturing, marketing and selling those product candidates for which we may obtain marketing approval, securing coverage and reimbursement for those product candidates for which we may obtain marketing approval, and satisfying any post-marketing requirements. We may never succeed in these activities and, even if we do, may never generate revenue that is significant or large enough to achieve profitability. Our failure to become and remain profitable would decrease the value of the company and could impair our ability to raise capital, maintain our research and development efforts, expand our business or continue our operations. A decline in the value of the company could also cause investors to lose all or part of their investment.

We will need substantial additional funding. If we are unable to raise capital when needed, we would be forced to delay, reduce or eliminate our product development programs or commercialization efforts.

We expect our expenses to increase in connection with our ongoing activities, particularly as we continue the research and development of, continue and initiate clinical trials of, and seek marketing approval for ELX-02, and as we become obligated to make milestone payments pursuant to our outstanding license agreements. In addition, if we obtain marketing approval for any of our current or future product candidates, we expect to incur significant commercialization expenses related to sales, marketing, manufacturing and distribution of the approved product. Our future capital requirements will depend on many factors, including:

- the scope, progress, results and costs of drug discovery, clinical development, laboratory testing and clinical trials for ELX-02 and other product candidates;
- the costs, timing and outcome of any regulatory review of ELX-02 and other product candidates;
- the cost of any other product candidate programs we pursue;
- the costs and timing of commercialization activities, including manufacturing, marketing, sales and distribution, and securing coverage and reimbursement for any product candidates that receive marketing approval;
- the costs of preparing, filing and prosecuting patent applications, maintaining and enforcing our intellectual property rights and defending intellectual property-related claims;
- our ability to establish and maintain collaborations on favorable terms, if at all; and
- the extent to which we acquire or in-license other product candidates and technologies.

Identifying potential product candidates and conducting preclinical studies and clinical trials are time consuming, expensive and uncertain processes that take years to complete, and we may never generate the necessary data or results required to obtain marketing approval or achieve product sales for any of our current or future product candidates. In addition, our product candidates, if approved, may not achieve commercial success. Our commercial revenue, if any, will be derived from sales of products that we do not expect to be commercially available for several years, if at all.

Accordingly, despite our prior public equity offerings and debt financing, we will need substantial additional funding in connection with our continuing operations and to achieve our goals. However, our existing cash and cash equivalents may prove to be insufficient for these activities. If we are unable to raise capital when needed or on attractive terms, we would be forced to delay, reduce or eliminate our research and development programs, product portfolio expansion or future commercialization efforts. Adequate additional financing may not be available to us on acceptable terms, or at all. In

addition, we may seek additional financing due to favorable market conditions or strategic considerations, even if we believe we have sufficient funds for our operating plans.

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

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

Risks Related to Our Business and Operations

Maintaining and improving our financial controls and the requirements of being a public company may strain our resources, divert management's attention and affect our ability to attract and retain qualified board members.

The trading market for our common stock is influenced by the research and reports that securities or industry analysts publish. As a public company, we are subject to the reporting requirements of the Securities Exchange Act of 1934, as amended, or the Exchange Act, the Sarbanes-Oxley Act of 2002, or the Sarbanes-Oxley Act, and Nasdaq stock market rules. The requirements of these rules and regulations have increased and will continue to significantly increase our legal and financial compliance costs, including costs associated with the hiring of additional personnel, making some activities more difficult, time-consuming or costly, and may also place undue strain on our personnel, systems and resources. The Exchange Act requires, among other things, that we file annual, quarterly and current reports with respect to our business and financial condition.

The Sarbanes-Oxley Act requires, among other things, that we maintain disclosure controls and procedures and internal control over financial reporting. Ensuring that we have adequate internal financial and accounting controls and procedures in place, as well as maintaining these controls and procedures, is a costly and time-consuming effort that needs to be re-evaluated frequently. Section 404 of the Sarbanes-Oxley Act, or Section 404, requires that we annually evaluate our internal control over financial reporting to enable management to report on the effectiveness of those controls. In connection with the Section 404 requirements, we test our internal controls and could, as part of that documentation and testing, identify material weaknesses, significant deficiencies or other areas for further attention or improvement.

Implementing any appropriate changes to our internal controls may require specific compliance training for our directors, officers and employees, require the hiring of additional finance, accounting and other personnel, entail substantial costs to modify our existing accounting systems, and take a significant period of time to complete. These changes may not, however, be effective in maintaining the adequacy of our internal controls, and any failure to maintain that adequacy, or consequent inability to produce accurate financial statements on a timely basis, could increase our operating costs and could materially impair our ability to operate our business. Moreover, adequate internal controls are necessary for us to produce reliable financial reports and are important to help prevent fraud. As a result, our failure to satisfy the requirements of Section 404 on a timely basis could result in the loss of investor confidence in the reliability of our financial statements, which in turn could cause the market value of our common stock to decline. Our ability to maintain effective internal controls over financial reporting could be more difficult due to the measures imposed by municipalities in efforts to combat the COVID-19 pandemic, such as quarantines, containment zones and limitations on travel, which may limit the availability of employees and other personnel necessary to adequately monitor and oversee the effectiveness of our internal controls.

Various rules and regulations applicable to public companies make it more difficult and more expensive for us to maintain directors' and officers' liability insurance, and we may be required to accept reduced coverage or incur substantially higher costs to maintain coverage. If we are unable to maintain adequate directors' and officers' liability insurance, our

ability to recruit and retain qualified officers and directors, especially those directors who may be deemed independent for purposes of the Nasdaq stock market rules, will be significantly curtailed.

We are seeking to expand our business through strategic initiatives. Our efforts to identify opportunities or complete transactions that satisfy our strategic criteria may not be successful, and we may not realize the anticipated benefits of any completed acquisition, collaboration or other strategic transaction.

Our business strategy includes expanding our product candidates and capabilities. We regularly evaluate potential merger, acquisition, partnering and in-license opportunities that we expect will expand our pipeline or product offerings, and enhance our research or development programs.

We may engage in strategic transactions that could cause us to incur additional liabilities, commitments or significant expense. Any such transactions will be dependent on our ability to appropriately evaluate the potential risks and uncertainties, integrate any new technology, product and/or business, and generate revenues (including through up-front payments, milestones and/or royalties) sufficient to meet our underlying objectives.

Any strategic transaction undertaken may result in unforeseen development costs, timeline delays, regulatory approval challenges and uncertainties relating to the commercial market opportunity, any of which could cause us to fail to realize the anticipated value of the transaction and may have a material adverse effect on our business and financial condition.

To manage effectively our current and future potential growth, we must also continue to enhance and develop our global employee base, and our operational and financial processes. Supporting our growth strategy will require significant capital expenditures and management resources, including investments in research, development, sales and marketing, manufacturing and other areas of our operations. The development or expansion of our business, any acquired business or any acquired or in-licensed products may require a substantial capital investment by us. We may not have these necessary funds, or they might not be available to us on acceptable terms or at all. We may also seek to raise funds by selling shares of our capital stock, or securities convertible into our capital stock, which could dilute current stockholders' ownership interest in our company.

Our business could be affected by litigation, government investigations and enforcement actions.

We operate in many jurisdictions in a highly regulated industry and we could be subject to litigation, government investigation and enforcement actions on a variety of matters in the U.S. or foreign jurisdictions, including, without limitation, intellectual property, regulatory, product liability, environmental, whistleblower, Qui Tam, false claims, privacy, anti-kickback, anti-bribery, securities, commercial, employment, and other claims and legal proceedings which may arise from conducting our business. Any of these actions or proceedings may result in significant costs, fines, penalties or imposition of burdensome restrictions on the company, any of which could have a material adverse effect on our business, results of operations and financial condition.

Comprehensive tax reform bills could adversely affect our business and financial condition.

On December 22, 2017, and effective January 1, 2018, the U.S. government enacted H.R. 1, "An Act to provide for reconciliation pursuant to titles II and V of the concurrent resolution on the budget for fiscal year 2018" (informally titled the "Tax Cuts and Jobs Act", or TCJA), which includes significant changes to the taxation of business entities. The TCJA, among other things, contains significant changes to corporate taxation, including reduction of the corporate tax rate from a top marginal rate of 35% to a flat rate of 21%, limitation of the tax deduction for interest expense to 30% of adjusted earnings (except for certain small businesses), implementation of a "base erosion anti-abuse tax" which requires U.S. corporations to make an alternative determination of taxable income without regard to tax deductions for certain payments to affiliates, taxation of certain non-U.S. corporations' earnings considered to be "global intangible low taxed income" (also referred to as "GILTI"), repeal of the alternative minimum tax, or AMT, for corporations and changes to a taxpayer's ability to either utilize or refund the AMT credits previously generated, changes in the attribution rules relating to shareholders of certain "controlled foreign corporations", limitation of the deduction for net operating losses to 80% of current year taxable income and elimination of net operating loss carrybacks, one time taxation of offshore earnings at reduced rates regardless of whether they are repatriated, elimination of U.S. tax on foreign earnings (subject to certain important exceptions), immediate deductions for certain new investments instead of deductions for depreciation expense over time, and modifying or repealing many business deductions and credits. In response to the COVID-19 pandemic, the CARES Act was signed into law in March 2020. The CARES Act lifts certain deduction limitations originally imposed by the TCJA. Corporate taxpayers may carry back net operating losses (NOLs) originating during 2018 through 2020 for up to five years, which was not previously allowed. The CARES Act also eliminates the 80% of taxable income limitations by allowing corporate entities to fully utilize

NOL carryforwards to offset taxable income in 2018, 2019 or 2020. Taxpayers may generally deduct interest up to the sum of 50% of adjusted taxable income plus business interest income (30% limit under the TCJA) for tax years beginning January 1, 2019 and 2020. The CARES Act allows taxpayers with alternative minimum tax credits to claim a refund in 2020 for the entire amount of the credits instead of recovering the credits through refunds over a period of years, as originally enacted by the TCJA.

Notwithstanding the reduction in the corporate income tax rate, the TCJA remains subject to interpretation and further guidance from U.S. taxing authorities and as a result the overall impact of this tax reform is uncertain and may change due to interpretation changes, and our business and financial condition could be adversely affected. In addition, it is uncertain if and to what extent various U.S. states will conform their tax laws to the TCJA. The impact of the TCJA on holders of our common stock is also uncertain and could be adverse. We are unable to predict what tax reform may be proposed or enacted in the future or what effect such changes would have on our business, but such changes, to the extent they are brought into tax legislation, regulations, policies or practices, could affect our effective tax rates in the future in countries where we have operations and have an adverse effect on our overall tax rate in the future, along with increasing the complexity, burden and cost of tax compliance. We urge our stockholders to consult with their legal and tax advisors with respect to the TCJA and the CARES Act and the potential tax consequences of investing in or holding our common stock.

Our ability to use our net operating losses to offset future taxable income may be subject to certain limitations.

As of December 31, 2019, we had U.S. federal and state net operating loss, or "NOL", carryforwards of $99.4 million and $31.9 million, respectively, and federal research tax credit carryforwards of $3.0 million. Certain U.S. NOL carryforwards will begin to expire, if not utilized, beginning in 2020 through 2037, and the research tax credits will expire beginning in 2027 through 2037. Included in these U.S. federal NOL carryforwards are $23.5 million of NOLs generated after the effective date of the TCJA which are not subject to expiration. Under the TCJA, federal NOLs generated in 2018 and future years may be carried forward indefinitely but may not be carried back and are only eligible to offset up to a maximum of 80% of taxable income generated in a given year. It is uncertain if and to what extent various U.S. states will conform their net operating loss rules to the TCJA.

In general, under Section 382 of the United States Internal Revenue Code of 1986, as amended, or the Code, a corporation that undergoes an "ownership change" is subject to limitations on its ability to utilize its pre-ownership change NOLs to offset future taxable income. We may have experienced ownership changes in the past, including in connection with the Reverse Merger on December 19, 2017 at which time our pre-change U.S. federal NOL carryforward was $77.2 million and research tax credit was $0.7 million. We may experience additional ownership changes in the future as a result of subsequent shifts in our stock ownership, some of which may be outside of our control. Although we have not completed our analysis, it is reasonably possible that our federal NOLs available to offset future taxable income could materially decrease. This reduction will be offset by an adjustment to the existing valuation allowance for an equal and offsetting amount. Additionally, our state NOLs available to offset future state income could similarly decrease which would also be offset by an equal and offsetting adjustment to the existing valuation allowance. Given the offsetting adjustments to the existing valuation allowance, any ownership change is not expected to have an adverse material effect on our Consolidated Financial Statements. Finally, as of December 31, 2019, we had Israeli NOL carryforwards of $73.7 million, which carry forward indefinitely.

Our ability to utilize our NOLs is dependent on attaining profitability sufficient to offset such available NOLs prior to their expiration. In addition, we may not be able to utilize a portion of the NOLs reflected on our balance sheet, even if we attain profitability.

We could be subject to additional tax liabilities.

We are subject to federal, state and local taxes in the U.S. and Israel. Significant judgment is required in evaluating our tax positions and our worldwide provision for taxes. During the ordinary course of business, there are many activities and transactions for which the ultimate tax determination is uncertain. In addition, our tax obligations and effective tax rates could be adversely affected by changes in the relevant tax, accounting and other laws, regulations, principles and interpretations, including those relating to income tax nexus, by our earnings being lower than anticipated in jurisdictions where we have lower statutory rates and higher than anticipated in jurisdictions where we have higher statutory rates, by changes in foreign currency exchange rates, or by changes in the valuation of our deferred tax assets and liabilities. We may be audited in various jurisdictions, and such jurisdictions may assess additional taxes against us. Although we believe our tax estimates are reasonable, the final determination of any tax audits or litigation could be materially different from our historical tax provisions and accruals, which could have a material adverse effect on our operating results or cash flows in the period or periods for which a determination is made.

Changes in healthcare laws and implementing regulations, as well as changes in healthcare policy, may affect coverage and reimbursement of our product candidates in ways that we cannot currently predict, and these changes could adversely affect our business and financial condition.

In the U.S., a number of legislative and regulatory initiatives have focused on containing the cost of healthcare. The Patient Protection and Affordable Care Act, or PPACA, was enacted in March 2010. This law substantially changed the way healthcare is financed by both governmental and private insurers in the U.S., and significantly impacts the pharmaceutical industry. PPACA contains a number of provisions that are expected to impact our business and operations, in some cases in ways we cannot currently predict. Changes that may affect our business include those governing enrollment in federal healthcare programs, reimbursement changes, rules regarding prescription drug benefits under health insurance exchanges, expansion of the 340B program, expansion of state Medicaid programs, fraud and abuse enforcement and rules governing the approval of biosimilar products. These changes will impact existing government healthcare programs and will result in the development of new programs, including Medicare payment for performance initiatives and improvements to the physician quality reporting system and feedback program. In early 2016, CMS issued final regulations to implement the changes to the Medicaid Drug Rebate Program under PPACA. These regulations became effective on April 1, 2016. Moreover, in the future, Congress could enact legislation that further increases Medicaid drug rebates or other costs and charges associated with participating in the Medicaid Drug Rebate Program. Legislative changes to the PPACA also remain possible and appear likely under the current administration. The issuance of regulations and coverage expansion by various governmental agencies relating to the Medicaid Drug Rebate Program has increased and will continue to increase our costs and the complexity of compliance, has been and will be time-consuming, and could have a material adverse effect on our results of operations.

Governments in countries where we operate have adopted or have shown significant interest in pursuing legislative initiatives to reduce costs of healthcare. We expect that the implementation of current laws and policies, the amendment of those laws and policies in the future, as well as the adoption of new laws and policies, could have a material adverse effect on our industry generally and on our ability to generate or increase future product sales, if any, or successfully commercialize our product candidates, or could limit or eliminate our future spending on development projects. In many cases, these government initiatives, even if enacted into law, are subject to future rulemaking by regulatory agencies. Although we have evaluated these government initiatives and the impact on our business, we cannot know with certainty whether any such law, rule or regulation will adversely affect coverage and reimbursement of our product candidates, or to what extent, until such laws, rules and regulations are promulgated, implemented and enforced, which could sometimes take many years. The announcement or adoption of regulatory or legislative proposals could delay or prevent our entry into new markets, affect our reimbursement or sales in the markets where we are already selling our approved products, if any, and materially harm our business, financial condition and results of operations.

Our business could be adversely affected by the effects of widespread public health epidemics and other factors beyond our control.

Public health epidemics or widespread outbreaks of contagious diseases could adversely impact our business. Any outbreak of contagious diseases, and other adverse public health developments, such as the recent novel strain of coronavirus (COVID-19), initially limited to a region in China and now affecting the global community, could impact our operations depending on future developments, which are highly uncertain, largely beyond our control and cannot be predicted with certainty. These uncertain factors include the duration of the outbreak, new information which may emerge concerning the severity of the disease and the actions to contain or treat its impact, could adversely impact our operations, including among others, conduct of our clinical trials, employee mobility and productiveness, temporary closure of facilities, including clinical trial sites, our manufacturing capabilities, and third party service providers such as CROs, any of which could have an adverse impact on our business and our financial results. On March 25, 2020, we announced that enrollment in our clinical trials had been paused temporarily in response to the COVID-19 pandemic in order to avoid unnecessary exposure in at-risk populations, to maintain the integrity of our study data and to support global healthcare providers in their commitment to ensure patient safety. On June 17, 2020, we announced that enrollment in our Phase 2 clinical trial in cystic fibrosis had resumed in Israel and Europe, and on August 12, 2020, we announced that enrollment in our Phase 2 clinical trial in cystic fibrosis had resumed in the U.S. COVID-19 is continuing to evolve and we continue to work closely with our clinical sites and investigators. While we remain committed to completing enrollment in these Phase 2 proof of concept clinical trials and reporting top line data in the first half of 2021, we cannot provide assurances as to when this will be accomplished or whether we will incur significant additional costs, expend additional resources or be subject to additional regulatory requirements, including COVID-19 related disruptions, any of which may have a material adverse impact on our financial condition and results of operations.

We may be subject to numerous and varying privacy and security laws, and our failure to comply could result in penalties and reputational damage.

We are subject to laws and regulations covering data privacy and the protection of personal information including health information. The legislative and regulatory landscape for privacy and data protection continues to evolve, and there has been an increasing focus on privacy and data protection issues which may affect our business. In the U.S., we may be subject to state security breach notification laws, state health information privacy laws and federal and state consumer protections laws which impose requirements for the collection, use, disclosure and transmission of personal information. Each of these laws is subject to varying interpretations by courts and government agencies, creating complex compliance issues for us. If we fail to comply with applicable laws and regulations, we could be subject to penalties or sanctions, including criminal penalties if we knowingly obtain individually identifiable health information from a covered entity in a manner that is not authorized or permitted by HIPAA or for aiding and abetting the violation of HIPAA.

Numerous other countries have also developed, or are developing, laws governing the collection, use and transmission of personal information. EU member states and other jurisdictions have adopted data protection laws and regulations, which impose significant compliance obligations. For example, in May 2016, the EU formally adopted the General Data Protection Regulation, or GDPR, which applies to all EU member states as of May 25, 2018 and replaces the former EU Data Protection Directive. The regulation introduces new data protection requirements in the EU and imposes substantial fines for breaches of the data protection rules. The GDPR must be implemented into national laws by the EU member states and imposes strict obligations and restrictions on the ability to collect, analyze, and transfer personal data, including health data from clinical trials and adverse event reporting. Data protection authorities from different EU member states have interpreted the privacy laws differently, which adds to the complexity of processing personal data in the EU, and guidance on implementation and compliance practices are often updated or otherwise revised. Any failure to comply with the rules arising from the GDPR and related national laws of EU member states could lead to government enforcement actions and significant penalties against us, and adversely impact our operating results. The GDPR will increase our responsibility and liability in relation to personal data that we process and we may be required to put in place additional mechanisms ensuring compliance with EU data protection rules.

Security breaches, cyber-attacks, or other disruptions could expose us to liability and affect our business and reputation.

We are increasingly dependent on our information technology systems and infrastructure for our business. We collect, store, and transmit sensitive information including intellectual property, proprietary business information and personal information in connection with business operations. The secure maintenance of this information is critical to our operations and business strategy. Some of this information could be an attractive target of criminal attack by third parties with a wide range of motives and expertise, including organized criminal groups, “hacktivists,” patient groups, disgruntled current or

former employees, and others. Cyber-attacks are of ever-increasing levels of sophistication, and despite our security measures, our information technology and infrastructure may be vulnerable to such attacks or may be breached, including due to employee error or malfeasance. We have also implemented information security measures to protect patients' personal information against the risk of inappropriate and unauthorized external use and disclosure. The COVID-19 pandemic has caused us to modify our business practices, including permitting our employees to work from home. As a result, we are increasingly dependent upon our technology systems to operate our business and our ability to effectively manage our business depends on the security, reliability and adequacy of our technology systems and data, which includes use of cloud technologies. This increased remote usage of information systems increases the risks that our business may be disrupted due to a variety of reasons, including security breaches, power outages, unavailability of employees, use of non-company secured equipment and increased phishing and hack activity. However, despite these measures, and due to the ever-changing information cyber-threat landscape, we may be subject to data breaches through cyber-attacks. Any such breach could compromise our networks and the information stored there could be accessed, publicly disclosed, lost or stolen. If our systems become compromised, we may not promptly discover the intrusion. Like other companies in our industry, we have experienced attacks to our data and systems, including malware and computer viruses. If our systems failed or were breached or disrupted, patient and other data and information may become compromised, we could lose sales for approved products, if any, and suffer reputational damage and loss of confidence by patients, investors and business partners. Such incidents would result in notification obligations to affected individuals and government agencies, legal claims or proceedings, and liability under federal and state laws that protect the privacy and security of personal information. Any one of these events, or similar events occurring through one of our vendors that maintain such information on our behalf, could cause our business to be materially harmed and our results of operations to be adversely impacted.

We rely on third parties to conduct some or all aspects of our product manufacturing, protocol development, research and preclinical and clinical testing, and these third parties may not perform satisfactorily.

We do not expect to independently conduct all aspects of our product manufacturing, protocol development, research and preclinical and clinical testing. We currently rely, and expect to continue to rely, on third parties with respect to these items.

Any of these third parties may terminate their engagements with us at any time. If we need to enter into alternative arrangements, it could delay our product development activities. Our reliance on these third parties for research and development activities will reduce our control over these activities but will not relieve us of our responsibility to ensure compliance with all applicable laws and regulations and study protocols. If these third parties do not successfully carry out their contractual duties, meet expected deadlines or conduct our studies in accordance with regulatory requirements or our stated study plans and protocols, we will not be able to complete, or may be delayed in completing, the preclinical studies and clinical trials required to support future NDA submissions and approval of our product candidates.

Reliance on third-party manufacturers, testing sites, and investigators entails risks to which we would not be subject if we developed, researched, tested, and manufactured the product candidates ourselves, including:

- the inability to negotiate manufacturing, testing, and research agreements with third parties under commercially reasonable terms;
- reduced control as a result of using third-party manufacturers, testing laboratories, and research sites and investigators for all aspects of manufacturing, testing, and research activities;
- termination or nonrenewal of manufacturing, testing, or research agreements with third parties in a manner or at a time that is costly or damaging to us; and
- disruptions to the operations of our third-party manufacturers or suppliers, testing facilities, or research sites caused by conditions unrelated to our business or operations, including unrelated regulatory action against or the bankruptcy of the manufacturer or supplier, testing facility, or research site, or the unavailability of essential personnel to conduct or complete our research or clinical trials, such as, for example, a result of the COVID-19 pandemic.

Any of these events could lead to clinical trial delays, failure to obtain regulatory approval or impact our ability to successfully commercialize future products. Some of these events could be the basis for FDA action, including injunction, recall, seizure or total or partial suspension of production or testing. Any one of these events could cause our business to be materially harmed and our results of operations would be adversely impacted.

Our future success depends on our ability to retain key employees, consultants and advisors and to attract, retain and motivate qualified personnel.

The success of our business is dependent in large part on our continued ability to attract and retain our senior management, and other highly qualified personnel in our scientific, clinical, manufacturing and commercial organizations. Intense competition exists in the biopharmaceutical industry for these types of personnel. Our business is specialized and global and we must attract and retain highly qualified individuals across many geographies. We may not be able to continue to attract and retain the highly qualified personnel necessary for developing, manufacturing and commercializing our product candidates. If we are unsuccessful in our recruitment and retention efforts, or if our recruitment efforts take longer than anticipated, our business may be harmed. We may face difficulty in attracting and retaining key talent for a number of reasons, including management changes, the underperformance or discontinuation of one or more late stage programs, recruitment by competitors or delays in the recruiting and hiring process as a result of the COVID-19 pandemic. We cannot ensure that we will be able to hire or retain the personnel necessary for our operations or that the loss of any such personnel will not have a material impact on our financial condition and results of operations.

We are highly dependent on principal members of our senior management. While we have entered into employment agreements or offer letters with each of our executive officers, any of them could leave our employment at any time, as all of our employees are "at will" employees. Recruiting and retaining other qualified employees, consultants and advisors for our business, including scientific and technical personnel, will also be critical to our success. Competition for skilled personnel is intense and the turnover rate can be high. We may not be able to attract and retain personnel on acceptable terms given the competition among numerous pharmaceutical and biotechnology companies for individuals with similar skill sets. In addition, failure to succeed in preclinical studies or clinical trials may make it more challenging to recruit and retain qualified personnel. The inability to recruit or loss of the services of any executive, key employee, consultant or advisor may impede the progress of our research, development and commercialization objectives. If we fail to attract and retain highly qualified personnel, we may not be able to successfully develop, manufacture or commercialize our product candidates.

We have experienced recent changes in management and other key personnel in critical functions across our organization. Changes in management and other key personnel have the potential to disrupt our business, and any such disruption could adversely affect our operations, programs, growth, financial condition or results of operations. In addition, new members of management may have different perspectives on programs and opportunities for our business, which may cause us to focus on new business opportunities or reduce or change emphasis on our existing business programs. Further, if members of our management and other key personnel in critical functions across our organization are unable to perform their duties or have limited availability due to COVID-19, we may not be able to execute on our business strategy and/or our operations may be negatively impacted.

Risks Related to Intellectual Property

If we fail to adequately protect or enforce our intellectual property rights or secure rights to third party patents, the value of our intellectual property rights would diminish, and our business, competitive position and results of operations would suffer.

As of September 30, 2020, we owned or licensed 22 issued patents and 54 pending patent applications in the U.S. and abroad, not including U.S. provisional applications. However, with regard to the pending provisional applications, the filing of a patent application does not mean that we will be issued a patent, or that any patent eventually issued will be as broad as requested in the patent application or sufficient to protect our technology. Any modification required to a currently pending patent application may delay the approval of such patent application which could have a material adverse effect on our business, results of operations and financial condition. In addition, there are a number of factors that could cause our current or future issued patents to become invalid or unenforceable or that could cause our pending patent applications to not be granted, including known or unknown prior art, deficiencies in the patent application or lack of originality of the technology. Our competitive position and future revenue will depend in part on our ability and the ability of our licensors and collaborators to obtain and maintain patent protection for our product candidates, methods, processes and other technologies, to preserve our trade secrets, to prevent third parties from infringing on our proprietary rights and to operate without infringing the proprietary rights of third parties. However, we cannot predict:

- the degree and range of protection any patents will afford us against competitors and those who infringe upon our patents, including whether third parties will find ways to invalidate or otherwise circumvent our licensed patents;
- if and when patents will issue;

- whether or not others will obtain patents claiming aspects similar to those covered by our owned or licensed patents and patent applications; or
- whether we will need to initiate litigation or administrative proceedings, which may be costly, and whether we win or lose.

If patent rights covering our products or technologies are not sufficiently broad, they may not provide us with sufficient proprietary protection or competitive advantages against competitors with similar products and technologies. Furthermore, if the U.S. Patent and Trademark Office or foreign patent offices issue patents to us or our licensors, others may challenge the patents or circumvent the patents, or the patent office or the courts may invalidate the patents. Thus, any patents we own or license from or to third parties may not provide any protection against our competitors and those who infringe upon our patents.

Furthermore, the lives of our patents are limited. With regard to our lead compound ELX-02, patents that have issued or that may issue in the future from our primary composition of matter patent family are currently set to expire in 2031. We have pending patent families directed to specific methods of using and manufacturing ELX-02, and any patents that may issue from these families would be expected to expire in 2035 and 2038, respectively. However, these applications may not issue, and even if they do issue the resultant patents may not provide adequate coverage to meaningfully block competitors from launching their products. We will likely pursue additional patent protection relating to ELX-02 in the future, including for example additional methods of use or manufacture, specific formulations, or combinations of ELX-02 with other therapeutic agents. However, as with our pending patent families, any applications we file in the future may not issue or may not result in adequate coverage to adequately protect our assets.

Depending upon the timing, duration, and conditions of any FDA marketing approval for ELX-02, one or more of our patents may be eligible for patent term extension of up to five years under the Hatch-Waxman Act. However, we may not receive an extension if we fail to exercise due diligence during the testing phase or regulatory review process, fail to apply for an extension within applicable deadlines, or otherwise fail to satisfy applicable requirements. Moreover, the length of the extension could be less than we request. Only one patent per approved product can be extended, the extension cannot extend the total patent term beyond 14 years from approval and only those claims covering the approved drug, an approved method of using the approved drug, or a method of manufacturing the approved drug may be extended. If we are unable to obtain patent term extension or the term of any such extension is less than we request, the period during which we can enforce our patent rights for ELX-02 will be shortened and our competitors may obtain approval to market competing products sooner. As a result, our revenue from applicable products could be reduced. Further, if this occurs, our competitors may take advantage of our investment in development and trials by referencing our clinical and preclinical data and launch their product earlier than might otherwise be the case, and our business could be harmed.

If we cannot obtain new patents, maintain our existing patents and protect the confidentiality and proprietary nature of our trade secrets and other intellectual property, our business and competitive position may be harmed.

Our success will depend in part on our ability to obtain and maintain patent and regulatory protections for our product candidates, to preserve our trade secrets and other proprietary rights, to operate without infringing the proprietary rights of third parties, and to prevent third parties from circumventing our rights. Due to the time and expense of bringing new product candidates through development and regulatory approval to the marketplace, there is particular importance in obtaining patent and trade secret protection for significant new technologies, products and processes.

We have and may in the future obtain patents or the right to practice patents through ownership or license. Our patent applications may not result in the issue of patents in the U.S. or other countries. Our patents may not afford adequate protection for our products. Third parties may challenge our patents. If any of our patents are narrowed, invalidated or become unenforceable, competitors may develop and market products similar to ours that do not conflict with or infringe our patents rights, which could have a material adverse effect on our financial condition. We may also finance and collaborate in research conducted by government organizations, hospitals, universities or other educational or research institutions. Such research partners may be unwilling to grant us exclusive rights to technology or products developed through such collaborations. There is also a risk that disputes may arise as to the rights to technology or products developed in collaboration with other parties. Our product candidates are expensive and time-consuming to test and develop. Even if we obtain and maintain patents, our business may be significantly harmed if the patents are not broad enough to protect our products from copycat products.

Significant legal questions exist concerning the extent and scope of patent protection for biopharmaceutical products and processes in the U.S. and elsewhere. Accordingly, there is no certainty that patent applications owned or licensed by us

will issue as patents, or that our issued patents will afford meaningful protection against competitors. Once issued, patents are subject to challenge through both administrative and judicial proceedings in the U.S. and other countries. Such proceedings include re-examinations, inter partes reviews, post-grant reviews and interference proceedings before the U.S. Patent and Trademark Office, as well as opposition proceedings before the European Patent Office and other non-U.S. patent offices. Litigation may be required to enforce, defend or obtain our patent and other intellectual property rights. Any administrative proceeding or litigation could require a significant commitment of our resources and, depending on outcome, could adversely affect the scope, validity or enforceability of certain of our patent or other proprietary rights.

In addition, our business requires using sensitive technology, techniques and proprietary compounds that we protect as trade secrets. However, we may also rely heavily on collaboration with, or discuss the potential for collaboration with, suppliers, outside scientists and other biopharmaceutical companies. Collaboration and discussion of potential collaboration present a strong risk of exposing our trade secrets. If our trade secrets were exposed, it would help our competitors and adversely affect our business prospects.

If we are found to be infringing on patents owned by others, we may be forced to pay damages to the patent owner and/or obtain a license to continue the manufacture, sale or development of our product candidates. If we cannot obtain a license, we may be prevented from the manufacture, sale or development of our product candidates, which would adversely affect our business.

If we infringe the rights of third parties, we could be prevented from selling products, forced to pay damages and required to defend against litigation which could result in substantial costs and may have a material adverse effect on our business, results of operations and financial condition.

We have not received to date any claims of infringement by any third parties. However, as our product candidates progress into clinical trials and commercialization, if at all, our public profile and that of our product candidates may be raised and generate such claims. Defending against such claims, and occurrence of a judgment adverse to us, could result in unanticipated costs and may have a material adverse effect on our business and competitive position. If our products, methods, processes and other technologies infringe the proprietary rights of other parties, we may incur substantial costs and we may have to:

- obtain licenses, which may not be available on commercially reasonable terms, if at all;
- redesign our products or processes to avoid infringement, which could significantly impede development and impair or block our ability to secure regulatory approval of any redesigned product or process;
- stop using the subject matter claimed in the patents held by others, which could cause us to lose the use of one or more of our product candidates;
- defend litigation or administrative proceedings that may be costly whether we win or lose, and which could result in a substantial diversion of management resources; or
- pay damages.

Any costs incurred in connection with such events or the inability to develop or sell our products may have a material adverse effect on our business, results of operations and financial condition.

We rely on confidentiality agreements that could be breached and may be difficult to enforce which could have a material adverse effect on our business and competitive position.

Our policy is to enter agreements relating to the non-disclosure of confidential information with third parties, including our contractors, consultants, advisors and research collaborators, as well as agreements that purport to require the disclosure and assignment to us of the rights to the ideas, developments, discoveries and inventions of our employees and consultants while we employ them. However, these agreements can be difficult and costly to enforce. Moreover, to the extent that our contractors, consultants, advisors and research collaborators apply or independently develop intellectual property in connection with any of our projects, disputes may arise as to the proprietary rights to the intellectual property. If a dispute arises, a court may determine that the rights belong to a third party, and enforcement of our rights can be costly and unpredictable. In addition, we rely on trade secrets and proprietary know-how that we seek to protect in part by confidentiality agreements with our employees, contractors, consultants, advisors and other third parties. Despite the protective measures we employ, we still face the risk that:

- these agreements may be breached;
- these agreements may not provide adequate remedies for the applicable type of breach; or
- our trade secrets or proprietary know-how will otherwise become known.

Any breach of our confidentiality agreements or our failure to effectively enforce such agreements may have a material adverse effect on our business and competitive position.

If we cannot meet requirements under our license agreement, we could lose the rights to our product candidates, which could have a material adverse effect on our business.

We depend on the license agreement with TRDF to maintain the intellectual property rights to certain of our product candidates. Our license agreement requires us to make payments and satisfy performance obligations in order to maintain our rights under this agreement. This agreement lasts either throughout the life of the patents that are the subject of the agreement, or with respect to other licensed technology, for a number of years after the first commercial sale of the relevant product.

In addition, we are responsible for the cost of filing and prosecuting certain patent applications and maintaining certain issued patents licensed to us. If we do not meet our obligations under our license agreement in a timely manner, we could lose the rights to our proprietary technology, which could have a material adverse effect on our business, results of operations and financial condition.

Risks Related to Our Regional Operations

Potential political and economic instability in regions where we conduct business may adversely affect our results of operations.

In addition to our operations in the United States, we currently conduct certain research and clinical development activities through our regional operations located in Israel, and may, in the future, expand operations to other regional locations in Europe and elsewhere as circumstances require. Accordingly, political and economic conditions in Israel and the surrounding region in particular, may directly affect our operations. Regional instability may lead to a deterioration in the political and trade relationships that exist between countries in the region, making it more difficult to conduct operations.

In addition, our insurance does not cover losses that may occur as a result of an event associated with the security situation in the Middle East or for any resulting disruption in our operations. Although the Israeli government has in the past covered the reinstatement value of direct damages that were caused by terrorist attacks or acts of war, we cannot provide assurance that this government coverage will be maintained or, if maintained, will be sufficient to compensate us fully for damages incurred.

Furthermore, in the past, Israel and Israeli companies have been subjected to economic boycotts. Several countries still restrict business with Israel and with Israeli companies. These restrictive laws and policies, even though we are a U.S.-based company, may have an adverse impact on our operating results, financial conditions or the expansion of our business.

We received Israeli government grants for our research and development activities and programs. The terms of such grants may require us, in the future, to pay royalties and under certain circumstances, penalties in addition to payment of royalties.

Our research and development efforts were initially financed, in part, through royalty-bearing grants from the Israel Innovation Authority, or IIA. We received an aggregate of approximately $2.6 million from the IIA for the development of our technologies. With respect to such grants we are required to pay certain royalties (including accrued LIBOR interest) up to approximately $2.7 million. We are required to comply with the requirements of the Israeli Encouragement of Research, Development and Technological Innovation in the Industry Law, 5744-1984, as amended, and related regulations, or the R&D Law, with respect to these past grants. If we fail to comply with the R&D Law, we may be required to refund certain grants previously received and/or to pay interest and penalties and we may become subject to criminal charges.

With respect to such grants we are obligated to pay royalties at a rate of 3% to 6% from the revenue generated from the sale of any products or services developed using IIA grants up to a maximum amount equal to repayment of the grant proceeds received plus accrued interest. We have not commenced the payment obligation of these royalties since we have not yet generated revenue, and we have a contingent obligation with respect to such future royalty payments including LIBOR interest, in the amount of approximately $2.7 million.

The R&D Law and terms of the prior grants restrict the transfer of certain know-how, and the transfer of manufacturing or manufacturing rights of products developed with grant funds, outside of Israel, without the prior approval of the IIA. Therefore, if aspects of our technologies are deemed to have been developed with IIA funding according to the R&D Law, the discretionary approval of the IIA may be required for any assignment and/or transfer to third parties inside or outside of Israel of know-how or transfer outside of Israel of manufacturing or manufacturing rights and may result in payment of increased royalties and/or payment of additional amounts to the IIA. Furthermore, the IIA may impose certain conditions on any arrangement under which it permits us to transfer technology or development outside of Israel. Such approvals may not be granted by the IIA and any conditions imposed may not be acceptable to the Company.

The R&D Law and the regulations promulgated thereunder provide that the transfer of IIA-supported technology or know-how outside of Israel may involve the payment of additional amounts depending upon the value of the transferred technology or know-how, the amount of IIA support, the time of completion of the IIA-supported research project and other factors, up to a maximum of six times the amount of grants received. These restrictions and requirements for payment may impair our ability to sell our technology assets outside of Israel or to outsource or transfer development or manufacturing activities with respect to any product or technology outside of Israel. Furthermore, the consideration available to our stockholders in a transaction involving the transfer outside of Israel of technology or know-how developed with IIA funding may be reduced by any amounts that we are required to pay to the IIA. Our obligations and limitations pursuant to the R&D Law are not limited in time and may not be terminated by us at will. As of the date hereof, we have not been required to pay any royalties with respect to the IIA grants.

We may become subject to claims for remuneration or royalties for assigned service invention rights by our employees, which could result in litigation and adversely affect our business.

We enter into agreements with our employees pursuant to which they agree that any inventions created in the scope of their employment or engagement are assigned to us or owned exclusively by us, without the employee retaining any rights. A significant portion of our intellectual property has been developed by our employees in the course of their employment for us. Under the Israeli Patent Law, 5727-1967 (the "Patent Law"), inventions conceived by an employee during the scope of his or her employment with a company are regarded as "service inventions," which belong to the employer, absent a specific agreement between the employee and employer giving the employee service invention rights. The Patent Law also provides that if there is no such agreement between an employer and an employee, the Israeli Compensation and Royalties Committee (the "Committee"), a body constituted under the Patent Law, shall determine whether the employee is entitled to remuneration for his or her inventions. Previous decisions by the Committee have created uncertainty in this area regarding whether the right to receive remuneration for service inventions can be voluntarily waived by an employee and whether such waiver is enforceable. In addition, the Committee determined that even if such right to receive compensation and royalties for service inventions may be waived, the waiver should be specific. Subsequent court cases have not provided significant clarity on these matters.

Risks Related to Our Common Stock

Our stock price may be volatile, and purchasers of our common stock could incur substantial losses.

Our common stock began trading on The Nasdaq Global Market on April 26, 2018 under the symbol "ELOX." The trading price of our common stock has been volatile and may continue to be volatile and subject to wide fluctuations in the future. Many factors could have an impact on our stock price, including fluctuations in our or our competitors' operating results, clinical trial results or adverse events associated with our product candidates, product development by us or our competitors, changes in laws, including healthcare, regulatory, tax or intellectual property laws, intellectual property developments, acquisitions or other strategic transactions, changes in financial or operational estimates or projections and the perceptions of our investors that we are not performing or meeting expectations. The trading price of the common stock of many biopharmaceutical companies, including ours, has experienced extreme price and volume fluctuations, which have at times been unrelated to the operating performance of the companies whose stocks were affected. In addition, the securities market has from time to time experienced significant price and volume fluctuations that are not related to the operating performance of particular companies. These market fluctuations may also materially and adversely affect the market price of shares of our common stock.

Our directors, executive officers, principal stockholders and affiliated entities own a significant percentage of our capital stock, and they may make decisions that an investor may not consider to be in the best interests of our stockholders.

Our directors, executive officers, principal stockholders and affiliated entities beneficially own, in the aggregate, a significant percentage of our common stock, giving effect to options and other derivative securities that are held by such persons. As a result, if some or all of them acted together, they would have the ability to exert substantial influence over the election of our board of directors and the outcome of issues requiring approval by our stockholders. This concentration of ownership may have the effect of delaying or preventing a change in control of our company that may be favored by other stockholders. This could prevent the consummation of transactions favorable to other stockholders, such as a transaction in which stockholders might otherwise receive a premium for their shares over current market prices.

Future sales and issuances of our securities or rights to purchase securities, including pursuant to our equity incentive plans, could result in additional dilution of the percentage ownership of our stockholders and could cause the prices of our securities to fall.

Additional capital will be needed in the future to continue our planned operations. To the extent we raise additional capital by issuing equity securities, our stockholders may experience substantial dilution. We may sell common stock, convertible securities or other equity securities in one or more transactions at prices and in a manner, we determine from time to time. If we sell common stock, convertible securities or other equity securities in one or more transactions, existing investors may be materially diluted by subsequent sales, and new investors could gain rights superior to our existing stockholders.

Pursuant to our 2018 Equity Incentive Plan, our management is authorized to grant stock options and other equity-based awards to our employees, directors and consultants. As of September 30, 2020, individuals held share awards to purchase or receive an aggregate of 5,635,904 shares of our common stock. If our board of directors elects to increase the number of shares available for future grant by the maximum amount each year, our stockholders may experience additional dilution, which could have a negative effect on our share price.

Risks Related to the Reverse Merger

The risks arising with respect to the historic Sevion business and operations may be different from what we anticipate, which could lead to significant, unexpected costs and liabilities and could materially and adversely affect our business going forward.

We may not have fully anticipated the extent of the risks associated with the reverse merger between Sevion and Eloxx Limited completed in December 2017. After the reverse merger, Sevion's historic business was discontinued, but prior to the transaction Sevion had a long operating history. As a consequence, we may be subject to claims, demands for payment, regulatory issues, costs and liabilities that were not and are not currently expected or anticipated. Notwithstanding our exercise of due diligence pre-transaction and risk mitigation strategies post-transaction, the risks involved with taking over a business with a long operating history and the costs and liabilities associated with these risks may be greater than we anticipate. Further, we do not have rights of indemnification against the pre-transaction stockholders of Sevion. We may not be able to contain or control the costs or liabilities associated with Sevion's historic business, which could materially and adversely affect our business, liquidity, capital resources or results of operation, and may divert management's time and attention from conducting the business of the Company.

Item 6. Exhibits

The following is a list of exhibits filed as part of this Report. Where so indicated, exhibits that were previously filed are incorporated by reference. For exhibits incorporated by reference, the location of the exhibit in the previous filing is indicated.

Exhibit Number	Exhibit Description	Incorporated by Reference			
		Form	File No.	Exhibit	Filing Date
31.1*	Certification of the Company's Principal Executive Officer pursuant to Rule 13a-14(a) and Rule 15d-14(a) of the Securities and Exchange Act of 1934, as amended, pursuant to Section 302 of the Sarbanes-Oxley Act of 2002.				
31.2*	Certification of the Company's Principal Financial Officer pursuant to Rule 13a-14(a) and Rule 15d-14(a) of the Securities and Exchange Act of 1934, as amended, pursuant to Section 302 of the Sarbanes-Oxley Act of 2002.				
32.1***	Certification of the Company's Principal Executive Officer pursuant to 18 U.S.C. Section 1350, as adopted pursuant to Section 906 of the Sarbanes-Oxley Act of 2002.				
32.2***	Certification of the Company's Principal Financial Officer pursuant to 18 U.S.C. Section 1350, as adopted pursuant to Section 906 of the Sarbanes-Oxley Act of 2002.				
101.INS*	Inline XBRL Instance Document – the instance document does not appear in the Interactive Data File because its XBRL tags are embedded within the XBRL document.				
101.SCH*	Inline XBRL Taxonomy Extension Schema Document.				
101.CAL*	Inline XBRL Taxonomy Extension Calculation Linkbase Document.				
101.DEF*	Inline XBRL Taxonomy Extension Definition Linkbase Document.				
101.LAB*	Inline XBRL Taxonomy Extension Label Linkbase Document.				
101.PRE*	Inline XBRL Taxonomy Extension Presentation Linkbase Document.				
104	Cover Page Interactive Data File (embedded within the Inline XBRL document)				

* Filed herewith.

*** This certification is being furnished solely to accompany this Quarterly Report on Form 10-Q pursuant to 18 U.S.C. Section 1350, and is not being filed for purposes of Section 18 of the Securities Exchange Act of 1934, as amended, or otherwise subject to the liability of that section, nor shall it be deemed incorporated by reference into any filing of the registrant under the Securities Act of 1933, as amended, or the Securities Exchange Act of 1934, as amended, whether made before or after the date hereof, regardless of any general incorporation language in such filing.

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.

ELOXX PHARMACEUTICALS, INC.

Date: November 6, 2020

by: /s/ Stephen G. MacDonald

Stephen G. MacDonald

Vice President, Finance and Accounting, and Treasurer

(Principal Financial Officer and Principal Accounting Officer)

Exhibit 31.1

CERTIFICATION

I, Gregory C. Williams, certify that:

1. I have reviewed this Quarterly Report on Form 10-Q of Eloxx Pharmaceuticals, Inc.;

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

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

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

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

(b) Designed such internal control over financial reporting, or caused such internal control over financial reporting to be designed under our supervision, to provide reasonable assurance regarding the reliability of financial reporting and the preparation of financial statements for external purposes in accordance with generally accepted accounting principles;

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

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

5. The registrant's other certifying officer(s) 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: November 6, 2020

/s/ Gregory C. Williams PhD, MBA

Gregory C. Williams, PhD, MBA
Chief Executive Officer
(Principal Executive Officer)

Exhibit 31.2

CERTIFICATION

I, Stephen G. MacDonald, certify that:

1. I have reviewed this Quarterly Report on Form 10-Q of Eloxx Pharmaceuticals, Inc.;

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

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

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

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

(b) Designed such internal control over financial reporting, or caused such internal control over financial reporting to be designed under our supervision, to provide reasonable assurance regarding the reliability of financial reporting and the preparation of financial statements for external purposes in accordance with generally accepted accounting principles;

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

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

5. The registrant's other certifying officer(s) 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: November 6, 2020

/s/ Stephen G. MacDonald
Stephen G. MacDonald
Vice President, Finance and Accounting, and Treasurer
(Principal Financial Officer and Principal Accounting Officer)

Exhibit 32.1

CERTIFICATION[1]

Pursuant to the requirement set forth in Rule 13a-14(b) of the Securities Exchange Act of 1934, as amended (the "Exchange Act"), and Section 1350 of Chapter 63 of Title 18 of the United States Code (18 U.S.C. §1350), I, Gregory C. Williams, Chief Executive Officer of Eloxx Pharmaceuticals, Inc. (the "Company"), hereby certify that, to the best of my knowledge:

1. The Company's Quarterly Report on Form 10-Q for the period ended September 30, 2020, to which this Certification is attached as Exhibit 32.1 (the "Quarterly Report") fully complies with the requirements of Section 13(a) or Section 15(d) of the Exchange Act, and

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

IN WITNESS WHEREOF, the undersigned have set their hands hereto as of the 6th day of November, 2020.

/s/ Gregory C. Williams PhD, MBA
Gregory C. Williams, PhD, MBA
Chief Executive Officer
(Principal Executive Officer)

(1) This certification accompanies the Form 10-Q to which it relates, is not deemed filed with the Securities and Exchange Commission and is not to be incorporated by reference into any filing of Eloxx Pharmaceuticals, Inc. under the Securities Act of 1933, as amended, or the Securities Exchange Act of 1934, as amended (whether made before or after the date of the Form 10-Q), irrespective of any general incorporation language contained in such filing.

Exhibit 32.2

CERTIFICATION[1]

Pursuant to the requirement set forth in Rule 13a-14(b) of the Securities Exchange Act of 1934, as amended (the "Exchange Act"), and Section 1350 of Chapter 63 of Title 18 of the United States Code (18 U.S.C. §1350), I, Stephen G. MacDonald, Vice President, Finance and Accounting, and Treasurer of Eloxx Pharmaceuticals, Inc. (the "Company"), hereby certify that, to the best of my knowledge:

1. The Company's Quarterly Report on Form 10-Q for the period ended September 30, 2020, to which this Certification is attached as Exhibit 32.2 (the "Quarterly Report") fully complies with the requirements of Section 13(a) or Section 15(d) of the Exchange Act, and

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

IN WITNESS WHEREOF, the undersigned have set their hands hereto as of the 6th day of November, 2020.

/s/ Stephen G. MacDonald
Stephen G. MacDonald
Vice President, Finance and Accounting, and Treasurer
(Principal Financial Officer and Principal Accounting Officer)

(1) This certification accompanies the Form 10-Q to which it relates, is not deemed filed with the Securities and Exchange Commission and is not to be incorporated by reference into any filing of Eloxx Pharmaceuticals, Inc. under the Securities Act of 1933, as amended, or the Securities Exchange Act of 1934, as amended (whether made before or after the date of the Form 10-Q), irrespective of any general incorporation language contained in such filing.