

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

**FORM 10-K**

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

**For the fiscal year ended December 31, 2019**

**OR**

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

**For the transition period from \_\_\_\_\_ to \_\_\_\_\_**

**Commission file number: 001-31326**

**ELOXX PHARMACEUTICALS, INC.**

**(Exact Name of Registrant as Specified in Its Charter)**

**Delaware**

(State or Other Jurisdiction of  
Incorporation or Organization)

**84-1368850**

(I.R.S. Employer  
Identification No.)

**950 Winter Street**

**Waltham, Massachusetts 02451**

**(Address of Principal Executive Offices and Zip Code)**

**(781) 577-5300**

**(Registrant's Telephone Number, Including Area Code)**

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

<b>Title of each class</b>	<b>Name of each exchange on which registered</b>
<b>Common Stock, \$0.01 par value</b>	<b>NASDAQ Global Market</b>

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

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

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

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

Indicate by check mark whether the registrant has submitted electronically every Interactive Data File required to be submitted and 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, a smaller reporting company or an emerging growth company. See definitions of "accelerated filer", "large accelerated filer", "smaller reporting company" or "emerging growth company" in Rule 12b-2 of the Exchange Act.

Large accelerated filer   
Non-accelerated filer

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 Act). Yes  No

The aggregate market value of the voting stock held by non-affiliates of the registrant on June 28, 2019, the last business day of the registrant's most recently completed second quarter, was \$268,970,171, based on the closing price for such stock as reported on the NASDAQ Capital Market on that date.

As of March 1, 2020, there were 40,118,481 shares of the Registrant's common stock, par value \$0.01 per share, outstanding.

**DOCUMENTS INCORPORATED BY REFERENCE**

Certain information required by Part III, Items 10-14 of this Form 10-K is incorporated by reference to the Registrant's definitive Proxy Statement for the 2020 Annual Meeting of Stockholders to be filed with the Securities and Exchange Commission pursuant to Regulation 14A not later than 120 days after the end of the fiscal year covered by this Form 10-K, provided that if such Proxy Statement is not filed within such period, such information will be included in an amendment to this Form 10-K to be filed within such 120-day period.

**ELOXX PHARMACEUTICALS INC.**  
**TABLE OF CONTENTS**

	<u>Page</u>
<b><u>PART I</u></b>	
ITEM 1. <u>Business</u>	1
ITEM 1A. <u>Risk Factors</u>	29
ITEM 1B. <u>Unresolved Staff Comments</u>	50
ITEM 2. <u>Properties</u>	50
ITEM 3. <u>Legal Proceedings</u>	50
ITEM 4. <u>Mine Safety Disclosures</u>	50
<b><u>PART II</u></b>	51
ITEM 5. <u>Market for Registrant's Common Equity, Related Stockholder Matters and Issuer Purchases of Equity Securities</u>	51
ITEM 6. <u>Selected Financial Data</u>	51
ITEM 7. <u>Management's Discussion and Analysis of Financial Condition and Results of Operations</u>	52
ITEM 7A. <u>Quantitative and Qualitative Disclosure About Market Risk</u>	61
ITEM 8. <u>Financial Statements and Supplementary Data</u>	61
ITEM 9. <u>Changes in and Disagreements with Accountants on Accounting and Financial Disclosure</u>	62
ITEM 9A. <u>Controls and Procedures</u>	62
ITEM 9B. <u>Other Information</u>	65
<b><u>PART III</u></b>	66
ITEM 10. <u>Directors, Executive Officers and Corporate Governance</u>	66
ITEM 11. <u>Executive and Director Compensation</u>	66
ITEM 12. <u>Security Ownership of Certain Beneficial Owners and Management and Related Stockholder Matters</u>	66
ITEM 13. <u>Certain Relationships and Related Transactions, and Director Independence</u>	66
ITEM 14. <u>Principal Accounting Fees and Services</u>	66
<b><u>PART IV</u></b>	67
ITEM 15. <u>Exhibits and Financial Statement Schedules</u>	67
ITEM 16. <u>Form 10-K Summary</u>	67
<b><u>SIGNATURES</u></b>	73

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

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;
- 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 reverse merger and potentially significant, unexpected costs and liabilities arising with respect to the historic Sevion business and operations.

## PART I

### ITEM 1. BUSINESS

#### 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 disorders. 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 disorders 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 mRNA stability and enabling functional protein synthesis. 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 and nephropathic cystinosis. ELX-02 is an investigational drug that has not been approved by any global regulatory body. In addition, during 2019 we announced a new program studying 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. 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”). Professor Timor Baasov, the inventor of our compounds, has served as our senior consultant since our incorporation.

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 provide support for both continuing our clinical development programs and evaluating the suitability of our ERSG library for development in additional renal disorders, including ADPKD and cystinuria. Our preclinical candidate pool consists of a library of novel ERSG drug candidates identified based on read-through potential and cytoplasmic ribosomal selectivity.

Our research and development strategy targets rare or ultra-rare diseases where a high unmet medical need exists, an identified 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 identifiable. 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 studies in both cystic fibrosis and nephropathic cystinosis.

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 disorders: cystic fibrosis, nephropathic cystinosis, ADPKD, a variety of IRDs (including Usher Syndrome), primary ciliary

dyskinesia, cystinuria, 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, 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.

Currently, the clinical programs for our lead investigational drug candidate, ELX-02, are focused on development for cystic fibrosis and nephropathic cystinosis patients with diagnosed nonsense mutations. With ELX-02, 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 U.S. 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 trial were presented in 2019 at both the European Cystic Fibrosis 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 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, 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 actively dosing patients in the U.S. and Israel and continuing to enroll patients at leading global investigator sites. We expect to achieve full enrollment and to report topline results during the first half of 2020. In the U.S., the Cystic Fibrosis 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 (ClinicalTrials.gov Identifiers: NCT04126473 and NCT04135495), which is being conducted at TDN member sites.

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. In Europe and Israel, Professor Eitan Kerem, M.D., Head of the Division of Pediatrics, Children’s Hospital, Hadassah Medical Center in Israel, is the global lead 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) and we are currently enrolling and dosing patients in both trials. 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.

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 of ERSG compounds successfully reach IRD-relevant tissue layers and can restore protein production in a relevant animal model after an intravitreal injection. We are encouraged that drug delivery to the back of the eye with our compounds is possible through intravitreal administration, the most commonly utilized therapeutic delivery route for retinal specialists. We believe this approach will enable distribution of the ERSG compound throughout the retina. We plan to present these data at the Association for Research in Vision and Ophthalmology (ARVO) Annual Meeting May 3-7, 2020 in Baltimore, Maryland.

We are also evaluating the suitability of our ERSG library for development in rare renal disorders associated with nonsense mutations, such as ADPKD and cystinuria. ADPKD is a relatively common inherited genetic kidney

disease occurring in between 1:400 and 1:1,000 patients and 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 IND.

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. There are no currently approved therapeutics that target the nonsense mutation mediated impairment of cystinosin, the cystine-selective transport channel in the lysosomal membrane that is attributed as the cause for the accumulation of cystine in this disease state. Given the high proportion of pediatric patients in each of these 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), and the FDA has granted orphan drug designation to ELX-02 for the treatment of nephropathic cystinosis, MPS I, and Rett syndrome.

In September 2019, we appointed Dr. Thomas Haverty as our Chief Medical Officer. Dr. Haverty has over 30 years of pharmaceutical and biotechnology experience leading clinical research and operations teams with responsibility for large development portfolios in virtually all classes of molecules and indications. Prior to joining us in his current capacity, Dr. Haverty, a Board-certified Nephrologist, served as a consultant to us following a long tenured career at Johnson & Johnson, Schering-Plough and Merck Research Labs. Dr. Haverty has successfully led the development and approval of over 20 leading drugs.

### **Our Technology**

Nonsense mutations, also known as premature termination or stop codons, are single point mutations within the DNA sequence which are either inherited or acquired that result in the premature termination of the translational process leading to truncated or absent proteins. Nonsense mutations are involved in a large number of genetic diseases such as cystic fibrosis, nephropathic cystinosis, ADPKD, primary ciliary dyskinesia, and an estimated 300 IRDs, including Usher Syndrome. According to the Human Gene Mutation Database (<http://www.hgmd.cf.ac.uk/ac/index.php>), nonsense mutations account for approximately twelve percent (12%) of patients with a given genetic disease. The disease phenotypes caused by nonsense mutations are frequently more severe than those caused by other kinds of mutations because these mutations often lead to a complete loss of protein production or function. In general, these diseases do not have specific therapies beyond symptomatic and palliative interventions.

In eukaryotic cells, the cytoplasmic ribosome is responsible for the production of proteins by a process called translation. As part of the translation process, the genetic information stored in DNA is transcribed to mRNA to specify the sequence of amino acids, the building blocks of protein, required to produce a functional product. The mRNA is decoded by a complex known as the ribosome in the cytoplasm of the cell. The ribosome ratchets along the mRNA and matches three nucleotide stretches, referred to as a codon, with a corresponding aminoacyl transfer RNA (a-tRNA) which carry the correct amino acid to add onto the growing protein chain.

Normal translation termination in eukaryotic cells occurs when a native (canonical) termination codon enters the ribosomal decoding region. The ribosome pauses at these special termination sequences, since there are no complementary a-tRNA for these codons. A complex of termination factors, including eRF1 and eRF3, will next bind to the ribosome and initiate the release of the newly synthesized protein.

When a nonsense mutation occurs, a single DNA nucleotide is substituted for the typical nucleotide, changing a codon for an amino acid into a stop codon. For example, a tryptophan “TGG” codon may be changed to read “TGA” or “TAG”, either of which would result in a premature termination signal for the protein. While non-functional, truncated proteins can result from this type of mutation, more often the protein product is unstable and degraded. Since most genes have two copies (referred to as alleles) within the genome, a nonsense mutation on a single copy may be compensated if the other copy still codes for a functional protein. However, when mutations occur on both alleles, disease may occur. The two mutations can be the same mutation (homozygous) or two different disease-causing mutations (heterozygous).

Cells have a system to detect nonsense mutation bearing mRNA copies and remove them from the cell. This process is called nonsense mediated mRNA decay. During the first round of translation, the ribosome removes proteins that are found along the newly made mRNA at regions called exon-exon borders. These regions are remnants of a splicing process that occurs in the nucleus while the RNA is being transcribed. If a stop codon is found by the ribosome during this first round of translation in a location before the last of these exon-exon junction protein complexes, the mRNA is targeted for degradation. Therefore, nonsense mutations result in a “double hit” for the gene, unstable mRNA and reduced functional protein (shown in Figure 1 below).

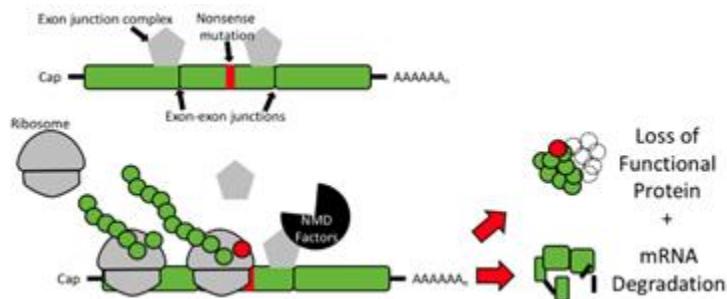


Figure 1: Nonsense Mutations Cause Unstable mRNA and Reduced Functional Protein

Read-through of nonsense mutations is a naturally occurring process that can be attributed to the redundancy “wobble” in the three-nucleotide codon structure. Native read-through occurs in up to 1% of instances in which a ribosome pauses at a stop codon. During read-through, either the typical healthy or a near-cognate substitute tRNA competes out the termination factor complex to enable the insertion of an amino acid and continuation of translation. By directly binding to the ribosome decoding region, ELX-02 enhances the likelihood of this process by increasing the probability of a tRNA to out-compete the termination complex. As read-through allows the ribosome to proceed with translation through the premature stop, a reduction in nonsense mediated decay and stabilization of the mRNA pool is an additional expected outcome of this process (shown in Figure 2 below). Indeed, ELX-02 is found to increase both the mRNA and functional protein across multiple nonsense mutations. Beyond ELX-02, multiple compounds within our ERSG library demonstrate results consistent with this mechanism of action.

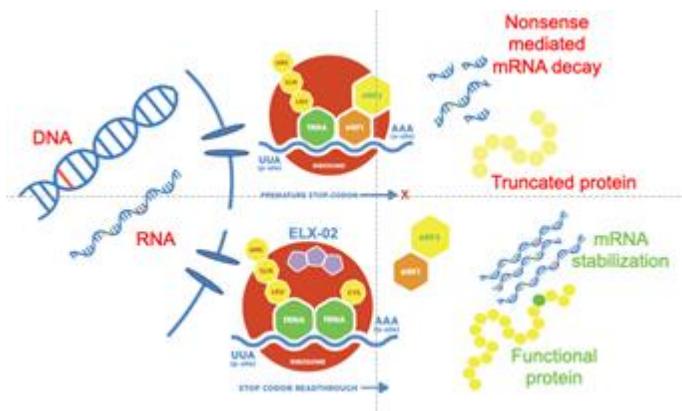


Figure 2: Read-through Allows the Ribosome to Proceed with Translation through the Premature Stop, Reducing mRNA Decay and Increasing the Translation of Functional Protein

We believe that the segment of cystic fibrosis, nephropathic cystinosis, ADPKD, and IRD patients with diagnosed nonsense mutations on one or both alleles represents a high unmet medical need as there are currently no approved therapeutics targeting the impairment caused by these mutations. There are existing *in vitro* assays, animal models and/or biomarker screens that have been demonstrated to be useful in assessing the potential therapeutic benefit of development compounds for these disease states. The design of clinical trials and the endpoints for measuring clinical benefit have been established for the currently approved therapeutics for these disorders. We believe these to be attractive development targets based on the potential use of these precedents to de-risk the program.

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 of genetic disorders and improve the lives of patients. Our preclinical data in animal models of nonsense mutations suggest that drug product candidates from our read-through compound library may have potential beneficial effects for each of the following diseases: cystic fibrosis, nephropathic cystinosis, a variety of IRDs (including Usher Syndrome), primary ciliary dyskinesia, ADPKD, cystinuria, 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, muscles and others. We believe that additional molecules from our ERSG library have the potential to demonstrate a sufficient activity profile to become drug product candidates, and we are conducting ongoing preclinical evaluation of these molecules.

### ***Current Data Indicating the Mechanism of Action of ELX-02***

ELX-02 is a novel eukaryotic ribosomal selective glycoside (ERSG) with increased selectivity for the eukaryotic ribosome. In contrast, aminoglycosides, such as gentamicin, are potent antibiotics that bind with high affinity to the decoding site in the prokaryotic ribosome and inhibit protein translation in bacteria as well as the eukaryotic ribosomes. The early observations of eukaryotic read-through activity were made in patients treated with aminoglycosides due to bacterial infections. In eukaryotic cells, aminoglycosides may induce a conformational change that reduces the codon-anticodon recognition, enhancing the ability of an a-tRNA to compete with the release factor complex for binding to the premature termination codon and increasing the probability that translational read-through of premature termination codons occurs. However, despite the promising clinical observations of read-through activity, aminoglycoside use as a read-through therapy is restricted since aminoglycosides may cause damage to the kidney and ear after prolonged administration. The effect of aminoglycosides on the hair cells of the ear and the proximal tubular cells of the kidney may be attributed, in part, to the inhibition of mitochondrial protein production in these cells. Due to the markedly decreased affinity for the prokaryotic and mitochondrial ribosomes, ERSG compounds have little of the antibiotic activity associated with aminoglycosides. When compared in laboratory tests to gentamicin, a classic aminoglycoside, ELX-02 thus far has shown a 100-fold lower antibacterial activity and nine-fold higher read-through activity for nonsense mutations; this has been attributed to higher selectivity towards the cytoplasmic eukaryotic ribosome. Because ERSGs are selective for the eukaryotic cytoplasmic ribosome and have markedly decreased affinity for the mitochondrial ribosome, ERSGs are anticipated to show a safety and tolerability profile acceptable for chronic use. We believe that compounds with increased read-through activity and a reduced affinity for the mitochondrial ribosome may demonstrate an acceptable profile for chronic use. We believe that our library of ERSG compounds has the potential to show improvements in read-through activity with a reduced potential for impairment of the mitochondrial ribosome and may have an improved safety profile.

ERSGs with improved selectivity for the eukaryotic cytoplasmic ribosome are expected to demonstrate improved rates of full-length protein translation and a reduction in triggered nonsense mediated decay. By binding to the cytoplasmic ribosome and reducing ribosomal stalling, ERSGs are expected to demonstrate increases in mRNA and full-length protein production. Therefore, our read-through activity screening program has used these endpoints to evaluate our compound library portfolio.

Using a well-characterized cell line bearing a nonsense mutation, we are evaluating our compound library for both read-through mediated protein restoration and stabilization of mRNA. While plasmid-based evaluations offer a convenient methodology to evaluate across disease related mutations, this approach is limited to demonstrating the potential for protein restoration alone. Therefore, we have focused on using our molecules to detect read-through of a native mutation, which is subject to nonsense mediated decay, in order to better model an actual disease setting. In this cell line, the protein of interest and its mRNA are significantly reduced. We evaluated a subset of our compounds in the cell by quantitative polymerase chain reaction (qPCR), western blot protein analysis and for proper localization of the restored protein by immunofluorescence. Consistent with our overall hypothesis, we have found that molecules in our library significantly increase both the mRNA (dose-dependent increases of 30-fold over vehicle control) and protein of interest (increased normalized full-length protein 4.7-fold over vehicle control) as well as the protein's proper sub-cellular localization. In addition to supporting our overall mechanism of action hypothesis, these studies have enabled us to develop a robust ( $z'$  factor = 0.88), high-throughput assay within this system to further evaluate compound-mediated read-through across our library.

Our mechanism of action is further supported within disease-relevant model systems, further detailed below. *In vitro* models of nephropathic cystinosis and CF demonstrate dose-dependent increases in target mRNA as well as significant increases in functional protein. Unlike previous read-through programs reported by other groups, ELX compounds have consistently demonstrated activity against both of these hallmark consequences of nonsense

mutations. We believe this understanding of ELX compound activity has enabled our clinical development programs to move forward with confidence.

## Scope of Activities

We intend to be the global leader in the application of the science of translational read-through and the associated pathway of NMD.

### Cystic Fibrosis

Cystic fibrosis (CF) is the most prevalent genetic disease in the western world and affects an estimated 70,000 to 100,000 patients worldwide, with the vast majority of affected individuals in the U.S., Canada, Europe, and Australia. CF is the most common fatal inherited disease in Caucasians. The incidence of CF varies across the globe ranging between one in 3,500 births in the U.S. and one in 2,000 to 3,000 births in Europe and Australia.

Approximately 13% of CF patients carry a nonsense mutation on the *CFTR* gene. CF is a progressive disease caused by a deficiency in CFTR activity with insufficient ionic transconductance in the cell membrane, which, in turn, leads to the accumulation of thick mucus in vital organs, particularly the lungs, pancreas and gastrointestinal tract. As a result, CF patients experience respiratory infections, chronic lung inflammation, and poor absorption of nutrients as well as many other conditions, and, in most cases, progressive respiratory failure. The life expectancy of CF patients has improved, and recently the Cystic Fibrosis Foundation reported that a baby born with cystic fibrosis (CF) today can now expect to live a median 44 years.

Cystic Fibrosis occurs at a rate of one in 2,500 to 6,000 births, depending on the region and ethnic origin. Patients with CF caused by nonsense mutations have some of the most severe forms of the disease and, other than palliative therapies, no treatment currently exists for them. Mutations in the gene that encodes CFTR protein, which play a critical role in regulating the viscosity of the mucus layer that lines human organs, cause CF. The CFTR protein forms an ion channel that regulates the flow of ions in and out of the cells of vital organs such as the lungs, pancreas and gastrointestinal tract. We refer to this as ion flow. In tissues like these, CFTR protein conducts ions leading to osmosis as water is drawn out of the cell hydrating the cell surface. Through regulation of the location of the ions across the cell membrane, the amount of salts in the fluid both inside and outside the cell remains balanced.

In CF patients, the *CFTR* gene is defective, and as a result, CF patients lack the functional CFTR protein ion channel necessary to regulate ion flow. An altered ion concentration gradient between the inside and the outside of the cell reduces the amount of water molecules outside the cell, causing the accumulation of thick mucus on the epithelial surface as shown below in Figure 3.

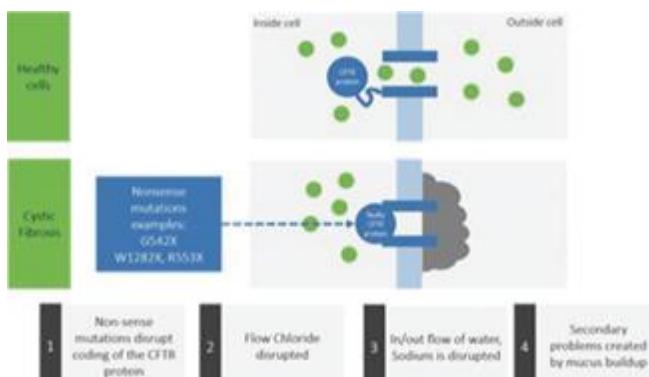


Figure 3: Ion Flow in Normal CFTR Protein Compared to Mutant CFTR Protein

The deficiency in CFTR protein activity in CF patients is particularly problematic in the lungs, where the build-up of thick mucus obstructs airflow and impairs proper immune response, which leads to chronic infection and persistent inflammation. In the pancreas and the gastrointestinal tract, the build-up of mucus prevents the release of digestive enzymes that help the body break down food and impairs the absorption of nutrients, resulting in poor growth and development.

### *Cystinosis*

Cystinosis is an ultra-rare autosomal recessive lysosomal storage disease. Mutations in the *CTNS* gene (cystinosin), on the short arm of chromosome 17 (17p13), cause the primary defect in the disease. Cystinosin is a ubiquitous cystine-selective transport channel in the lysosomal membrane. Loss-of-function mutations prevent cystine efflux from the lysosome, causing massive accumulation of intra-lysosomal cystine in tissues throughout the body, and lead to apoptotic cell death, impaired physiology and end organ damage.

Affected children may appear fairly well until the age of 4 to 6 months, when progressive dysfunction and atrophy of the proximal renal tubule cause Fanconi syndrome and failure to thrive. By 10 to 12 years of age, dialysis or kidney transplantation is required to treat end-stage renal disease. Although the renal allograft is spared, lifespan is diminished by the inexorable dysfunction of other organs.

The most common nonsense mutation in the *CTNS* gene is *W138X*, which has an overall incidence rate of one in every 62,500 live births in Quebec, Canada.

Current treatment includes cysteamine bitartrate (Cystagon® or Procysbi®). Cystagon was approved in the U.S. and Europe in 1994 and Procysbi was approved in the U.S. and Europe in 2013. Both therapies delay but do not cure the condition and despite treatment, patients eventually require dialysis and renal transplantation and experience significant morbidity in other organ systems. See above for information about our Phase 2 clinical trial in nephropathic cystinosis.

### *Autosomal Dominant Polycystic Kidney Disease*

Polycystic Kidney Disease (PKD) is a progressive genetic disorder causing the growth of cysts in the kidneys, reducing kidney function and ultimately leading to kidney failure or end stage renal disease. It is found in all races and occurs equally in men and women. The autosomal dominant form of polycystic kidney disease (ADPKD) is the most common genetic nephropathology, accounting for 5 to 8% of end stage renal disease. Worldwide, ADPKD affects approximately 4 to 7 million individuals and accounts for 7 to 15% of patients on renal replacement therapy. In North America and Europe, ADPKD is responsible for 6 to 10% of end stage renal disease cases. It is estimated that one per 800 to 1,000 in the population carries a mutation for this condition.

Approximately 85 to 90% of patients with ADPKD have the mutations in the *PKD1* gene; most of the remaining patients have the *PKD2* gene. Estimates suggest over 30% of ADPKD mutations are nonsense variants and therefore represent a favorable target for read-through therapeutics given the large patient population, substantial subset of nonsense mutations, disease severity and limited treatment options. 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 IND.

### *Inherited Retinal Disorders*

Inherited retinal disorders (also called inherited retinal dystrophies, or IRDs) are a group of rare eye disorders caused by inherited gene mutations which can result in vision loss or blindness. Some IRDs, such as Retinitis Pigmentosa (RP), Usher Syndrome, or Choroideremia (CHM) are associated with a gradual loss of vision, eventually leading to complete blindness. In other conditions such as Leber's Congenital Amaurosis (LCA), patients may be born with or experience vision loss in infancy or early childhood.

A specific IRD, Usher Syndrome, is the most common genetic disorder involving hearing and vision abnormalities and is the most common cause of deafness-blindness with visual impairment due to photoreceptor degeneration. Three types of Usher Syndrome have been identified based on genetic causes, severity of hearing loss and age of onset. USH1 patients are typically born with severe hearing loss and are diagnosed early in life with vision problems beginning at or before age of 10 years. USH2 patients are characterized by progressive hearing loss from birth, with onset of vision loss at adolescence. USH3 has the slowest progression, vision loss develops within the second decade of life. In most countries, USH1 accounts for one-third of all patients with USH2 accounting for about two-thirds of all USH patients. U.S. prevalence is estimated at approximately 6,200 Type 1 and approximately 8,900 Type 2 patients. It is estimated that there are over 4,000 patients in North America with nonsense forms of Usher Syndrome. Effective read-through has been demonstrated *in vitro* for *USH2A* (USH2), *PCDH15* (USH1) and *USH1C* (USH1) nonsense mutations. *USH2A* nonsense variants are estimated to affect more than 2,500 patients in

the U.S. alone. There are four *USH2A* splice site variants, with the largest having over 70 exons, potentially limiting gene therapy development due to vector size limitations.

RP causes night blindness in adolescence and gradual visual field loss in adulthood (most patients are legally blind by age 40). RP can occur as a localized disorder with visual impairment only, or as part of a broader syndrome (e.g., Usher Syndrome). More than 50 different causative genes have been identified and the U.S. incidence is estimated at approximately 67,000 patients. Given this high genetic heterogeneity even in nonsense mediated disease (no single gene comprises more than 10% of RP cases), there is a significant need to identify broader cross-gene therapies. Read-through proof of principle has been shown via *in vitro* models of *USH2A*, *RHO*, *MERTK*, and *RP2* nonsense mutation variants, with 5% to 20% rescue of protein function demonstrated.

In December 2017, the FDA approved Spark Therapeutics' Luxturna® as the first gene therapy for retinal dystrophy caused by mutations in the *RPE65* gene and targeting approximately 1,000 to 2,000 patients with confirmed biallelic *RPE65* mutation-associated retinal dystrophy. While many gene therapies focus on specific IRDs, many target genes share related ocular disease pathways, suggesting a broader gene-agnostic approach may provide greater treatment opportunities. IRDs such as Usher Syndrome and RP represent favorable targets for read-through therapeutics given the high unmet need and the opportunity to target multiple nonsense mutations across a variety of genes. Read-through therapeutics offer potential treatment for patient subsets not addressed by gene therapy due to gene size or small patient subsets.

### *Primary Ciliary Dyskinesia*

Primary Ciliary Dyskinesia (PCD) is a genetically heterogeneous disease which is predominantly inherited as an autosomal recessive disorder. PCD is associated with abnormal ciliary function resulting in impaired mucociliary clearance, recurrent/chronic upper and lower respiratory tract infection and male infertility. PCD prevalence is estimated to involve one in 20,000 individuals; however, disease symptom variability and low familiarity with PCD likely leads to substantial patient under-diagnosis. PCD patient treatment largely focuses on airway clearance (devices and aerosolized therapies), infection control (anti-microbial therapy), and inflammation control (antibiotic therapy). There have been few randomized trials of PCD treatment to date.

More than 30 different genes have been identified to be causative for PCD with approximately 25% of identified gene mutations having nonsense mutations. *In vitro* studies of aminoglycoside-mediated read-through in PCD nonsense mutations found positive read-through efficacy in several UGA associated stop codons. PCD is an attractive disease target given the high unmet medical need, positive preclinical read-through data, potential for treatment across multiple genes, high prevalence of nonsense mutations, and lack of disease modifying treatments.

### *Other Nonsense Mediated Genetic Disorders*

Muscular dystrophies are genetic disorders involving progressive muscle wasting and weakness. Duchenne muscular dystrophy (DMD) is the most common and one of the most severe types of muscular dystrophy with the average age of death for DMD patients in the mid-twenties. DMD occurs when a mutation in the dystrophin gene prevents the cell from making a functional dystrophin protein. Because the dystrophin gene is located on the X chromosome, DMD occurs almost exclusively in young boys. Genetic tests are available to determine if a patient's DMD is caused by a nonsense mutation. Based on a publication from Prior, et al. (1995) in the American Journal of Human Genetics, it is estimated that a nonsense mutation is the cause of DMD in approximately 13% of patients. Treatments for DMD include Translarna™ (ataluren), approved in the European Union ("EU") for the treatment of the underlying cause of DMD. EXONDYS 51® (eteplirsen) injection is also approved in the US for the treatment of DMD patients with exon 51 skipping mutations.

Mucopolysaccharidosis type I (MPS I) is a chronic, progressive genetic disorder caused by a deficiency of the enzyme alpha-L-iduronidase (IDUA). Children with severe MPS I often die in the first decade of life due to respiratory failure, cardiac valvulopathy, and cardiorespiratory problems. The deficiency of the IDUA enzyme leads to the accumulation of a class of molecules called glycosaminoglycans (GAGs). The accumulation of GAGs causes disruption in the movement of molecules inside the cell and leads to the subsequent dysfunction of cells, tissues and organs. Globally, MPS I occurs in about one in every 100,000 births for the severe form and one in 500,000 for the attenuated form. About 70% of MPS I patients carry one of two nonsense mutations: Q70X and W402X. Estimates

suggest that 50 to 80% of all MPS I patients present with the severe versus attenuated form of the disease. MPS I disease-specific treatments include enzyme replacement therapy and hematopoietic stem cell transplantation .

Rett syndrome is a X-linked neurodevelopmental disorder that predominantly affects girls and has a worldwide incidence of one in every 10,000 to 15,000 female births. The condition generally follows normal development in the first 6 to 18 months of age, followed by a period of regression in language and motor skills including repetitive stereotyped hand movements. Rett syndrome patients also have numerous comorbidities that are thought to contribute to a shortened lifespan. There is a high unmet medical need in Rett syndrome given no current treatment exists for the underlying cause of the disease. Loss-of-function mutations in the gene encoding the transcriptional regulator *methyl-CpG binding protein 2* (“MECP2”) account for most cases of Rett syndrome. Nonsense mutations in the *MECP2* gene account for approximately 30% of Rett syndrome cases. The most prominent nonsense mutations found in Rett syndrome, *R168X*, *R255X*, *R270X* and *R294X*, are all caused by a change of arginine to the stop codon, UGA.

### ***Status of Clinical Programs***

We have completed our Phase 1a and Phase 1b studies. In support of our Phase 2 studies currently underway, we have advanced our global health authority interactions and manufacturing activities.

#### ***Phase 1 Clinical Studies***

We completed a Phase 1 program in healthy volunteers designed to support studies of ELX-02 in patient populations in any indication caused by nonsense mutations and assess the safety of ELX-02. This phase of testing included a small number of healthy volunteers. The studies assessed the effects of ELX-02 on humans and measured bioavailability, excretion, safety and side effects, as well as the pharmacokinetics (what the body does to the drug) with increasing doses. Our Phase 1 studies included single ascending dose, or Phase 1a, and multiple ascending dose, or Phase 1b, studies.

The Phase 1a single ascending dose (“SAD”) study was completed in 2018 and the results were published in *Clinical Pharmacology in Drug Development* in January 2019. The SAD study included five doses of ELX-02 ranging from 0.3 mg/kg to 7.5 mg/kg in order to determine the pharmacokinetic profile and safety from single administration. The completed Phase 1a study enrolled healthy volunteers at Tel Aviv Sourasky Medical Center, Clinical Research Center (EL-001 (NCT02807961)) and at SGS Life Sciences Services, Clinical Pharmacology Unit (EL-006 (NCT03292302)). ELX-02 was shown to be generally well tolerated over the dose range of the SAD studies with no observations of renal toxicity. During the study, observations of transient adverse events resulting from ELX-02 administration were mild with the exception of a single moderate auditory adverse effect of interest but of unclear physiological significance and without clinical impact. Overall the results were supportive of continued clinical development.

During the first half of 2019, we completed a multiple ascending dose (MAD) study for ELX-02. The study was designed as a randomized, double-blinded, placebo-controlled, multiple dose escalating study in healthy subjects. The study was expanded to include additional cohorts to evaluate lower drug concentrations and/or administration schedule. In addition, we completed a renal study with ELX-02 in subjects with mild, moderate, and severe renal impairment. The results from the renal impairment study provide support for both continuing our clinical development programs and evaluating the suitability of our ERSG library for development in additional renal disorders, including ADPKD and cystinuria.

The results from the MAD trial were presented in 2019 at both the European Cystic Fibrosis 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.

#### ***Phase 2 Clinical Studies***

Currently, our Phase 2 clinical trial programs for our lead investigational drug candidate, ELX-02, are focused on development for cystic fibrosis and nephropathic cystinosis patients with diagnosed nonsense mutations. With ELX-02, we have completed a Phase 1 SAD trial at sites in Israel and Belgium, a MAD trial in Belgium and the

U.S., and a renal impairment study in the U.S. 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.

Our Phase 2 cystic fibrosis clinical trial program for ELX-02 is actively dosing patients in the U.S. and Israel and continuing to enroll patients at leading global investigator sites. We expect to achieve full enrollment and to report topline results during the first half of 2020. In the U.S., the Cystic Fibrosis Foundation is providing funding for a portion of the trial and we will form a joint program advisory group with the CF Foundation focused on the development of ELX-02 for cystic fibrosis. The TDN has sanctioned the Phase 2 study protocol (ClinicalTrials.gov Identifiers: NCT04126473 and NCT04135495), which is being conducted at TDN member sites.

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. In Europe and Israel, Professor Eitan Kerem, M.D., Head of the Division of Pediatrics, Children's Hospital, Hadassah Medical Center in Israel, is the global lead 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) and we are currently enrolling and dosing patients in both trials. During October 2019, we completed an interim CMC review meeting with 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.

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 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, 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.

### ***Status of Preclinical Programs***

We have completed a comprehensive series of preclinical studies to assess the safety, pharmacokinetics and pharmacology of ELX-02. In addition, we have verified read-through activity of additional library molecules and identified a set of promising candidate molecules to evaluate across new indications such as ADPKD. We have also continued our preclinical assessment of ELX compound activity in IRDs, validating read-through activity against several Usher Syndrome mutations and initiating ocular tolerability and IND-enabling studies. We are also progressing preclinical development of ELX compounds in ADPKD.

### ***Safety and Pharmacokinetic Studies of ELX-02***

A comprehensive toxicology program in accordance with the ICH guideline M3 (R2) was completed for ELX-02 to support clinical studies.

In definitive, 28-day, repeat-dose toxicity studies in rats and dogs, ELX-02 had little or no effect on body weight, food consumption, clinical signs of toxicity, ophthalmology, cardiovascular parameters, hematology or coagulation parameters. At dose levels higher than those intended for humans, ELX-02 has no cochlear toxicity as evidenced in anatomic and functional hearing studies in 28-day rat studies. We completed 3-month toxicology studies in juvenile rats and in young dogs, as well as chronic toxicology studies in these two species for 6- and 9-months, respectively. These studies showed no mortality and no significant in-life toxicity. ELX-02 was not genotoxic in the core battery of in vitro and in vivo genotoxicity assays. Collectively, these results support the

human lifetime use of ELX-02. ELX-02 is 98% bioavailable following sub-cutaneous administration. Additionally, ELX-02 does not undergo metabolism and is excreted unchanged almost exclusively via the urine.

### *Pharmacology Studies of ELX-02*

We have conducted a series of preclinical studies to demonstrate the primary pharmacodynamics of ELX-02 in several genetic disease indications. We have tested the translational read-through capabilities of ELX-02 *in vitro* and *in vivo*, in cells and in animal models of nonsense mutations.

We have shown the *in vitro* read-through activity of ELX-02 in an array of plasmids engineered to contain nonsense mutations of genetic diseases and in cell-based models of CF, cystinosis, DMD, MPS 1, and Rett syndrome.

In CF, ELX-02 induced about 30% of wild-type CFTR levels after 48 hours in heterozygous *G542/F508del* human bronchial epithelial cells. In the *G542X* transgenic mouse, ELX-02 showed an approximate 5-fold increase in CFTR activity compared to control after twice weekly treatment for four weeks with 60 mg/kg.

In order to evaluate read-through across a series of CF genotypes, we initiated a program to evaluate restoration of CFTR activity in patient-derived organoids. An organoid is a three-dimensional, multi-cellular *in vitro* system derived from stem cells isolated from rectal biopsies. When grown suspended in an extracellular matrix substrate, the cells adopt a polarized endothelial spheroid structure. CFTR protein localizes to the apical surface (the inner surface of the sphere) and is capable of driving chloride ions into the inner lumen. When an ion imbalance, relative to the ion concentration surrounding the organoid is present, water is driven into the inner lumen of the organoid as a means to equilibrate the concentration. This action results in the swelling of the organoid, a function measurable by high-content imaging. Similar to measuring CFTR conductivity in other systems, such as human bronchial epithelial cells, CFTR activity is stimulated in the organoid system with forskolin to produce cyclic adenosine monophosphate, a signaling molecule that stimulates CFTR channel activity. Organoids derived from nonsense mutation CF patients lack apparent swelling in this system due to their lack of functional CFTR. As a stem cell derived system, organoids are a renewable model permitting repeat testing from a single patient's biopsy. We partner with Hubrecht Organoid Technology (HUB), a not-for-profit organization founded out of the Hubrecht Institute, KNAW and University Medical Center Utrecht in the Netherlands to evaluate the nonsense-bearing CF patient organoids in its biobank. These studies demonstrate that across the most common CF causing nonsense alleles (*G542X*, *W1282X*, *R553X*, *R1162X*, *R1066C* and *E60X*) accounting for greater than 75% of the CF nonsense allele population, ELX-02 significantly increases CFTR function and *CFTR* mRNA in a dose dependent fashion. ELX-02 mediated CFTR function is observed in both homozygous and complex heterozygous nonsense bearing patient-derived organoids. ELX-02 is the first clinical development candidate molecule to demonstrate activity against these nonsense mutations in the organoid model system. Activity in the organoid swelling assay is dependent on both proper production and localization of functional CFTR protein; therefore, the activity observed with ELX-02 supports that the CFTR protein resulting from read-through of these mutations is both functional and localized to the correct cellular location. *CFTR* mRNA levels were monitored by NanoString, which demonstrated that *G542X* and *W1282X* CF organoids have the expected reduction in *CFTR* mRNA resulting from nonsense mediated decay. ELX-02 increased *CFTR* mRNA dose-dependently to the range observed in healthy organoids. While approved CF therapies lack efficacy against this type of CF mutation, organoids with responsive mutations (e.g. *G551D*, *F508del*) demonstrate activity in the organoid swelling assay in a manner that correlates with clinical responsiveness in informative endpoints such as FEV1 and sweat chloride change. When considering the ELX-02 results with the correlation reports of other approved molecules in this assay, the response mediated by ELX-02 read-through meets or exceeds the activity necessary to significantly improve FEV1 and sweat chloride concentration. We continue to evaluate new organoid genotypes in an effort to prioritize additional nonsense alleles for inclusion in future clinical trials.

Eloxx is a member of the European HIT-CF project consortium, a European Union funded preclinical and clinical research program evaluating the efficacy and safety of disease modifying drug candidates, including ELX-02, in Cystic Fibrosis (CF) patients with rare genetic mutations. The goal of the project is to investigate whether a positive response to therapies in a patient derived organoid can be predictive of clinical response in a controlled trial.

HIT-CF represents a new era in CF treatment and personalized medicine, as it has the potential to shift therapeutic trials from patients to the laboratory. HIT-CF has informed Eloxx that over 100 organoids from unique individuals with rare nonsense mutations have already been enrolled in the program and preclinical drug response

screening is ongoing. The HIT-CF consortium plans to complete the preclinical stage and initiate the clinical phase in 2020.

In cystinosis, ELX-02 increased read-through of the *CTNS W138X* mutation by 30-fold in a cell-free plasmid assay system. In primary homozygous *W138X* fibroblasts, ELX-02 led to a dose-dependent increase in normalized *CTNS* mRNA levels, consistent with a decrease in nonsense mediated mRNA decay. Functional protein restoration was supported in primary fibroblasts by demonstrating an ELX-02 mediated reduction in cellular cystine levels to wild-type levels. When combined with the standard of care, cysteamine, ELX-02 reduction in cellular cystine levels was additive. These data support that ELX-02 has the potential to impact the primary etiology of this ultra-rare disease.

In IRDs, a series of academic collaborations have shown that the candidate ELX compounds demonstrate dose-dependent increases in read-through of Usher Syndrome 2A and 1F mutations in a cellular plasmid-based reporter assay; they also show restoration of Usher 1C protein and harmonin. Upcoming studies will confirm these findings. Additionally, we are evaluating multiple ELX compounds for ocular tolerability, as measured by preservation of the electroretinogram and retinal histology, when delivered by intravitreal injection in rabbits. These studies demonstrate ELX compounds to have a favorable tolerability profile in comparison to the reference aminoglycoside, gentamicin. These findings are consistent with our hypothesis that the improved specificity for the eukaryotic cytosolic ribosome over the mitochondrial ribosome will reduce off-target associated toxicities.

#### *Ongoing Screening and IND Enabling Studies of Eloxx ERSG Library Compounds*

Screening of our ERSG library in new indications has provided a foundation for expansion into additional therapeutic efforts. In new indications, compounds are evaluated for read-through potential against prevalent gene-specific nonsense mutations, tissue exposure and functional evidence of the target protein's production. This data-centric approach enables selection of the ideal ERSG and establishes the preclinical assays required for our genetics-based personalized medicine strategy in new indications. We believe that one or more of these preclinical ERSG drug candidates are potentially suitable for clinical development and we are continuing to conduct pre-IND studies to evaluate their potential.

#### *Manufacturing Status*

We have completed the manufacture of the clinical drug product to support our full Phase 2 clinical trial program. We have further identified a commercial manufacturer and are engaged in process development and scale up activities required to support Phase 3 clinical development. During October 2019, we completed an interim CMC review meeting with 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.

### **Intellectual Property**

#### *Patent Portfolio*

Our licensed and owned patent portfolio includes three families of patents and applications relating to our lead compound, ELX-02 (formerly known as NB124), and over 40 other read-through inducing compounds. Each of these families is described briefly below.

With regard to ELX-02, our primary composition of matter coverage derives from a first family of patents and applications that we exclusively license through the Research and License Agreement with TRDF dated August 29, 2013. This family includes claims directed to ELX-02 and other read-through inducing compounds, as well as claims directed to pharmaceutical compositions of the disclosed compounds and methods of using the compounds and compositions to treat genetic disorders associated with premature stop codon mutations including cystic fibrosis, cystinosis, Duchenne's muscular dystrophy, ataxia-telangiectasia, Hurler syndrome, hemophilia A and B, Usher Syndrome, and Tay-Sachs. Patents that have issued or which may issue in the future from this family are currently expected to expire in 2031, not including any extensions of term for which we may be eligible that we may be granted. As of December 31, 2019, this family included issued patents in the U.S., Europe, Canada, Hong Kong, Israel and Japan, and pending applications in the U.S., Europe, Hong Kong, India, Israel and Japan.

We own two additional families that may provide additional protection for specific uses or methods of manufacturing ELX-02 beyond the current expiration date of the primary composition of matter patents. The first of these is directed to the use of ELX-02 and other read-through inducing compounds for the treatment of Rett syndrome. As of December 31, 2019, this family included pending applications in the U.S., Europe, Canada, Hong Kong, and Japan. Any patents issuing from this family are currently expected to expire in 2035, not including any extensions of term for which we may be eligible that we may be granted. The second is directed to methods for large-scale synthesis of ELX-02 and other read-through inducing compounds. As of December 31, 2019, this family includes pending applications in the U.S., Europe, Australia, Brazil, Canada, China, Hong Kong, Israel, India, and Japan. Any patents issuing from this family are currently expected to expire in 2038, not including any extensions of term for which we may be eligible that we may be granted.

Our other three families of licensed and owned patents and applications are directed to additional read-through inducing compounds. The first of these is exclusively licensed under the Research and License Agreement with TRDF, and includes claims directed to ELX-03 (formerly known as NB84) and other compounds, as well as claims directed to pharmaceutical compositions and methods of treating genetic disorders including cystic fibrosis, Duchenne muscular dystrophy, ataxia-telangiectasia, Hurler syndrome, hemophilia A, hemophilia B, Usher Syndrome, and Tay-Sachs. Patents that have issued or which may issue in the future from this family are currently expected to expire in 2027 and 2028, not including any extensions of term for which we may be eligible that we may be granted. As of December 31, 2019, this family included issued patents in the U.S., Europe, Canada, India, Israel and Japan, and pending applications in the U.S., Europe and Hong Kong.

The second and third of these families include claims directed to additional read-through inducing compounds and pharmaceutical compositions and uses thereof. As of December 31, 2019, one of these families included pending applications in the U.S., Europe, Australia, Brazil, Canada, China, Hong Kong, India, Israel and Japan. The other family includes pending applications in the U.S., Europe, Australia, Brazil, Canada, China, EAPO, Israel, India, Japan, South Korea, Mexico, Malaysia, New Zealand, and Singapore. Patents that may issue from these families are currently expected to expire in 2036 and 2038, respectively, not including any extensions of term for which we may be eligible that we may be granted.

#### **Patent Term Extension**

The term of a U.S. patent is 20 years from its earliest effective filing date, assuming all maintenance fees are paid, the patent has not been terminally disclaimed, and the patent has not been invalidated through administrative and/or court proceedings. The term of foreign patents varies but is generally also 20 years from the earliest effective filing date. In certain instances, the term of U.S. and certain foreign patents may be extended.

In the U.S., patent term may be extended in certain instances by patent term adjustment, or PTA, which compensates for administrative delays by the U.S. Patent & Trademark Office (USPTO) during examination of the patent. We have received a PTA for one of our exclusively licensed patents relating to ELX-01 and other related read-through inducing compounds, extending the expiration date of that patent from 2027 to 2028. However, we do not know whether any PTA will be granted for any of our future patents.

For pharmaceutical products that have received FDA approval, the term of a U.S. patent covering the approved product, a method of manufacturing the approved product, or an approved method of use of the product may be extended under the Drug Price Competition and Patent Term Restoration Act of 1984, referred to as the Hatch-Waxman Act, in certain instances if specific statutory and regulatory requirements are satisfied. The Hatch-Waxman Act provides for a patent term extension, or PTE, of up to five years as compensation for effective patent term lost during product development and the FDA regulatory review process. PTE is only available for the first approval of a particular product, the total patent term including the restoration period must not exceed 14 years from the date of FDA approval, and only one patent may be extended for a particular regulatory review period. In Europe, a similar mechanism exists for extending patent term up to five years through the grant of a Supplementary Protection Certificate (SPC) following European Medicines Agency (the “EMA”) approval. Similar regulatory extensions are available or may be available in the future in other jurisdictions. If and when ELX-02 or other of our read-through inducing compounds are approved by the FDA, EMA, or other foreign regulatory authorities, we will apply for these and other patent term extensions on patents covering the approved products, methods of use, or methods of manufacture if the patents are eligible for such extension. However, we cannot provide any assurance that such extensions will be granted for any of our currently issued or future patents.

## **Trade Secrets and Know-How**

With respect to our ERSG-based technology platform, we primarily rely on trade secrets and know-how to protect the proprietary nature of our platform. However, trade secrets and know-how can be difficult to protect. We seek to protect our proprietary technology and processes, in part, by confidentiality agreements with our employees, consultants, scientific advisors and contractors. We also seek to preserve the integrity and confidentiality of our data, know-how and trade secrets by maintaining physical security of our premises and physical and electronic security of our information technology systems. While we have confidence in these individuals, organizations and systems, agreements or security measures may be breached, and we may not have adequate remedies for any breach. In addition, our trade secrets may otherwise become known or be independently discovered by competitors. To the extent that our consultants, contractors or collaborators use intellectual property owned by others in their work for us, disputes may arise as to the rights in related or resulting know-how and inventions.

## **License Agreements**

### ***Research and License Agreement with Technion Research and Development Foundation Ltd.***

On August 29, 2013, we entered into a license agreement with TRDF, which was further amended and addended to reflect, inter alia, the assignment of patents and extension of research periods, with respect to certain technology relating to aminoglycosides and the redesign of aminoglycosides for the treatment of human genetic diseases caused by premature stop mutations and further results of the research of the technology, in order to develop and commercialize products based on such technology. The license agreement provides us with an exclusive, worldwide, non-transferrable license, with a right to grant sublicenses, and royalty-bearing licenses to the TRDF inventions, TRDF patent rights, TRDF's interest in the joint inventions and joint patent rights, and certain materials and research results owned by TRDF, solely with respect to products in the field of prevention, diagnosis or treatment of any human disease or condition therefor. In return for the license we will pay TRDF (i) milestone payments with respect to each licensed product upon the achievement of certain pre-defined goals by us or one of our sublicensees as follows: \$100,000 upon first dosing of a patient in a Phase II clinical study; \$1,000,000 upon first dosing of a patient in a pivotal study; and \$5,000,000 upon first filing of a new drug application (NDA); (ii) certain royalties in the low- to mid-single-digit percentages of all net sales (subject to change in the case of (a) sublicensing to a big pharmaceutical or biotechnology company, or (b) payment of royalties to third parties, or (c) commercialization by a third party of an authorized generic to a licensed product); and (iii) a low- to mid-double-digit percentage of any non-royalty sublicense income. In addition to the milestone payments, we undertook to annually fund the research activities under the license, currently in the estimated annual amount of \$0.1 million per year. The license agreement further provides TRDF with an additional pre-emptive right, in force until the first exit event, to invest an amount equal to up to 5% of the amount contemplated to be raised in a proposed investment. TRDF is also entitled, until the closing of an exit event, to appoint an observer to the board under certain restrictions such as confidentiality or conflict of interest. Furthermore, we will reimburse TRDF for all patent filing expenses as of the effective date of the license agreement and for past patent filing expenses in the amount of several hundred thousand New Israeli Shekels upon the occurrence of certain conditions.

Under the license agreement, TRDF reserved the right, for itself, the Technion and other not-for-profit research organizations to utilize the technology solely for educational purposes. Furthermore, Professor Baasov, the principal investigator, had ongoing research programs involving covered compounds (as defined in the agreement) that are being funded by the National Institute of Health in the U.S., or the NIH, under sub-awards from the University of Alabama and the University of Michigan, and it is possible that such research programs will overlap with the research conducted according to the terms of our agreement. In the case of any such overlap, the work product of such research will be subject to the terms and conditions of such sub-awards, including certain obligations under 35 U.S.C. §§ 200-212 or 37 C.F.R. § 401 et seq. in the case of any TRDF inventions that are also "subject invention" as defined in 35 U.S.C. § 201.

The license agreement shall continue in full force and effect on a product-by-product and country-by-country basis until the expiration of all payment obligations for any such licensed product as described above. Upon the expiration, we will have a fully paid-up, worldwide non-exclusive, perpetual, irrevocable license (with the right to grant sublicenses) to use certain materials and the research results, solely with respect to products in the field of prevention, diagnosis or treatment of any human disease or condition.

## **Manufacturing**

ELX-02 is manufactured under current Good Manufacturing Practice (“cGMP”) conditions and is formulated as a sterile frozen liquid or lyophilized powder in glass vials. ELX-02 is administered by parenteral subcutaneous (SC) injection after appropriate dilution or reconstitution, as required.

We do not own or operate manufacturing or distribution facilities for the production of clinical quantities of ELX-02 or for our other preclinical product candidates. We currently rely, and expect to continue to rely, on third parties for the manufacture, packaging, labeling and distribution of clinical supplies of ELX-02 as well as any other candidate that we may develop.

We engage separate manufacturers for drug substance and drug product. We have a relationship with a manufacturer that is capable of providing fill and finish services for our clinical product at the current scale. To support later clinical trials, transfer of the manufacturing and release to a manufacturer with higher lot scale capacity will be needed for our clinical product.

All of our current drug candidates are organic compounds of low molecular weight. We have selected our lead compounds not only on the basis of their potential efficacy and safety but also for their ease of synthesis and reasonable cost of their starting materials. ELX-02 is manufactured in reliable and reproducible synthetic processes. We currently use a single third-party manufacturing source for the production of a key raw material, produced by bacterial fermentation; however, we have identified several other acceptable sources for this production.

We currently obtain clinical supplies of ELX-02 from third-party manufacturers pursuant to agreements that include specific supply timelines and volume expectations. If a manufacturer should become unavailable to us for any reason, we would seek to obtain supply from another manufacturer engaged by us for the applicable product or service. In the event that we were unable to procure the applicable supply from a currently qualified manufacturer, we believe that there are a number of potential replacements for each of our outsourced services; however, we would likely experience delays in our ability to supply ELX-02 in advancing our clinical trials while we identify and qualify replacement suppliers.

## **Government Regulation**

### ***Drug Development and Approval in the United States***

The preclinical studies and clinical testing, manufacture, labeling, storage, record keeping, advertising, promotion, export, and marketing, among other things, of our product candidates are subject to extensive regulation by governmental authorities in the U.S., the EU and other territories. In the U.S., pharmaceutical products are regulated by the FDA under the Federal Food, Drug, and Cosmetic Act (the “FDCA”) and other laws, including, in the case of biologics, the Public Health Service Act. Failure to comply with FDA requirements, both before and after product approval, may subject us and/or our partners, contract manufacturers, and suppliers to administrative or judicial sanctions, including FDA refusal to approve applications, warning letters, product recalls, product seizures, total or partial suspension of production or distribution, fines and/or criminal prosecution.

The process for obtaining regulatory approval to market a medicine is expensive, often takes many years, and can vary substantially based on the type, complexity, and novelty of the product candidates involved. The steps required before a drug may be approved for marketing of an indication in the U.S. generally include:

- (a) preclinical laboratory tests and animal tests;
- (b) submission to the FDA of an IND application for human clinical testing, which must become effective before human clinical trials may commence;
- (c) adequate and well-controlled human clinical trials to establish the safety and efficacy of the product candidate for its intended use;
- (d) submission to the FDA of an NDA;
- (e) FDA pre-approval inspection of the manufacturing and clinical study sites identified in the NDA; and
- (f) FDA review and approval of the NDA.

Preclinical studies include laboratory evaluation of product chemistry and formulation, as well as toxicological and pharmacological animal studies to assess the potential safety and efficacy of the product candidates. Preclinical safety tests intended for submission to FDA must be conducted in compliance with FDA’s

current Good Laboratory Practice (“cGLP”) regulations and the U.S. Department of Agriculture’s Animal Welfare Act. The results of the preclinical tests, together with manufacturing information and analytical data, are submitted to the FDA as part of an IND application that must become effective before human clinical trials may be commenced. The IND will automatically become effective 30 days after receipt by the FDA, unless the FDA before that time raises concerns about the drug candidate or the conduct of the trials as outlined in the IND. The IND sponsor and the FDA must resolve any outstanding concerns before clinical trials can proceed. We cannot assure you that submission of an IND will result in FDA authorization to commence clinical trials or that once commenced, other concerns will not arise. The FDA may stop the clinical trials at any time by placing them on “clinical hold” because of concerns about the safety of the product being tested, or for other reasons.

Clinical trials involve the administration of the investigational product to healthy volunteers or to patients, under the supervision of qualified principal investigators. The conduct of clinical trials is subject to extensive regulation, including compliance with the FDA’s bioresearch monitoring regulations and current Good Clinical Practice (“cGCP”) requirements, which establish standards for conducting, recording data from, and reporting the results of clinical trials, and are intended to assure that the data and reported results are credible and accurate, and that the rights, safety, and well-being of study participants are protected.

Clinical trials must be conducted in accordance with protocols that detail the objectives of the study, the criteria for determining subject eligibility and ineligibility, the dosing plan, patient monitoring requirements, timely reporting of adverse events, and other elements necessary to ensure patient safety, and any efficacy criteria to be evaluated. Each protocol must be submitted to FDA as part of the IND; further, each clinical study at each clinical site must be reviewed and approved by an independent institutional review board, or IRB, prior to the recruitment of subjects. The IRB’s role is to protect the rights and welfare of human subjects involved in clinical studies by evaluating, among other things, the potential risks and benefits to subjects, processes for recruiting subjects and obtaining informed consent, monitoring of data to ensure subject safety, and provisions to protect the subjects’ privacy. Foreign studies conducted under an IND application must meet the same requirements that apply to studies being conducted in the U.S. Data from a foreign study not conducted under an IND may be submitted in support of an NDA if the study was conducted in accordance with cGCP and FDA is able to validate the data.

Clinical trials are typically conducted in three sequential phases, but the phases may overlap, and different trials may be initiated with the same drug candidate within the same phase of development in similar or differing patient populations. Phase I studies may be conducted in a limited number of patients but are usually conducted in healthy volunteer subjects. The drug is usually tested for safety and, as appropriate, for absorption, metabolism, distribution, excretion, pharmacokinetics and pharmacodynamics. Phase II usually involves studies in a larger, but still limited patient population to preliminarily evaluate the efficacy of the drug candidate for specific, targeted indications; to determine dosage tolerance and optimal dosage; and to identify possible short-term adverse effects and safety risks. Phase III trials are undertaken to gather additional information to evaluate the product’s overall risk-benefit profile, and to provide a basis for physician labeling. Phase III trials evaluate clinical efficacy of a specific endpoint and test further for safety within an expanded patient population at geographically dispersed clinical study sites. Phase I, Phase II or Phase III testing might not be completed successfully within any specific time period, if at all, with respect to any of our product candidates. Results from one trial are not necessarily predictive of results from later trials. Furthermore, the FDA, sponsor or IRB may suspend clinical trials at any time on various grounds, including a finding that the subjects or patients are being exposed to an unacceptable health risk.

We must register each controlled clinical trial, other than Phase I trials, on a website administered by the NIH (<http://clinicaltrials.gov>). Registration must occur not later than 21 days after the first patient is enrolled, and the submission must include descriptive information (e.g., a summary in lay terms of the study design, type and desired outcome), recruitment information (e.g., target number of participants and whether healthy volunteers are accepted), location and contact information, and other administrative data (e.g., FDA identification numbers). Within one year of a trial’s completion, information about the trial including characteristics of the patient sample, primary and secondary outcomes, trial results written in lay and technical terms, and the full trial protocol must be submitted to the ClinicalTrials.gov databank. The results information is posted to the website unless the drug has not yet been approved, in which case the NIH posts the information shortly after approval. An NDA, and certain other submissions to the FDA, require certification of compliance with these clinical trials database requirements. There are proposals to expand the applicability of these registration requirements to additional studies.

The results of the preclinical studies and clinical trials, together with other detailed information, including information on the manufacture and composition of the product and proposed labeling for the product, are submitted to the FDA as part of an NDA requesting approval to market the product candidate for a proposed indication. Under the Prescription Drug User Fee Act, as amended, the fees payable to the FDA for reviewing an NDA, as well as

annual fees for commercial manufacturing establishments and for approved products, can be substantial. The NDA review fee alone can exceed \$2.45 million subject to certain limited deferrals, waivers and reductions that may be available to a qualifying NDA sponsor for a specific NDA. Each NDA submitted to the FDA for approval is typically reviewed for administrative completeness and reviewability within sixty days following submission of the application. If the FDA finds the NDA sufficiently complete, the FDA will “file” the NDA, thus triggering a full review of the application. The FDA may refuse to file any NDA that it deems incomplete or not properly reviewable at the time of submission. Current FDA performance goals provide for action on a standard application within 10 months of the 60-day filing date. The FDA, however, may not approve a drug within these established timeline goals and its review clock for a particular NDA is subject to change from time to time because the review process is often significantly extended by FDA requests for additional information or clarification. We may encounter difficulties or unanticipated costs in our efforts to secure necessary FDA approvals, which could delay or preclude us from marketing our product candidates. As part of its review, the FDA may refer applications for novel drug products or drug products that present difficult questions of safety or efficacy to an advisory committee composed of outside experts, for evaluation and a recommendation as to whether the application should be approved and under what conditions. The advisory committee process may cause delays in the approval timeline. The FDA is not bound by the recommendation of an advisory committee, but it considers such recommendations carefully, particularly after negative recommendations or limitations, when making drug approval decisions.

Further, the outcome of the FDA review process, even if generally favorable, may not be an actual approval but instead a “complete response letter” communicating the FDA’s decision not to approve the application at that time, outlining the deficiencies in the NDA that need to be addressed in order to be eligible for approval, and identifying what information and/or data (including any additional preclinical or clinical data) is required before the application can be approved. Even if such additional information and data are submitted, the FDA may decide that the NDA still does not meet the standards for approval. Data from clinical trials are not always conclusive and the FDA may interpret data differently than we do.

The development testing and approval processes require substantial time, effort and financial resources, and each may take years to complete. Data obtained from clinical trials are not always conclusive and may be susceptible to varying interpretations, which could delay, limit or prevent regulatory approval. The FDA may not grant approval on a timely basis, or at all.

The FDA typically will inspect one or more clinical sites to assure compliance with cGCP before approving an NDA. The FDA also will inspect the facility or the facilities at which the product is manufactured before the NDA is approved to assure compliance with cGMP. The FDA will not approve the product unless cGCP and cGMP compliance is satisfactory. The FDA may also take into account results of inspections performed by certain counterpart foreign regulatory agencies in assessing compliance with cGCP or cGMP. The FDA has entered into international agreements with foreign agencies, including the EMA, in order to facilitate this type of information sharing. If the FDA determines the application, clinical sites, manufacturing process or manufacturing facilities are not acceptable, it will outline the deficiencies in the NDA submission and often will request additional testing or information.

The FDA may deny approval of an NDA if applicable statutory or regulatory criteria are not satisfied, or may require additional testing or information, which can delay the approval process. FDA approval of any application may include many delays or may never be granted. Notwithstanding the submission of any requested additional information, the FDA ultimately may decide that an NDA does not satisfy the regulatory criteria for approval. If a product is approved, the approval will impose limitations on the indicated use or uses for which the product may be marketed, may require that warning statements be included in the product labeling, and may require that additional studies be conducted following approval as a condition of the approval. The FDA may limit the indications for use, approve narrow labeling relegating a drug to second-line or later-line use, add limitations of use to the labeling or place other conditions on approvals, which could restrict the marketing of an approved product. Further, the FDA may require that certain contraindications, warnings or precautions be included in the product labeling. FDA also may impose restrictions and conditions on product distribution, prescribing or dispensing in the form of a Risk Evaluation Mitigation Strategy (“REMS”) to ensure the safe use of the drug, or otherwise limit the scope of any approval. In determining whether a REMS is necessary, the FDA must consider the size of the population likely to use the drug, the seriousness of the disease or condition to be treated, the expected benefit of the drug, the duration of treatment, the seriousness of known or potential adverse events, and whether the drug is a new molecular entity. A REMS may be required to include various elements, such as a medication guide or patient package insert, a communication plan to educate health care providers of the drug’s risks, limitations on who may prescribe or

dispense the drug, or other measures that the FDA deems necessary to assure the safe use of the drug. To market an approved product for other indicated uses, or to make certain manufacturing or other changes, requires FDA review and approval of an NDA Supplement or new NDA, which may themselves necessitate further clinical testing, and the payment of applicable review fees. Additional post-marketing testing and surveillance to monitor the safety or efficacy of a product may be required. In addition, new government requirements may be established that could delay or prevent regulatory approval of our product candidates under development.

Under the Pediatric Research Equity Act of 2003 (“PREA”), NDAs or supplements to NDAs must contain data to assess the safety and effectiveness of the drug for the claimed indications in all relevant pediatric subpopulations and to support dosing and administration for each pediatric subpopulation for which the drug is determined by the FDA to be safe and effective. The FDA may grant deferrals for submission of data or full or partial waivers, to a qualifying NDA sponsor. Unless otherwise required by regulation, PREA does not apply to any drug for an indication for which orphan designation has been granted. As the FDA has not issued regulations applying PREA to orphan-designated indications, submission of a pediatric assessment is not presently required for an application to market a product for an orphan-designated indication. However, PREA compliance may be required if approval is sought for other indications for which the drug has not received orphan designation.

### ***Post-approval Requirements***

After FDA approval of a product is obtained, we will be required to comply with a number of post-approval requirements, including, among other things, establishment registration and product listing, record-keeping requirements, reporting certain adverse reactions and production problems to the FDA, providing updated safety and efficacy information, and complying with requirements concerning advertising and promotional labeling. As a condition of approval of an NDA, the FDA may require the applicant to conduct additional clinical trials or other post-market testing and surveillance to further monitor and assess the drug’s safety and efficacy.

The FDA regulates strictly the marketing, labeling, advertising and promotion of drug products that are placed on the market. Although physicians may prescribe a drug for off-label uses, manufacturers may only promote for the approved indication(s) and in accordance with the approved labeling. The FDA and other agencies actively enforce the laws and regulations prohibiting the promotion of off-label uses and any other false or misleading promotion. Failure to comply with the laws and regulations governing advertising and promotion can have negative consequences, including adverse publicity, warning and untitled letters from the FDA, requests for corrective advertising or communications with doctors, and civil penalties or criminal prosecution.

In addition, the distribution of approved prescription pharmaceutical products is subject to the Prescription Drug Marketing Act (“PDMA”), which regulates the distribution of drugs and drug samples at the federal level and sets minimum standards for the registration and regulation of drug distributors by the states. Similarly, the Drug Supply Chain Security Act (“DSCSA”) regulates the distribution of prescription pharmaceutical drugs, requiring implementation of an electronic interoperable system to identify and trace each prescription drug at the saleable unit level through the distribution system. The DSCSA also imposes obligations on drug manufacturers related to suspect product identification and removal, verification, dealing only with authorized trading partners, and other elements. The DSCSA is planned to be incrementally implemented over a 10-year period, with serialization of prescription drug products distributed in the U.S. effective November 27, 2017 for drug manufacturers. The PDMA, DSCSA, and state laws limit the distribution of prescription pharmaceutical product samples and impose requirements to ensure accountability in distribution.

Also, quality control and manufacturing procedures must continue to conform to cGMP after approval. The FDA periodically inspects manufacturing facilities post-approval to assess continued compliance with cGMP, which imposes certain procedural and recordkeeping requirements. Accordingly, manufacturers must continue to expend time, money and effort in the area of process and quality control to maintain compliance with cGMP and other aspects of regulatory compliance.

We rely, and expect to continue to rely, on third parties for the production of clinical and any future commercial quantities of our product candidates. Future FDA inspections may identify compliance issues at our facilities or at the facilities of our contract manufacturers that may disrupt production or distribution or require substantial resources to correct. In addition, discovery of problems with a product or the failure to comply with applicable requirements may result in restrictions on a product, manufacturer or holder of an approved NDA,

including withdrawal or recall of the product from the market or other voluntary, FDA-initiated or judicial action that could delay or prohibit further marketing.

Once approval is granted, the FDA may withdraw the approval if compliance with regulatory requirements is not maintained or if issues bearing on the product's safety or efficacy are discovered. Newly discovered or developed safety or effectiveness data or other information may also necessitate changes to a product's approved labeling, including the addition of new warnings and contraindications, and also may require the implementation of other risk management measures. New government requirements, including those resulting from new legislation, may be established that could delay or prevent FDA approval of our products under development or negatively impact the marketing of any future approved products.

### ***Orphan Drug Designation***

We have received orphan drug designation from the FDA for ELX-02 for the treatment of MPS I for the treatment of Rett syndrome, and for the treatment of cystinosis. The FDA may grant orphan drug designation to drugs intended to treat a "rare disease or condition," which is defined as a disease or condition that affects fewer than 200,000 individuals in the U.S., or more than 200,000 individuals in the U.S. and for which there is no reasonable expectation that the cost of developing and making available in the U.S. a drug for this type of disease or condition will be recovered from sales in the U.S. for that drug. Orphan drug designation must be requested before applying for marketing approval. Orphan drug designation does not convey any advantage in, or shorten the duration of, the regulatory review and approval process. Orphan drug designation can provide opportunities for grant funding towards clinical trial costs, tax advantages and FDA user-fee benefits. In addition, if a product which has an orphan drug designation subsequently receives the first FDA approval for the indication for which it has such designation, the product is entitled to orphan drug exclusivity, which means the FDA may not approve any other application to market the same drug for the same indication for a period of seven years, except in limited circumstances, such as a showing of clinical superiority to the product with orphan exclusivity. Competitors may receive approval of different drugs or biologics for the indication(s) for which the orphan product has exclusivity.

### ***Rare Pediatric Disease Designation and Priority Review Voucher***

Some orphan drugs may also qualify for designation as a "rare pediatric disease" under Section 529 of the FDCA. Section 529 is similar to the Orphan Drug Act, as both require that the "rare disease or condition" affect fewer than 200,000 persons in the U.S. In the Advancing Hope Act of 2016, Section 529 was changed so that the "rare pediatric disease" must also meet the additional criteria of being a serious or life-threatening disease in which the serious or life-threatening manifestations primarily affect individuals aged from birth to 18 years, including age groups often called neonates, infants, children, and adolescents. Under Section 529 of the FDCA, the FDA will award priority review vouchers to sponsors of rare pediatric disease product applications that meet these criteria. Under this program, a sponsor who receives an approval for a drug or biologic for a "rare pediatric disease" may qualify for a voucher that can be redeemed to receive a priority review of a subsequent marketing application for a different product.

### ***Hatch-Waxman Regulatory Exclusivity***

Market and data exclusivity provisions under the FDCA can delay the submission or the approval of certain applications for competing products. The FDCA provides a five-year period of non-patent data exclusivity within the U.S. to the first applicant to gain approval of an NDA for a new chemical entity. A drug is a new chemical entity if the FDA has not previously approved any other new drug containing the same active moiety. During the exclusivity period, the FDA generally may not accept for review an abbreviated new drug application ("ANDA") or a 505(b)(2) NDA submitted by another company that references the previously approved drug. An ANDA or 505(b)(2) NDA may be submitted after four years if it contains a certification of patent invalidity or non-infringement.

For some applications that do not qualify for five-year exclusivity, the FDCA provides a shorter three-year period of market exclusivity. Three-year exclusivity applies to an NDA, 505(b)(2) NDA, or supplement to an existing NDA or 505(b)(2) NDA if new clinical investigations, other than bioavailability studies, that were conducted or sponsored by the applicant, are deemed by the FDA to be essential to the approval of the application, for example, for new indications, dosages, strengths or dosage forms of an existing drug. This three-year exclusivity covers only the conditions of use associated with the new clinical investigations and, as a general matter, does not prohibit the FDA from approving ANDAs or 505(b)(2) NDAs for generic versions of the original, unmodified drug.

product. Five-year and three-year exclusivity will not delay the submission or approval of a full NDA; however, an applicant submitting a full NDA would be required to conduct or obtain a right of reference to all of the preclinical studies and adequate and well-controlled clinical trials necessary to demonstrate safety and effectiveness.

### **Pediatric Exclusivity**

Pediatric exclusivity is another type of non-patent market exclusivity in the U.S. and, if granted, provides for the attachment of an additional six months of market protection to the term of any existing Orange Book-listed patents or regulatory exclusivity, including the non-patent exclusivity periods described above. This six-month exclusivity may be granted based on the voluntary completion of a pediatric study or studies in accordance with an FDA-issued “Written Request” for such a study or studies.

### **Regulation Outside the United States**

In order to market any product outside of the U.S., we would need to comply with numerous and varying regulatory requirements of other countries regarding safety and efficacy and governing, among other things, clinical trials, marketing authorization, commercial sales and distribution of any future approved products. Whether or not we obtain FDA approval for a product, we would need to obtain the necessary approvals by the comparable regulatory authorities of foreign countries before we can commence clinical trials or marketing of the product in those countries. The approval process varies from country to country and can involve additional product testing and additional administrative review periods. The time required to obtain approval in other countries might differ from and be longer than that required to obtain FDA approval. Regulatory approval in one country does not ensure regulatory approval in another, but a failure or delay in obtaining regulatory approval in one country may negatively impact the regulatory process in others.

### **Regulation in the European Union**

We have obtained an orphan medicinal product designation from the European Commission, following an evaluation by the EMA’s Committee for Orphan Medicinal Products, for ELX-02 for the treatment of MPS I and for the treatment of cystic fibrosis. The European Commission can grant orphan medicinal product designation to products for which the sponsor can establish that it is intended for the diagnosis, prevention, or treatment of (1) a life-threatening or chronically debilitating condition affecting not more than five in 10,000 people in the EU, or (2) a life-threatening, seriously debilitating or serious and chronic condition in the EU and that without incentives it is unlikely that sales of the drug in the EU would generate a sufficient return to justify the necessary investment. In addition, the sponsor must establish that there is no other satisfactory method approved in the EU of diagnosing, preventing or treating the condition, or if such a method exists, the proposed orphan drug will be of significant benefit to patients. Orphan drug designation is not a marketing authorization. It is a designation that provides a number of benefits, including fee reductions, regulatory assistance, and the possibility to apply for a centralized EU marketing authorization, as well as 10 years of market exclusivity following a marketing authorization. During this market exclusivity period, neither the EMA, nor the European Commission nor the Member States can accept an application or grant a marketing authorization for a “similar medicinal product.” A “similar medicinal product” is defined as a medicinal product containing a similar active substance or substances as contained in an authorized orphan medicinal product, and which is intended for the same therapeutic indication. The market exclusivity period for the authorized therapeutic indication may be reduced to six years if, at the end of the fifth year, it is established that the orphan designation criteria are no longer met, including where it is shown that the product is sufficiently profitable not to justify maintenance of market exclusivity. In addition, a competing similar medicinal product may in limited circumstances be authorized prior to the expiration of the market exclusivity period, including if it is shown to be safer, more effective or otherwise clinically superior to our product. Our product can lose orphan designation, and the related benefits, prior to us obtaining a marketing authorization if it is demonstrated that the orphan designation criteria are no longer met.

### **Overview of Application Process**

To obtain regulatory approval of a drug under the EU’s regulatory systems and authorization procedures, an applicant may submit a Marketing Authorization Application (“MAA”) under a centralized, decentralized, or national procedure. The centralized procedure is compulsory for certain medicinal products, including orphan medicinal products, like ELX-02 and medicinal products produced by certain biotechnological processes, and optional for certain other innovative products. The centralized procedure enables applicants to obtain a marketing

authorization that is valid in all EU member states based on a single application. Under the centralized procedure, the EMA’s Committee for Human Medicinal Products (“CHMP”) is required to adopt an opinion on a valid application within 210 days, excluding clock stops, during which additional written or oral information is to be provided by the applicant in response to questions. More specifically, on day 120 of the procedure, once the CHMP has received the preliminary assessment reports and opinions from the rapporteur and co-rapporteur, the CHMP prepares a list of potential outstanding issues, referred to as “other concerns” or “major objections.” These are sent to the applicant together with CHMP’s recommendation. The CHMP can make one of two recommendations: (1) the marketing authorization could be granted provided that satisfactory answers are given to the “other concerns” and/or “major objections” identified and that all conditions outlined in the list of outstanding issues are implemented and complied with; or (2) the product is not approvable since there are “major objections.”

Applicants have three months from the date of receiving the potential outstanding issues to respond to the CHMP and can request a three-month extension if necessary. The granting of a marketing authorization will depend on the recommendations and potential major objections identified by the CHMP as well as the ability of the applicant to adequately respond to these findings. An accelerated assessment can be granted by the CHMP in exceptional cases, when a medicinal product is expected to be of major public health interest, in particular from the viewpoint of therapeutic innovation. In this circumstance, the EMA ensures that the opinion of the CHMP is given within 150 days. After the adoption of the CHMP opinion, a decision on the MAA must be adopted by the European Commission, after consulting the EU member states, which in total should be completed in 67 days.

An applicant for an MAA may request a re-examination in the event of a negative opinion, in connection with which CHMP appoints new rapporteurs. Within 60 days of receipt of the negative opinion, the applicant must submit a document explaining the basis for its request for re-examination. The CHMP has 60 days to consider the applicant’s request for re-examination. The applicant may request an oral explanation before the CHMP, which is routinely granted, following which CHMP will adopt a final opinion. The final opinion, whether positive or negative, is published by the CHMP shortly following the CHMP meeting at which the oral explanation takes place.

#### *Conditional Marketing Authorizations*

In specific circumstances, EU legislation enables applicants to obtain a marketing authorization on a conditional basis prior to obtaining the comprehensive clinical data required for an application for a full marketing authorization. Such conditional approvals may be granted for products designated as orphan medicinal products, if (1) the risk-benefit balance of the product is positive, (2) it is likely that the applicant will be in a position to provide the required comprehensive clinical trial data, (3) the product fulfills unmet medical needs, and (4) the benefit to public health of the immediate availability on the market of the medicinal product concerned outweighs the risk inherent in the fact that additional data are still required. A conditional marketing authorization may contain specific obligations to be fulfilled by the marketing authorization holder, including obligations with respect to the completion of ongoing or new studies, and with respect to the collection of pharmacovigilance data. Conditional marketing authorizations are valid for one year, and may be renewed annually, if the risk-benefit balance remains positive, and after an assessment of the need for additional or modified conditions and/or specific obligations. The timelines for the centralized procedure described above also apply with respect to the review by the CHMP of applications for a conditional marketing authorization. The granting of a conditional marketing authorization will depend on the applicant’s ability to fulfill the conditions imposed within the agreed upon deadline.

#### *Variations to Conditional Marketing Authorizations*

After the granting of a conditional marketing authorization, the marketing authorization holder may apply to vary the conditional marketing authorization under a variation procedure. In the case of the introduction of an additional therapeutic indication, the timeframe for the variation procedure for the initial assessment of the dossier is generally 90 days (plus up to 20 days for validation).

In the framework of a variation application assessment procedure, however, the EMA may send one or more requests for supplementary information to the marketing authorization holder, requiring that additional information be provided by the marketing authorization holder to support its variation application. Such supplementary requests will be sent together with a timetable stating the date by when the marketing authorization holder must submit the requested data and, where appropriate, the extended evaluation period to be applied to such variation procedure. The 90-day variation procedure may be suspended for up to three months for the marketing authorization holder to submit its responses to such supplementary requests. The marketing authorization holder will be notified of the outcome of the CHMP’s assessment of the variation procedure within 15 days from the adoption of the CHMP opinion. If unfavorable, the CHMP opinion may be subject to a re-examination procedure upon the marketing

authorization holder's request. This may imply an additional minimum two-month procedure. If the CHMP opinion is favorable, the European Commission will vary the marketing authorization to introduce the additional therapeutic indication within approximately two months from the receipt of the final CHMP opinion.

#### *Additional Requirements and Considerations*

Prior to obtaining a marketing authorization in the EU, applicants have to demonstrate compliance with all measures included in an EMA-approved Pediatric Investigation Plan ("PIP"), covering all subsets of the pediatric population, unless the EMA has granted (1) a product-specific waiver, (2) a class waiver, or (3) a deferral for one or more of the measures included in the PIP. In the case of orphan medicinal products, completion of an approved PIP can result in an extension of the aforementioned market exclusivity period from ten to twelve years.

In the EU, independently generated data submitted as part of a full marketing authorization application dossier are protected by regulatory data protection ('data exclusivity') for a period of eight years from the granting of a marketing authorization for a 'reference product'. This means that for a period of eight years, competent authorities may not accept marketing authorization applications that rely on the independently generated data in the marketing authorization dossier of the reference product. Generic medicinal products that rely on the independently generated data of the reference product may not be placed on the market for 10 years from the granting of the initial marketing authorization for the reference medicinal product. These periods of data exclusivity and market exclusivity do not prevent other companies from obtaining a marketing authorization based on their own independently generated data.

If we are able to obtain a marketing authorization for ELX-02 for any indication in the EU, we will be required to comply with a range of requirements applicable to the manufacturing, marketing, promotion and sale of medicinal products. We must, for example, comply with the EU's stringent pharmacovigilance or safety reporting rules, pursuant to which post-authorization studies and additional monitoring obligations can be imposed. Other requirements relate to, for example, the manufacturing of products and active pharmaceutical ingredients in accordance with good manufacturing practice standards. Competent authorities of EU member states may conduct inspections to verify our compliance with applicable requirements, and we will have to continue to expend time, money and effort to remain compliant. Non-compliance with EU requirements regarding safety monitoring or pharmacovigilance, and with requirements related to the development of products for the pediatric population, can also result in significant financial penalties in the EU. Similarly, failure to comply with the EU's requirements regarding the protection of individual personal data can also lead to significant penalties and sanctions. Individual EU member states may also impose various sanctions and penalties in case we do not comply with locally applicable requirements.

Off-label promotion of medicinal products is prohibited in the EU. The applicable laws at the EU level and in the individual EU member states also prohibit the direct-to-consumer advertising of prescription-only medicinal products. Violations of the rules governing the promotion of medicinal products in the EU could be penalized by administrative measures, fines and imprisonment. These laws may further limit or restrict our promotional activities with health care professionals. In addition, legislation adopted at the EU level and by individual EU member states require that promotional materials and advertising in relation to medicinal products comply with the product's Summary of Product Characteristics ("SmPC"), as approved by the competent authorities. The SmPC is the document that provides information to physicians concerning the safe and effective use of the medicinal product. Promotion of indications not covered by the SmPC is specifically prohibited.

The EMA is responsible for coordinating inspections to verify compliance with the principles of GCP, cGMP, GLP, and good pharmacovigilance practice ("GVP"). These inspections are also intended to verify compliance with other aspects of the supervision of authorized medicinal products in use in the EU. The EMA coordinates any inspection requested by the CHMP in connection with the assessment of MAAs. Inspections may be routine or triggered by issues arising during the assessment of the dossier or by other information, such as previous inspection experience. Inspections usually are requested during the initial review of an MAA but could also arise post-authorization.

Inspectors are drawn from member states of the EU and the European Economic Area. Following an inspection, the inspectors provide a written inspection report to the inspected site or applicant and provide an opportunity for response. Some inspection reports require follow-up and may result in additional adverse consequences due to critical or major findings. The inspectors and the CHMP will comment on any response from an inspected site or applicant and may monitor future compliance with any proposed corrective action plan.

Possible consequences of critical and major findings resulting from GCP inspections include rejection of clinical trial data, causing significant delays in obtaining final marketing authorization, or other direct action by national regulatory authorities.

### *Early Access Programs*

Many jurisdictions around the world allow the supply of unauthorized medicinal products in the context of strictly regulated and exceptional early access programs, and some countries may provide reimbursement for drugs provided in the context of such programs. In the EU, the legal basis for early access programs, also referred to as named-patient and compassionate use programs, is set out in the EU legislation regulating the authorization, manufacture, distribution and marketing of medicinal products. Detailed regulatory requirements applicable to early access programs have been adopted and implemented by EU member states in their national laws. The promotion, advertising and marketing of unauthorized medicinal products is generally prohibited, and authorization for early access programs must generally be obtained from national competent authorities, which might not grant such authorization. Obtaining authorization for an early access program in one country does not ensure that authorization will be obtained in another country. U.S. law permits “expanded access” (also known as compassionate use and treatment use) for certain patients with serious diseases who have no comparable alternative treatment options. To provide expanded access in the U.S., sponsors must submit detailed regulatory information to the FDA. FDA authorization depends on several different factors, including whether expanded access will interfere with related clinical trials or drug development. In addition, the Right to Try Act was signed into law in the U.S. on May 30, 2018 and allows certain eligible patients to have access to certain eligible investigational drugs outside of clinical trials if the patient’s licensed treating physician certifies that the patient has exhausted FDA-approved treatment options and cannot participate in a clinical trial of the investigational drug. Sponsors may not promote products as safe or effective for expanded access or right to try uses.

### **Pharmaceutical Pricing and Reimbursement**

The containment of healthcare costs has become a priority of federal, state and foreign governments, and the prices of pharmaceuticals have been a focus of this effort. The U.S. government, state legislatures and foreign governments have shown significant interest in implementing cost-containment programs to limit the growth of government-paid healthcare costs, including price controls, restrictions on reimbursement and requirements for substitution of generic products for branded prescription drugs. For example, the Medicare Prescription Drug, Improvement, and Modernization Act of 2003 expanded Medicare coverage for drug purchases by the elderly and changed the way Medicare covers and pays for pharmaceutical products. Cost reduction initiatives and other provisions of this law may decrease the coverage and reimbursement rate that we may receive for any products approved in the U.S. Likewise, healthcare reform measures under the Patient Protection and Affordable Care Act of 2010, as amended by the Health Care and Education Reconciliation Act of 2010, referred to together as the Affordable Care Act, contain provisions that may reduce the profitability of drug products by increasing the minimum level of Medicaid rebates payable by manufacturers of brand-name drugs from 15.1% to 23.1%, effective 2011, extending the Medicaid rebate to Medicaid managed care plans, changing the Medicaid rebate rates for line extensions or new formulations of oral solid dosage forms, mandating discounts for certain Medicare Part D beneficiaries, and imposing a non-deductible annual fee on pharmaceutical manufacturers or importers who sell “branded prescription drugs,” effective 2011, expanding the types of entities eligible for the “Section 340B discounts” for outpatient drugs, requiring manufacturers to participate in a coverage gap discount program, under which they must agree to offer point-of-sale discounts (75% effective as of 2019) off negotiated prices of applicable branded drugs to eligible beneficiaries during their coverage gap period, as a condition for the manufacturer’s outpatient drugs to be covered under Medicare Part D and creating a process for approval of biologic therapies that are similar or identical to approved biologics. There are numerous steps required to implement the Affordable Care Act, and implementation remains ongoing. Congress also has enacted, and may continue to seek, legislative changes that alter, delay, or eliminate some of its provisions. On February 1, 2016, the Centers for Medicare and Medicaid Services released a long-awaited new rule, the Medicaid Program Covered Outpatient Drug Final Rule, effective April 1, 2016, implementing various provisions of the Affordable Care Act related to “covered outpatient drugs,” including revising the calculation of “average manufacturer price” and addressing other issues relating to Medicaid price reporting and reimbursement. These and other changes contribute to the uncertainty of the ongoing implementation and impact of the Affordable Care Act; they also underscore the potential for additional reform going forward. Certain provisions of enacted or proposed legislative changes may negatively impact coverage and reimbursement of healthcare items and services.

Increasing pricing pressure continues from managed care organizations, government agencies and programs, particularly for new and innovative therapies, that could negatively affect the company’s sales and profit margins for

any product candidate for which we receive regulatory approval for commercial sale. In the U.S., these include practices of managed care groups, federal and state exchanges, and institutional and governmental purchasers. Changes to the health care system enacted as part of health care reform in the U.S., as well as increased purchasing power of entities that negotiate on behalf of Medicare, Medicaid, and private sector beneficiaries, could negatively impact the company's sales and profit margins. Such pressures may also increase the risk of litigation or investigations by the government regarding pricing calculations. There has also been recent negative publicity and Congressional scrutiny around pharmaceutical drug pricing in the U.S. These dynamics may give rise to negative reactions to pricing decisions for products for which we may receive regulatory approval in the future, possibly limiting our ability to generate revenue and attain profitability. Moreover, the pharmaceutical industry will likely face greater regulation and political and legal action in the future. In this healthcare regulatory climate, there may be significant delays in and impediments to obtaining coverage and reimbursement for newly approved drugs. Any regulatory approval of our products is limited to specific diseases and indications for which our products have been deemed safe and effective by the FDA. Coverage by federal healthcare programs may be more limited than the purposes for which the drug is approved by the FDA or comparable foreign regulatory authorities' coverage of the same products. In the U.S. and markets in other countries, sales of any products for which we receive regulatory approval for commercial sale will depend in part on the extent to which the costs of the products will be covered and reimbursed by third-party payors, including government healthcare programs such as Medicare and Medicaid, private health insurers and other organizations. Obtaining reimbursement for orphan drugs may be particularly difficult because of the higher prices typically associated with drugs directed at smaller populations of patients. In addition, third-party payors are likely to impose strict requirements for reimbursement in connection with the use of a higher priced drug. Net prices for products may be reduced by mandatory discounts or rebates required by government healthcare programs or private payors and by any future relaxation of laws that presently restrict imports of products from countries where they may be sold at lower prices than in the U.S.

The process for determining whether a payor will provide coverage for a product may be separate from the process for setting the price or reimbursement rate that the payor will pay for the product once coverage is approved. Third-party payors may limit coverage to specific products on an approved list, or formulary, which might not include all of the FDA-approved products for a particular indication. Third-party payors are increasingly challenging the price and examining the cost-effectiveness of medical products and services. We may need to conduct expensive pharmacoeconomic studies in order to demonstrate the cost-effectiveness of our products. Our product candidates may not be considered cost-effective. In the future, we may need to conduct direct head-to-head studies to demonstrate clinical superiority and cost-effectiveness. Our product candidates may not be considered clinically superior and cost-effective to competitor products.

The marketability of any products for which we receive regulatory approval for commercial sale may suffer if the government and other third-party payors fail to provide adequate coverage and reimbursement. In addition, there is an increasing emphasis on managed care in the U.S. that may negatively impact pharmaceutical pricing.

In some countries, particularly the countries of the EU, the pricing of prescription pharmaceuticals is subject to governmental control. In these countries, pricing and reimbursement negotiations with governmental authorities can take considerable time after the receipt of marketing approval for a product. In addition, there can be considerable pressure by governments and other stakeholders on prices and reimbursement levels, including as part of cost containment measures. In some countries, governments can set conditions that must be satisfied for prices to be set at a certain value. Political, economic and regulatory developments may further complicate pricing and reimbursement negotiations, and pricing negotiations may continue after reimbursement has been obtained. Reference pricing used by various EU member states, and parallel distribution (arbitrage between low-priced and high-priced member states), can further reduce prices. In some countries, we may be required to conduct a clinical trial or other studies that compare the cost-effectiveness of our product candidate to other available therapies in order to obtain reimbursement or pricing approval.

### ***Freedom of Information Requests***

We are also subject, in the U.S. and many other countries, to various regulatory schemes that require disclosure of clinical trial data or allow access to our data via freedom of information requests. We have been and may, from time to time, be notified by regulators, such as the EMA or the competent authorities of EU member states or the FDA that they have received a freedom of information request for documents that they hold relating to our company, including information related to our product candidates.

## **Fraud and Abuse Laws**

Any present or future arrangements with third-party payors, healthcare providers and professionals and customers may expose us to broadly applicable fraud and abuse and other healthcare laws and regulations that may restrict certain marketing and contracting practices. These laws include, and are not limited to, anti-kickback and false claims statutes.

Both the federal Foreign Corrupt Practices Act (“FCPA”), and the UK Bribery Act of 2010 (“Bribery Act”), are broad in scope and require companies to make and keep books and records that accurately and fairly reflect the transactions of the company and to devise and maintain an adequate system of internal accounting controls. The FCPA prohibits the offering, promising, giving, or authorizing others to give anything of value, either directly or indirectly, to a non-U.S. government official in order to improperly influence any act or decision, secure any other improper advantage, or obtain or retain business. Under the Bribery Act, companies which carry on a business or part of a business in the United Kingdom may be held liable for bribes given, offered or promised to any person, including non-UK government officials and private persons, by employees and persons associated with the company in order to obtain or retain business or a business advantage for the company.

The federal Anti-Kickback Statute in the U.S. prohibits, among other things, knowingly and willfully offering, paying, soliciting or receiving remuneration, directly or indirectly, in cash or kind, to induce or reward either the referral of an individual for, or the purchase, or order or recommendation of, any good or service, for which payment may be made under federal and state healthcare programs such as Medicare and Medicaid. This statute has been broadly interpreted to apply to manufacturer arrangements with prescribers, purchasers and formulary managers, among others. Although a number of statutory exemptions and regulatory safe harbors exist to protect certain common activities from prosecution, the exemptions and safe harbors for this statute are narrow, and practices that involve compensation intended to induce prescriptions, purchases, or recommendations may be subject to scrutiny if they do not qualify for an exemption or safe harbor. Our practices may not always meet all of the criteria for safe harbor protection. Further, the Affordable Care Act amended the intent requirement of the federal anti-kickback and criminal health care fraud statutes. This amendment provides that a person or entity no longer needs to have knowledge of these statutes or specific intent to violate them. In addition, 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 federal False Claims Act. Several other countries, including the United Kingdom, have enacted similar anti-kickback, fraud and abuse laws and regulations.

The federal False Claims Act imposes civil penalties, including through civil whistleblower or qui tam actions, against individuals or entities for knowingly presenting, or causing to be presented, to the federal government, claims for payment that are false or fraudulent or making a false statement to avoid, decrease or conceal an obligation to pay money to the federal government. Several pharmaceutical and health care companies have been prosecuted under these laws for allegedly providing free product to customers with the expectation that the customers would bill federal programs for the free product. Other companies have been prosecuted for causing false claims to be submitted because of these companies’ marketing of a product for unapproved, and thus non-reimbursable, uses. Potential liability under the federal False Claims Act includes mandatory treble damages and significant per claim penalties, currently set at \$5,500 to \$11,000 per false claim. The majority of states also have statutes or regulations similar to the federal anti-kickback statute and False Claims Act, which apply to items and services reimbursed under Medicaid and other state programs; furthermore, in several states, these statutes and regulations apply regardless of the payor. Sanctions under these federal and state laws may include civil monetary penalties, exclusion of a manufacturer’s product from reimbursement under government programs, debarment, criminal fines, and imprisonment.

The Affordable Care Act included a provision requiring certain providers and suppliers of items and services to Federal Health Care Programs to report and return overpayments within sixty days after they are “identified,” or the Overpayment Statute. In February 2016, the Centers for Medicare and Medicaid Services (“CMS”) released long-awaited regulatory guidance (in the form of a final rule) to Medicare Part A and Part B providers and suppliers regarding how to comply with the Overpayment Statute. CMS had previously released a final rule addressing overpayments involving Medicare Part C and Part D providers in May 2014. Although Medicare Part A/B/C/D providers and suppliers have faced federal False Claims Act liability since 2010 for failures to comply with the Overpayment Statute, these final rules interpreting the Overpayment Statute provide guidance to providers and suppliers regarding how to comply appropriately with applicable obligations, and guidance to government regulators and enforcement authorities regarding monitoring and prosecuting suspected violations. This final rule is not directly

applicable to manufacturers but may impact their customers and potential customers who are Medicare providers and suppliers.

The federal Physician Payments Sunshine Act, enacted as part of the Affordable Care Act, and its implementing regulations, require manufacturers of drugs, devices, biologics and medical supplies to report to the Department of Health and Human Services information related to payments and other transfers of value made to covered recipients, such as physicians and teaching hospitals, as well as physician ownership and investment interests. Payments made to physicians and certain research institutions for clinical trials are included within the ambit of this law. Pharmaceutical manufacturers are required to report and disclose payments and ownership and investment interests held by physicians and their immediate family members during the preceding calendar year. Manufacturers were required to make these first reports for information collected in 2013 by March 31, 2014. Such information is publicly available from the Secretary of Health and Human Services in a searchable format, with data collected in each calendar year published the following June. Failure to submit required information may result in civil monetary penalties of up to \$150,000 per year (and up to \$1.0 million per year for “knowing failures”) for all payments, transfers of value or ownership or investment interests not reported in an annual submission. If not preempted by this federal law, several states currently require pharmaceutical companies to report expenses relating to the marketing and promotion of pharmaceutical products and to report gifts and payments to individual physicians in those states. Depending on the state, legislation may prohibit various other marketing related activities, or require the posting of information relating to clinical studies and their outcomes. In addition, certain states, such as California, Nevada, Connecticut and Massachusetts, require pharmaceutical companies to implement compliance programs or marketing codes and several other states are considering similar proposals. Manufacturers that fail to comply with these state laws can face civil penalties.

Statutory requirements to disclose publicly payments made to healthcare professionals and healthcare organizations have also been enacted in certain EU member states. In addition, self-regulatory bodies of the pharmaceuticals industry, such as the European Federation of Pharmaceutical Industries and Associations (“EFPIA”) have published codes of conduct to which its members have agreed to abide by, that require the public disclosure of payments made to healthcare professionals and healthcare organizations.

The Health Insurance Portability and Accountability Act of 1996 (“HIPAA”), as amended by the Health Information Technology for Economic and Clinical Health Act, imposes criminal liability for executing a scheme to defraud any healthcare benefit program and for knowingly and willfully falsifying, concealing or covering up a material fact or making any materially false statements in connection with the delivery of or payment for healthcare benefits, items or services. HIPAA also imposes obligations, including mandatory contractual terms, with respect to safeguarding the privacy, security and transmission of individually identifiable health information, and imposes criminal and civil liability for violations of these obligations. Recently, the U.S. federal government criminally prosecuted an employee of a pharmaceutical company for an alleged violation of the privacy requirements under HIPAA. Furthermore, certain privacy laws and genetic testing laws may apply directly to our operations and/or those of our collaborators and may impose restrictions on our use and dissemination of individuals’ health information.

The foregoing discussion should be read in conjunction with the information appearing under “Risk Factors” and the subheading which begins “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.” This risk factor contains important information regarding some of the risks to our business arising as a result of fraud and abuse laws.

## **Competition**

Our industry is highly competitive and subject to rapid and significant technological change. New therapies and treatments based on innovative discoveries emerge frequently.

Our potential competitors are public and private companies, pharmaceutical companies and biotechnology companies who may be engaged in targeting the same biological processes that our compounds are designed to impact and who may be developing products for the same indications as our investigational drug candidates. Potential competitors could also include academic institutions, government agencies, other public and private research organizations and charitable venture philanthropic organizations that conduct research, seek patent protection and/or establish collaborative arrangements for research, development, manufacturing and commercialization.

Many of our competitors have substantially greater financial resources, technical resources, expertise in research and development, manufacturing, preclinical testing, conducting clinical trials, obtaining regulatory approvals and marketing approved products than we do. These competitors also compete with us in recruiting and retaining qualified scientific and management personnel and establishing clinical trial sites and patient registration for clinical trials. As a result, our competitors may commercialize products more rapidly or effectively than we do, which would affect our competitive position, the likelihood that our drug candidates, if approved, would achieve and maintain market acceptance and our ability to generate meaningful revenues from our products.

Our commercial opportunities could be reduced or eliminated if our competitors develop and commercialize products that are safer, more effective, have fewer side effects, are more convenient or are more affordable than any products that we develop. The key competitive factors affecting the success of ELX-02 and our other product candidates are their impact on the targeted diseases, superiority over competing products, long-term safety, convenience, price and the availability of coverage and reimbursement from government and other third-party payors.

Several companies are involved in researching and developing molecules targeting suppression of nonsense mutations and enhancement of translational read-through. However, we believe that ELX-02 is the only drug candidate in clinical development designed to treat nonsense mutations in CFTR, the underlying cause of cystic fibrosis and cystinosis, our lead indications. Additional competition to ELX-02 may arise from other programs that do not target a specific *CFTR* mutation class but work via other mechanisms.

## **Employees**

As of February 29, 2020, we had 29 full-time employees. Of these employees, six were located at our Rehovot, Israel research and development facility and 23 were located in the U.S. We also use consultants as necessary to support key functions.

Our success is highly dependent on our ability to attract and retain qualified employees. Competition for employees is intense in the pharmaceutical industry. None of our employees are covered by a labor union or are subject to collective bargaining agreement and we have never experienced any work stoppage. We consider our relations with our employees to be good.

## **Additional Information**

On December 19, 2017, Sevion Therapeutics, Inc. (“Sevion”), a Delaware corporation, acquired Eloxx Pharmaceuticals, Ltd. (“Eloxx Limited”), an Israeli company, pursuant to a merger between the companies (the “Transaction”). Upon consummation of the Transaction, Sevion adopted the business plan of Eloxx Limited and discontinued the pursuit of Sevion’s business plan. In connection with the Transaction, Sevion acquired all of the outstanding capital stock of Eloxx Limited in exchange for the issuance of an aggregate 20,316,656 shares of the Sevion’s common stock, par value \$0.01 per share (the “Common Stock”), after giving effect to a 1-for-20 reverse split effected immediately prior to the Transaction. As a result of the Transaction, Eloxx Limited became a wholly-owned subsidiary of Sevion. While Sevion was the legal acquirer in the Transaction, Eloxx Limited was deemed the accounting acquirer. Immediately after giving effect to the Transaction, on December 19, 2017, Sevion changed its name to Eloxx Pharmaceuticals, Inc.

Our principal executive offices are located at 950 Winter Street, Waltham, Massachusetts 02451, and our phone number is (781) 577-5300. In December 2017, we changed our name from Sevion Therapeutics Inc. to Eloxx Pharmaceuticals, Inc. and also changed our ticker symbol from SVON to ELOX. Our wholly-owned subsidiary is Eloxx Pharmaceuticals, Ltd.

Our internet address is <http://www.eloxxpharma.com>. The public can access, through a link on the “Investors” section of our website, free of charge, all reports and other information on file with the Securities and Exchange Commission (“SEC”), immediately after we electronically submit such material to the SEC. In addition, we will provide electronic or paper copies of our filings free of charge upon request. Information contained on our corporate website or any other website is not incorporated into this Report and does not constitute a part of this Report.

In addition, the public may read and copy any materials filed by the Company with the SEC at the SEC’s Public Reference Room located 100 F Street, NE, Washington, DC 20549. Interested parties may call 1-800-SEC-

0330 for further information. The SEC also maintains a website containing publicly available information, at <https://www.sec.gov>.

We post our code of business conduct and ethics, which applies to all employees, including all executive officers, senior financial officers and directors, in the “Corporate Governance” sub-section of the “Investors” section of our corporate website at [www.eloxxpharma.com](http://www.eloxxpharma.com). Our code of business conduct and ethics complies with Item 406 of SEC Regulations S-K and the Rules of the NASDAQ Stock Market. We intend to disclose any changes to the code that affect the provisions required by Item 406 of Regulation S-K and any waivers of the code of ethics for our executive officers, senior financial officers or directors, on our corporate website.

## **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 Annual Report on Form 10-K, 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 pre-clinical 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; 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.

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, or 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 IND (investigational new drug application) administered by the individual's treating physician) may result in a partial or full clinical hold on our on-going clinical trials. A clinical hold may result in the inability to enroll new patients in our studies until the hold is removed and 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 of 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 and 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; 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 available 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.

***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 investigational new drug applications (“INDs”) and pre-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.

***We are subject to extensive governmental regulation including the requirements of 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 FDA's, the EMA's and other foreign regulatory authorities' regulatory requirements 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 cystinosis, 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 cystinosis as well as for the treatment of MPS I and the treatment of 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, acquisitions and joint ventures 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 December 31, 2019, had an accumulated deficit of \$(137.0) million. Historically, we have financed our operations primarily through equity capital investments, and to a lesser extent from loans and grants from the Israeli Innovation Authority of the Ministry of Economy and Industry, or the IIA. 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 further into clinical trials;
- continue the preclinical development of our research programs and advance candidates into clinical trials;
- identify additional product candidates and advance them into preclinical development;
- pursue regulatory authorization to conduct clinical trials of additional product candidates;
- seek marketing approvals for our product candidates that successfully complete clinical trials;
- 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, including personnel to support product development;

- 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 our Company could also cause you to lose all or part of your 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 product 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 many years, if at all.

Accordingly, despite the public offerings of common stock we completed in April 2018 and June 2019, 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, your ownership interest will be diluted, and the terms of these securities may include liquidation or other preferences that adversely affect your 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.

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”), which includes significant changes to the taxation of business entities. The Tax Cuts and Jobs Act, 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. Notwithstanding the reduction in the corporate income tax rate, the Tax Cuts and Jobs Act 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 Tax Cuts and Jobs Act. The impact of the Tax Cuts

and Jobs Act 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 Tax Cuts and Jobs 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 Tax Cuts and Jobs Act which are not subject to expiration. Under the Tax Cuts and Jobs Act, 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 Tax Cuts and Jobs Act.

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 changes 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 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.

***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, or are developing, laws governing the collection, use and transmission of personal information as well. 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 implemented information security measures to protect patients’ personal information against the risk of inappropriate and unauthorized external use and disclosure. 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.

***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 which are being conducted in Europe, Israel and the U.S., 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.

***We expect to 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.

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 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.

## 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 December 31, 2019, we owned or licensed 19 issued patents and 34 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 life of our patents is 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 patent 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 locations. 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 income, 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 the 2018 Plan, our management is authorized to grant stock options and other equity-based awards to our employees, directors and consultants. The 2018 Plan became effective on April 20, 2018. As of December 31, 2019, individuals held share awards to purchase or receive an aggregate of 5,201,615 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. 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 1B. UNRESOLVED STAFF COMMENTS**

None.

#### **ITEM 2. PROPERTIES**

Our principal executive offices are currently located at 950 Winter Street, Waltham, Massachusetts, and consist of 10,674 square feet of office space under lease until August 2021, with an option to extend the lease period for additional three years. We also lease additional office space in Morristown, New Jersey and Rehovot, Israel.

#### **ITEM 3. 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, 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 4. MINE SAFETY DISCLOSURES**

None.

## PART II

### ITEM 5. MARKET FOR REGISTRANT'S COMMON EQUITY, RELATED STOCKHOLDER MATTERS AND ISSUER PURCHASES OF EQUITY SECURITIES

#### Market Information for Common Stock

Since April 26, 2018, our common stock has traded on The NASDAQ Capital Market under the symbol “ELOX.” Prior to that date, our common stock traded on the OTCQB market under the symbol “ELOX”. Following the Reverse Merger and reverse stock split, and commencing December 20, 2017, the Company’s common stock symbol on the OTCQB market changed to “SVOND”, and subsequently changed to “ELOX” on January 19, 2018. Prior to the completion of the Transaction on December 19, 2017, our common stock was traded on the OTCQB market under the symbol “SVON.”

#### Holders

As of March 1, 2020, there were approximately 70 holders of record of our common stock. The actual number of holders of our common stock is greater than this number of record holders, and includes stockholders who are beneficial owners, but whose shares are held in street name by brokers or held by other nominees. This number of holders of record also does not include stockholders whose shares may be held in trust by other entities.

#### Recent Sales of Unregistered Securities

None, except as previously disclosed on our Quarterly Reports on Forms 10-Q and Current Reports on Forms 8-K.

#### Purchases of Equity Securities by the Issuer or Affiliated Purchaser

There were no repurchases of shares of our common stock during the fourth quarter ended December 31, 2019.

#### Dividend Policy

We have not paid dividends on our common stock since inception and we do not intend to pay any dividends in the foreseeable future. We expect that any earnings which we may realize will be retained to finance the growth of our Company. The declaration of dividends in the future will be at the election of our board of directors and will depend upon our earnings, capital requirements, financial position, general economic conditions, and other factors the board of directors deems relevant.

### ITEM 6. SELECTED FINANCIAL DATA

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

## **ITEM 7. MANAGEMENT'S DISCUSSION AND ANALYSIS OF FINANCIAL CONDITION AND RESULTS OF OPERATION**

*The following information should be read in conjunction with the consolidated financial statements and related notes thereto included in this Annual Report on Form 10-K (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 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 disorders. 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 disorders 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 mRNA stability and enabling functional protein synthesis. 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 and nephropathic cystinosis. ELX-02 is an investigational drug that has not been approved by any global regulatory body. In addition, during 2019 we announced a new program studying 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. 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"). Professor Timor Baasov, the inventor of our compounds, has served as our senior consultant since our incorporation.

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 provide support for both continuing our clinical development programs and evaluating the suitability of our ERSG library for development in additional renal disorders, including ADPKD and cystinuria. Our preclinical candidate pool consists of a library of novel ERSG drug candidates identified based on read-through potential and cytoplasmic ribosomal selectivity.

Our research and development strategy targets rare or ultra-rare diseases where a high unmet medical need exists, an identified 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 identifiable. 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 studies in both cystic fibrosis and nephropathic cystinosis.

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 of 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 of 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 disorders: cystic fibrosis, nephropathic cystinosis, ADPKD, a variety of IRDs (including Usher Syndrome), primary ciliary dyskinesia, cystinuria, 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, 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.

Currently, the clinical programs for our lead investigational drug candidate, ELX-02, are focused on development for cystic fibrosis and nephropathic cystinosis patients with diagnosed nonsense mutations. With ELX-02, 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 U.S. 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 trial were presented in 2019 at both the European Cystic Fibrosis 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.

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 actively dosing patients in the U.S. and Israel and continuing to enroll patients at leading global investigator sites. We expect to achieve full enrollment and to report topline results during the first half of 2020. In the U.S., the Cystic Fibrosis 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 (ClinicalTrials.gov Identifiers: NCT04126473 and NCT04135495), which is being conducted at TDN member sites.

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. In Europe and Israel, Professor Eitan Kerem, M.D., Head of the Division of Pediatrics, Children’s Hospital, Hadassah Medical Center in Israel, is the global lead 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) and we are currently enrolling and dosing patients in both trials. 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.

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 plan to present these data at the Association for Research in Vision and Ophthalmology (ARVO) Annual Meeting May 3-7, 2020 in Baltimore, Maryland.

We are also evaluating the suitability of our ERSG library for development in rare renal disorders associated with nonsense mutations, such as ADPKD and cystinuria. ADPKD is a relatively common inherited genetic kidney disease occurring in between 1:400 and 1:1,000 patients and 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 IND.

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. There are no currently approved therapeutics that target the nonsense mutation mediated impairment of cystinosin, the cystine-selective transport channel in the lysosomal membrane that is attributed as the cause for the accumulation of cystine in this disease state. Given the high proportion of pediatric patients in each of these 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), and the FDA has granted orphan drug designation to ELX-02 for the treatment of nephropathic cystinosis, MPS I, and Rett syndrome.

We believe that our cash, cash equivalents, and marketable securities of \$56.3 million at December 31, 2019, will enable us to meet the anticipated cash needs required to reach topline 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 Annual Report on Form 10-K. Since our inception, we have incurred significant operating losses. Our net losses were \$50.9 million, \$47.2 million and \$21.2 million for the years ended December 31, 2019, 2018 and 2017, respectively. As of December 31, 2019, we had an accumulated deficit of \$(137.0) million. To date, we have financed our operations primarily through equity capital investments, and to a lesser extent, from loans and grants from the Israeli Innovation Authority of the Ministry of Economy and Industry, or the IIA. 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 may increase if, and as, we:

- advance ELX-02 further into clinical trials;
- continue the preclinical development of our research programs and advance candidates into clinical trials;
- identify additional product candidates and advance them into preclinical development;
- pursue regulatory authorization to conduct clinical trials of additional product candidates;
- seek marketing approvals for our product candidates that successfully complete clinical trials;
- 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, including personnel to support product development;
- acquire or in-license other product candidates and technologies; and
- operate as a public company.

#### ***Critical Accounting Policies and Significant Judgments and Estimates***

Our discussion and analysis of our financial condition and results of operations is based on our consolidated financial statements and the notes thereto included elsewhere in this Report, which have been prepared in accordance with accounting principles generally accepted in the United States (“U.S. GAAP”). The preparation of these annual consolidated financial statements requires us to make estimates and judgments that affect the reported amounts of assets and liabilities and the disclosure of contingent assets and liabilities at the date of the consolidated financial statements, as well as the expenses during the reporting period. We evaluate our estimates and judgments on an ongoing basis. These items are monitored and analyzed by us 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 values of assets and liabilities that are not readily apparent from other sources. Actual results may differ from these estimates under different assumptions or conditions.

While our significant accounting policies are more fully described in Note 2 of the Notes to Consolidated Financial Statements appearing elsewhere in this Report, we believe that the following accounting policies related to accrued expenses and accrued clinical trial costs and contract research liabilities are the most critical accounting policies for fully understanding and evaluating our financial condition and results of operations.

#### ***Accrued Expenses and Accrued Clinical Trial Costs and Contract Research Liabilities***

As part of the process of preparing our financial statements, we are required to estimate accrued expenses. This process involves identifying services which have been performed on our behalf and estimating the level of service performed and the associated cost incurred for such service as of each balance sheet date in our financial statements. Given our current business, the primary area of uncertainty concerning accruals which could have a material effect on our operating results is with respect to service fees paid to contract manufacturers in conjunction with the production of clinical drug supplies, and to contract research organizations in connection with our pre-clinical research and clinical trials. In connection with all of the foregoing service fees, our estimates are most affected by our understanding of the status and timing of services provided. The majority of our service providers, including contract research organizations, invoice us in arrears for services performed. In the event that we do not identify some costs which have begun to be incurred, or we underestimate or overestimate the level of services performed or the costs of such services in a given period, our reported expenses for such period would be understated or overstated. We currently reflect the effects of any changes in estimates based on changes in facts and circumstances directly in our statement of operations in the period such change becomes known.

Our arrangements with contract research organizations in connection with clinical trials often provide for payment prior to commencing the project or based upon predetermined milestones throughout the period during which services are expected to be performed. We recognize expense relating to these arrangements based on the various services provided over the estimated time to completion. The date on which services commence, the level of services performed on or before a given date, and the cost of such services are often determined based on subjective judgments. We make these judgments based upon the facts and circumstances known to us based on the terms of the contract and our ongoing monitoring of service performance. During the years ended December 31, 2019 and 2018, we had arrangements with multiple contract research organizations whereby these organizations commit to performing services for us over multiple reporting periods. We recognize the expenses associated with these arrangements based on our expectation of the timing of the performance of components under these arrangements by these organizations. Generally, these components consist of the costs of setting up the trial, monitoring the trial, closing the trial and preparing the resulting data. Costs related to patient enrollment in clinical trials are accrued as patients are enrolled in the trial.

With respect to financial reporting periods presented in this Report, the timing of our actual costs incurred have not differed materially from our estimated timing of such costs. In light of the foregoing, we do not believe our practices for estimating future expenses and making judgments concerning the accrual of expenses are reasonably likely to change in the future.

## Results of Operations

### **Comparison of the Years Ended December 31, 2019, 2018 and 2017**

Dollar amounts in the following table are in thousands:

	Year Ended December 31,			2019 / 2018		2018 / 2017	
	2019	2018	2017				
<b>Operating expenses:</b>							
Research and development, net	\$ 25,842	\$ 20,489	\$ 16,398	\$ 5,353	26 %	\$ 4,091	25 %
General and administrative	24,713	26,482	2,702	(1,769)	(7) %	23,780	880 %
Reverse merger related expenses	—	594	1,290	(594)	(100) %	(696)	(54) %
Total operating costs	50,555	47,565	20,390	2,990	6 %	27,175	133 %
Loss from operations	(50,555)	(47,565)	(20,390)	(2,990)	6 %	(27,175)	133 %
Other (income) expense, net	319	(502)	824	821	(164) %	(1,326)	(161) %
Loss before income taxes	(50,874)	(47,063)	(21,214)	(3,811)	8 %	(25,849)	122 %
Provision for income taxes	—	122	—	(122)	(100) %	122	— %
Net loss	<u>\$ (50,874)</u>	<u>\$ (47,185)</u>	<u>\$ (21,214)</u>	<u>\$ (3,689)</u>	8 %	<u>\$ (25,971)</u>	122 %

#### *Research and development expenses, net*

Research and development expenses were \$25.8 million for the year ended December 31, 2019 compared to \$20.5 million for the year ended December 31, 2018, an increase of \$5.4 million. The increase was primarily related to an increase in salaries and other personnel related costs of \$2.3 million, an increase in stock-based compensation of \$0.7 million, and an increase in expenses related to subcontractors, consultants and advisors in connection with continued development of ELX-02 of \$2.3 million.

Research and development expenses were \$20.5 million for the year ended December 31, 2018 compared to \$16.4 million for the year ended December 31, 2017, an increase of \$4.1 million. The increase was primarily related to an increase in salaries and other personnel related costs of \$3.4 million and an increase in expenses related to subcontractors, consultants and advisors in connection with continued clinical phase development of ELX-02 of \$0.7 million.

#### *General and administrative expenses*

General and administrative expenses were \$24.7 million for the year ended December 31, 2019 compared to \$26.5 million for the year ended December 31, 2018, a decrease of \$1.8 million. The decrease was primarily related to a decrease in stock-based compensation of \$2.7 million and a decrease in expenses attributable principally to

infrastructure related costs including legal, accounting and other professional fees of \$1.1 million, offset by increases in salaries and other personnel related costs of \$2.0 million.

General and administrative expenses were \$26.5 million for the year ended December 31, 2018 compared to \$2.7 million for the year ended December 31, 2017, an increase of \$23.8 million. The increase was primarily related to an increase in stock-based compensation of \$11.6 million, an increase in salaries and other personnel related costs of \$4.4 million, and fees attributable principally to infrastructure related costs including legal, accounting and other professional fees of \$7.8 million.

#### *Reverse merger related expenses*

During the years ended December 31, 2018 and 2017, we recorded professional service fees of \$0.6 million and \$1.3 million, respectively, related to the reverse merger we completed on December 19, 2017. There were no reverse merger related expenses recorded during the year ended December 31, 2019.

#### *Other (income) expense, net*

We recorded \$0.3 million in other expense, net for the year ended December 31, 2019 compared to \$0.5 million in other income for the year ended December 31, 2018, an increase of \$0.8 million. The increase in other expense, net was primarily due to debt issuance costs and interest expense of \$1.6 million associated with our bank debt issued in January 2019, offset by an increase in interest income of \$0.2 million, investment income of \$0.3 million, and foreign currency exchange gains and other income of \$0.1 million. Interest and investment income increased primarily due to higher average cash balances as a result of \$32.2 million received from our public offering of common stock in June 2019, and \$15.0 million received from our bank debt in January 2019.

We recorded \$0.5 million in other income, net for the year ended December 31, 2018 compared to \$0.8 million in other expense, net for the year ended December 31, 2017, an increase of \$1.3 million. The change was primarily due to an increase in interest income of \$0.6 million due to higher average cash balances resulting from net proceeds received of \$53.6 million from our public offering of common stock in April 2018, a decrease in interest expense of \$0.6 million on debt issuance costs resulting from the conversion of the convertible loan into Series C preferred stock, and a decrease in foreign currency exchange losses of \$0.1 million.

#### *Provision for income taxes*

There was no provision for income taxes for the year ended December 31, 2019. We recorded a provision for income taxes of \$0.1 million for the year ended December 31, 2018, primarily as a result of current income tax expense for subsidiary operations and a correction of a prior year tax return partially offset by a prior year provision-to-return adjustment.

#### *Net operating loss carryforwards*

As of December 31, 2019, we had U.S. federal and state net operating loss (“NOL”) carryforwards of \$99.4 million and \$31.9 million, respectively, and federal research tax credit carryforwards of \$3.0 million. Certain U.S. net operating loss 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. These NOL carryforwards could expire unused and be unavailable to offset future income tax liabilities. Included in these U.S. federal NOL carryforwards are \$23.5 million of NOLs generated after the effective date of the Tax Cuts and Jobs Act of 2017 (the “Tax Act”), which are not subject to expiration, 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. Under the Tax Act, federal net operating losses incurred beginning in 2018 and in future years may be carried forward indefinitely, but the deductibility of such federal net operating losses is limited. It is uncertain if and to what extent various U.S. states will conform to the newly enacted federal tax law. Also, as of December 31, 2019, we had Israeli NOL carryforwards of \$73.7 million, which carry forward indefinitely.

## **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. Since our inception and through December 31, 2019, we have funded our operations primarily through equity capital investments, and to a lesser extent, from loans and grants.

We have a history of net losses and negative cash flows from operating activities since inception, and as of December 31, 2019, we had an accumulated deficit of \$(137.0) million. We expect to continue to incur net losses and use cash in our operations for the foreseeable future. To date, we have not generated revenue from the sale of any product or service, and we do not expect to generate significant revenue unless and until we obtain marketing approval for and commercialize one or more of our product candidates currently in development. A successful transition to profitable operations is dependent upon achieving a level of revenue adequate to support our cost structure.

We have financed our operations primarily from the sale of our equity securities, and to a lesser extent, from loans and grants. We may never achieve profitability, and unless and until we do, we will continue to need to raise additional cash to fund our operations. As discussed in Note 17 of the consolidated financial statements, on February 24, 2020, the Board of Directors of the Company approved a leadership and organizational re-alignment, which is expected to achieve annual cost savings of approximately \$4.9 million, with anticipated fiscal year 2020 savings of approximately \$2.4 million, net of severance costs. We believe that our cash, cash equivalents, and marketable securities of \$56.3 million at December 31, 2019 will enable us to meet the anticipated cash needs required to maintain our current and planned operations through at least the next 12 months from the issuance of this Annual Report on Form 10-K. 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 financing, we will evaluate options which may include reducing or deferring operating expenses, which may have a material adverse effect on our operations and future prospects.

### ***Principal Financing Activities***

In February 2016, Eloxx Limited issued shares of preferred stock and warrants to purchase shares of preferred stock for an aggregate gross amount of \$6.0 million.

In August 2016, Technion Investment Opportunities Fund L.P (the “TIOF”) and TRDF exercised 124,786 and 311,964 warrants, respectively, to purchase shares of preferred stock for total consideration of \$0.4 million.

In September 2016, Eloxx Limited achieved a milestone in connection with a prior issuance of securities, pursuant to which Eloxx Limited paid a \$0.1 million milestone payment and issued to investors additional shares of preferred stock and warrants to purchase preferred stock for an aggregate amount of \$3.7 million.

In May 2017, Eloxx Limited entered into a Share Purchase Agreement (the “2017 SPA”) with certain existing and new investors, whereby an aggregate gross amount of \$21.5 million, which included the conversion of the loan, was received by Eloxx Limited in exchange for the issuance of shares of preferred stock including shares of preferred stock that were issued as a result of the anti-dilution effect of the Reverse Merger. The related issuance costs were \$0.6 million.

Upon the closing of the Reverse Merger in December 2017, we issued 6,333,333 shares of common stock related to the 2017 SPA for an aggregate gross amount of \$17.5 million. Additionally, Sevion raised \$1.5 million prior to the Reverse Merger. The related issuance costs for these transactions were \$0.5 million.

On April 30, 2018, we completed an underwritten public offering of 5,899,500 shares of our common stock, including the exercise in full by the underwriter of its overallotment option to purchase an additional 769,500 shares,

at the public offering price of \$9.75 per share for gross proceeds of approximately \$57.5 million, before deducting the underwriting discounts and commissions and offering expenses of approximately \$3.9 million.

In November 2018, we entered into an Equity Distribution Agreement with Citigroup Global Markets Inc. and Cantor Fitzgerald & Co., pursuant to which we sold 236,462 shares of common stock and received proceeds of \$2.9 million, net of issuance costs of \$0.1 million through December 31, 2019, of which 35,362 shares of common stock, with received net proceeds of \$0.7 million were sold in January of 2019. The shares sold were pursuant to an effective registration statement as described below.

On January 30, 2019, we entered into a Loan and Security Agreement (the “Loan Agreement”) with Silicon Valley Bank (“SVB”), in its capacity as administrative agent, collateral agent and lender, and WestRiver Innovation Lending Fund VIII, L.P. (“WestRiver”). Pursuant to the terms and conditions of the Loan Agreement, the Lenders agreed to extend term loans to us in an aggregate principal amount of up to \$25 million, comprised of (i) an initial loan advance of \$15 million and (ii) a subsequent loan advance of \$10 million, subject to first achieving certain conditions (collectively, the “Term Loan Advances”). The initial term loan was funded on January 30, 2019. The subsequent loan advance was available at our election after the occurrence of certain milestone events relating to data from our clinical trials and receipt by us of certain minimum cash proceeds of at least \$75 million from an additional equity offering through a private placement or a public offering by December 31, 2019. These events did not occur, and thus we did not draw the second advance, and expired unused.

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 estimated offering expenses of \$0.2 million.

### **Cash Flows**

The following table presents the major components of net cash flows provided by (used in) operating, investing and financing activities for the periods presented (in thousands):

	<b>Year Ended December 31,</b>		
	<b>2019</b>	<b>2018</b>	<b>2017</b>
Net cash used in operating activities	\$ (39,391)	\$ (31,367)	\$ (15,935)
Net cash used in investing activities	\$ (33,524)	\$ (317)	\$ (114)
Net cash provided by financing activities	\$ 46,800	\$ 56,184	\$ 37,950

Our operating activities used cash of \$39.4 million, \$31.4 million and \$15.9 million for the years ended December 31, 2019, 2018 and 2017, respectively. Cash used in operations resulted primarily from our net losses adjusted for non-cash items and changes in working capital. During the year ended December 31, 2019, our net loss was \$50.9 million, partially offset by non-cash charges of \$11.4 million related to stock-based compensation expense and \$0.1 million of depreciation expense. Changes in working capital for the period were \$0.6 million, related primarily to accounts payable and accrued expenses. During the year ended December 31, 2018, our net loss was \$47.2 million partially offset by non-cash charges of \$13.4 million related to stock-based compensation expense and \$0.2 million of depreciation expense. Changes in working capital for the period were \$2.2 million related primarily to prepaid expenses and other current assets and accrued expenses. During the year ended December 31, 2017, our net loss was \$21.2 million, partially offset by non-cash charges of \$3.4 million for the provision related to the Technion exit fee, \$0.6 million related to the amortization and revaluation of the discount on our convertible loan, and \$0.1 million related to stock-based compensation expense. Changes in working capital for the period were \$1.0 million related to a decrease in prepaid expenses and other current assets and an increase in accrued expenses, offset by a decrease in accounts payable.

Our investing activities used cash of \$33.5 million, \$0.3 million and \$0.1 million for the years ended December 31, 2019, 2018 and 2017, respectively. Cash used in investing activities during the year ended December 31, 2019 consisted primarily of purchases of marketable securities of \$67.2 million, offset by proceeds of \$33.8 million received upon maturities of marketable securities. During the years ended December 31, 2018 and 2017, cash used in investing activities was primarily related to purchases of property and equipment.

Our financing activities provided cash of \$46.8 million, \$56.2 million and \$38.0 million for the years ended December 31, 2019, 2018 and 2017, respectively. During the year ended December 31, 2019, net cash provided by

financing activities resulted primarily from net proceeds of \$32.2 million from our public offering of common stock in June 2019, the issuance of debt of \$14.7 million (net of issuance costs) in January 2019, and proceeds of \$0.7 million from the sale of common stock, offset by \$1.4 million of taxes paid upon the vesting of restricted stock units. During the year ended December 31, 2018, net proceeds from the 2018 offering were \$53.6 million, proceeds from the equity sales agreement were \$2.2 million, and proceeds from share-based compensation arrangements were \$0.4 million. During the year ended December 31, 2017, net proceeds from the sale of preferred stock were \$18.4 million, net proceeds from the conversion of convertible loans into preferred stock were \$2.5 million, and net proceeds from the sale of common stock were \$17.0 million.

### **Form S-3 and Equity Sales**

On April 10, 2018, we filed a shelf registration statement (the “April 2018 Shelf”) on Form S-3 with the U.S. Securities and Exchange Commission (the “SEC”). The April 2018 Shelf (File No. 333-224207) was declared effective on April 20, 2018 and covers the offering, issuance and sale of up to \$125 million of our common stock, preferred stock, debt securities or warrants and other securities, either individually or in combination.

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 the April 2018 Shelf. We agreed to pay the Sales Agents a commission of up to 3% of the gross proceeds of any sales of common stock pursuant to the Agreement. We incurred approximately \$0.3 million related to legal, accounting and other fees in connection with the Agreement. 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.

On November 16, 2018, we filed a shelf registration statement (the “November 2018 Shelf”) on Form S-3 with the SEC. The November 2018 Shelf (File No. 333-228430) was declared effective on November 26, 2018 and covers the offering, issuance and sale of up to \$200 million of our common stock, preferred stock, debt securities or warrants and other securities, either individually or in combination, and is available for future issuances.

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 net proceeds of approximately \$34.5 million, before deducting underwriting discounts and commissions of \$2.1 million and estimated offering expenses of \$0.2 million.

### **Government Grants from the Israeli Innovation Authority (“IIA”)**

To date, we have received research and development grants from the IIA totaling \$2.6 million. We received research and development grants from the IIA of \$0.9 million for the year ended December 31, 2017. No grants were received for the years ended December 31, 2019 and 2018.

Under the research and development agreements with the IIA and pursuant to applicable law, we are required to pay royalties at the rate of 3% on sales to end customers of product candidates developed with funds provided by the IIA, up to an amount equal to 100% of the IIA research and development grants received, plus interest based on the 12-month LIBOR rate. If we do not generate sales of product candidates developed with funds provided by the IIA, we are not obligated to pay royalties or repay the grants.

As of December 31, 2019, we have not commenced the payment obligation of the royalties and have a contingent obligation with respect to royalty-bearing participation received or accrued, amounting to \$2.7 million, including accrued LIBOR interest.

### **Technion Research and Development Foundation Limited Agreement**

On August 29, 2013, we entered into an agreement (the “Technion Agreement”) with the Technion Research and Development Foundation Limited (“TRDF”), with respect to certain technology relating to aminoglycosides and the redesign of aminoglycosides for the treatment of human genetic diseases caused by premature stop mutations and further results of the research of the technology, in order to develop and commercialize products based on such technology. Under the Technion Agreement, TRDF is obligated to provide us with research services for an estimated annual payment of \$0.1 million, the precise amount to be agreed by the parties prior to the beginning of

each year of the research period. During the years ended December 31, 2019, 2018 and 2017, we recorded general and administrative expenses amounting to \$0, \$0 and \$7 thousand, respectively, in relation to the Technion Agreement. In addition, during the years ended December 31, 2019, 2018 and 2017, we recorded research and development expenses amounting to \$0.2 million, \$0.1 million and \$3.5 million, respectively, in relation to the Technion Agreement for reimbursement of costs incurred during the preparation, filing, prosecution and maintenance of the TRDF patent rights related to Eloxx Limited. As of December 31, 2019 and 2018, amounts recorded in accrued expenses were \$0.1 million and \$25 thousand, respectively.

In addition, TRDF granted us a license to use, market, sell or sub-license the rights of the product developed under the TRDF research results (the “Licensed Product”), as defined in the Technion Agreement, for the following considerations: (a) milestone payments up to total consideration of \$6.1 million, to be transferred upon meeting certain milestones as defined in the Technion Agreement; (b) certain royalties in the low- to mid-single-digit percentage of net sales (subject to change in the case of (x) sublicensing to a big pharmaceutical or biotechnology company, or (y) payment of royalties to third parties, or (z) commercialization by a third party of an authorized generic to a licensed product), for a period until the later of (i) the expiration of a valid claim on the Licensed Product in each country the Licensed Product is sold to, or (ii) a certain amount of years from the date of the first commercial sale of the Licensed Product in such country, and (c) a low- to mid-double-digit percentage of any non-royalty sub-license income received by us from a sub-licensed entity. In addition, we agreed to pay a fee to TRDF upon certain exit events as described in the Technion Agreement.

On August 9, 2017, we received a letter from TRDF regarding TRDF’s alleged entitlement to an exit fee in accordance with the Technion Agreement. We recorded a \$3.4 million research and development expense with an offsetting adjustment to additional paid-in capital for the year ended December 31, 2017, related to the planned issuance of shares to TRDF at fair market value on the purported date of the exit event. On June 30, 2018, we issued 569,395 shares to TRDF in satisfaction of this claim representing fulfilment of the exit payment obligation.

#### **Off-Balance Sheet Arrangements**

As of December 31, 2019 and 2018, we did 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, revenues or expenses, results of operations, liquidity, capital expenditures or capital resources that is material to investors.

#### **ITEM 7A. QUANTITATIVE AND QUALITATIVE DISCLOSURE ABOUT MARKET RISK**

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

#### **ITEM 8. FINANCIAL STATEMENTS AND SUPPLEMENTARY DATA**

The consolidated financial statements and supplementary data required by this item are set forth indicated in Item 15, set forth in this Report.

## **ITEM 9. CHANGES IN AND DISAGREEMENTS WITH ACCOUNTANTS ON ACCOUNTING AND FINANCIAL DISCLOSURE**

The Company's annual financial statements for the fiscal year ended December 31, 2017 were audited by Kost Forer Gabbay & Kasierer, a member of Ernst & Young Global, or EY, as the independent registered public accounting firm. On June 18, 2018, the Company appointed Deloitte & Touche LLP, or Deloitte, as the independent registered public accounting firm to audit the Company's financial statements for the year ended December 31, 2018. During the fiscal year ended December 31, 2017 and the subsequent interim period through June 18, 2018, there were (1) no disagreements (as defined in Item 301(a)(1)(iv) of Regulation S-K) with EY on any matter of accounting principles or practices, financial statement disclosure, or auditing scope or procedure, which disagreement, if not resolved to the satisfaction of EY, would have caused EY to make reference thereto in their reports on the Company's financial statements, and (ii) no "reportable events" (as defined in Item 304(a)(1)(v) of Regulation S-K).

During the year ended December 31, 2019 and the subsequent interim period, neither the Company nor anyone acting on its behalf consulted Deloitte regarding the application of accounting principles to a specified transaction, either completed or proposed, or the type of audit opinion that might be rendered on the Company's financial statements.

## **ITEM 9A. 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 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 December 31, 2019, our management, under the supervision 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 Securities and Exchange Act of 1934 (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 December 31, 2019, 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 periods 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 disclosure.

### **Management's Report on Internal Control over Financial Reporting**

Our management is responsible for establishing and maintaining adequate internal control over financial reporting, as such term is defined in Exchange Act Rules 13a-15(f) and 15d-15(f). Internal control over financial reporting is a process designed to provide reasonable assurance regarding the reliability of financial reporting and the preparation of financial statements for external purposes in accordance with generally accepted accounting principles.

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

Under the supervision and with the participation of our management, including our Chief Executive Officer and Chief Financial Officer, we conducted an evaluation of the effectiveness of our internal control over financial reporting as of December 31, 2019 based on the guidelines established in *Internal Control-Integrated Framework* (2013) issued by the Committee of Sponsoring Organizations of the Treadway Commission (“COSO”). Based on this evaluation, our management concluded that our internal control over financial reporting was effective as of December 31, 2019.

The effectiveness of our internal control over financial reporting as of December 31, 2019 has been audited by Deloitte & Touche LLP, an independent registered public accounting firm, as stated in their report, which appears herein.

### **Changes in Internal Control over Financial Reporting**

During the quarter ended December 31, 2019, 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.

## **REPORT OF INDEPENDENT REGISTERED PUBLIC ACCOUNTING FIRM**

To the stockholders and the Board of Directors of Eloxx Pharmaceuticals, Inc.

### **Opinion on Internal Control over Financial Reporting**

We have audited the internal control over financial reporting of Eloxx Pharmaceuticals, Inc. and subsidiaries (the “Company”) as of December 31, 2019, based on criteria established in *Internal Control — Integrated Framework (2013)* issued by the Committee of Sponsoring Organizations of the Treadway Commission (COSO). In our opinion, the Company maintained, in all material respects, effective internal control over financial reporting as of December 31, 2019, based on criteria established in *Internal Control — Integrated Framework (2013)* issued by COSO.

We have also audited, in accordance with the standards of the Public Company Accounting Oversight Board (United States) (PCAOB), the consolidated financial statements as of and for the year ended December 31, 2019, of the Company and our report dated March 6, 2020, expressed an unqualified opinion on those financial statements.

### **Basis for Opinion**

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

We conducted our audit in accordance with the standards of the PCAOB. Those standards require that we plan and perform the audit to obtain reasonable assurance about whether effective internal control over financial reporting was maintained in all material respects. Our audit included obtaining an understanding of internal control over financial reporting, assessing the risk that a material weakness exists, testing and evaluating the design and operating effectiveness of internal control based on the assessed risk, and performing such other procedures as we considered necessary in the circumstances. We believe that our audit provides a reasonable basis for our opinion.

### **Definition and Limitations of Internal Control over Financial Reporting**

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

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

/s/ Deloitte & Touche LLP

Boston, Massachusetts  
March 6, 2020

**ITEM 9B. OTHER INFORMATION**

None.

## **PART III**

### **ITEM 10. DIRECTORS, EXECUTIVE OFFICERS AND CORPORATE GOVERNANCE**

The information required by this Item 10 will be included under the captions “Executive Officers,” “Election of Directors,” “Section 16(a) Beneficial Ownership Reporting Compliance,” “Code of Ethics,” “Information Regarding Committees of the Board of Directors” and “Information regarding the Board of Directors and Corporate Governance” in our definitive proxy statement to be filed pursuant to Regulation 14A within 120 days after the end of the fiscal year covered by this Report.

### **ITEM 11. EXECUTIVE AND DIRECTOR COMPENSATION**

The information required by this Item 11 will be included under the captions “Executive Compensation” and “Director Compensation” in our definitive proxy statement to be filed pursuant to Regulation 14A within 120 days after the end of the fiscal year covered by this Report.

### **ITEM 12. SECURITY OWNERSHIP OF CERTAIN BENEFICIAL OWNERS AND MANAGEMENT AND RELATED STOCKHOLDER MATTERS**

The information required by this Item 12 will be included under the captions “Security Ownership of Certain Beneficial Owners and Management” and “Equity Compensation Plan Information” in our definitive proxy statement to be filed pursuant to Regulation 14A within 120 days after the end of the fiscal year covered by this Report.

### **ITEM 13. CERTAIN RELATIONSHIPS AND RELATED TRANSACTIONS, AND DIRECTOR INDEPENDENCE**

The information required by this Item 13 will be included, as applicable, under the captions of “Independence of the Board of Directors,” “Employment Arrangements” and “Transactions with Related Persons” in our definitive proxy statement to be filed pursuant to Regulation 14A within 120 days after the end of the fiscal year covered by this Report.

### **ITEM 14. PRINCIPAL ACCOUNTING FEES AND SERVICES**

The information required by this Item 14 will be included under the captions “Principal Accountant Fees and Services” and “Pre-Approval Policies and Procedures” in our definitive proxy statement to be filed pursuant to Regulation 14A within 120 days after the end of the fiscal year covered by this Report.

## **PART IV**

### **ITEM 15. EXHIBITS AND FINANCIAL STATEMENT SCHEDULES**

#### **Item 15(a)**

##### **(1) Financial Statements**

The financial statements required by this item are submitted in a separate section beginning on page F-1 of this Report.

##### **(2) Financial Statement Schedules**

Schedules have been omitted because of the absence of conditions under which they are required or because the required information is included in the financial statements or notes thereto beginning on page F-1 of this Report.

##### **(3) Exhibits:**

The exhibits listed in the Exhibit Index at the end of this report are filed or incorporated by reference as part of this Report.

#### **Item 15(b) Exhibits**

See (a)(3) above.

#### **Item 15(c) Financial Statement Schedules**

See (a)(2) above.

### **ITEM 16. FORM 10-K SUMMARY**

Not applicable.

## EXHIBIT INDEX

Exhibit No.	Description of Exhibit
2.1	<a href="#"><u>Agreement, dated as of May 31, 2017, by and among Sevion Therapeutics, Inc., Sevion Sub, Ltd. and Eloxx Pharmaceuticals Ltd. (incorporated by reference to Exhibit 2.1 of the Company's Current Report on Form 8-K filed on June 6, 2017, SEC File No. 001-31326)</u></a>
2.2	<a href="#"><u>Amendment to Agreement, dated as of August 1, 2017, by and among Sevion Therapeutics, Inc., Sevion Sub, Ltd. and Eloxx Pharmaceuticals Ltd. (incorporated by reference to Exhibit 2.3 of the Company's Annual Report on Form 10-K filed on October 13, 2017, SEC File No. 001-31326)</u></a>
2.3	<a href="#"><u>Second Amendment to Agreement, dated as of November 23, 2017, by and among Sevion Therapeutics, Inc., Sevion Sub, Ltd. and Eloxx Pharmaceuticals Ltd. (incorporated by reference to Exhibit 2.1 of the Company's Current Report on Form 8-K filed on November 29, 2017, SEC File No. 001-31326)</u></a>
3.1	<a href="#"><u>Amended and Restated Certificate of Incorporation of Senesco Technologies, Inc. filed with the State of Delaware on January 22, 2007. (incorporated by reference to Exhibit 3.1 of our Quarterly Report on Form 10-Q filed on February 14, 2007, SEC File No. 001-31326).</u></a>
3.2	<a href="#"><u>Certificate of Amendment to the Amended and Restated Certificate of Incorporation of Senesco Technologies, Inc. filed with the State of Delaware on December 13, 2007. (incorporated by reference to Exhibit 3.1 of our Quarterly Report on Form 10-Q filed on February 14, 2008, SEC File No. 001-31326).</u></a>
3.3	<a href="#"><u>Certificate of Amendment to the Amended and Restated Certificate of Incorporation of Senesco Technologies, Inc. filed with the State of Delaware on September 22, 2009. (incorporated by reference to Exhibit 3.3 of our Annual Report on Form 10-K filed on September 28, 2009, SEC File No. 001-31326).</u></a>
3.4	<a href="#"><u>Certificate of Amendment to the Amended and Restated Certificate of Incorporation of Senesco Technologies, Inc. filed with the State of Delaware on May 25, 2010. (incorporated by reference to Exhibit 3.1 to our Current Report on Form 8-K filed on May 28, 2010, SEC File No. 001-31326).</u></a>
3.5	<a href="#"><u>Certificate of Amendment to the Amended and Restated Certificate of Incorporation of Senesco Technologies, Inc. filed with the State of Delaware on December 22, 2011. (incorporated by reference to Exhibit 3.1 to our Quarterly Report on Form 10-Q filed on February 14, 2011, SEC File No. 001-31326).</u></a>
3.6	<a href="#"><u>Certificate of Amendment to the Amended and Restated Certificate of Incorporation of Senesco Technologies, Inc. filed with the State of Delaware on April 1, 2013. (incorporated by reference to Exhibit 3.1 to our Quarterly Report on Form 10-Q filed on May 15, 2013, SEC File No. 001-31326).</u></a>
3.7	<a href="#"><u>Certificate of Amendment to the Company's Amended and Restated Certificate of Incorporation, as filed with the Secretary of State of the State of Delaware on October 16, 2013. (incorporated by reference to Exhibit 3.1 of our Current Report on Form 8-K filed on October 21, 2013, SEC File No. 001-31326).</u></a>
3.8	<a href="#"><u>Certificate of Amendment to the Company's Amended and Restated Certificate of Incorporation, as filed with the Secretary of State of the State of Delaware on September 29, 2014. (incorporated by reference to Exhibit 3.1 of our Current Report on Form 8-K filed on October 3, 2014, SEC File No. 001-31326).</u></a>
3.9	<a href="#"><u>Certificate of Amendment to the Company's Amended and Restated Certificate of Incorporation, as filed with the Secretary of State of the State of Delaware on December 19, 2017. (incorporated by reference to Exhibit 3.1 of our Current Report on Form 8-K filed on December 22, 2017, SEC File No. 001-31326).</u></a>
3.10	<a href="#"><u>Certificate of Amendment to the Company's Amended and Restated Certificate of Incorporation, as filed with the Secretary of State of the State of Delaware on December 19, 2017. (incorporated by reference to Exhibit 3.2 of our Current Report on Form 8-K filed on December 22, 2017, SEC File No. 001-31326).</u></a>
3.11	<a href="#"><u>Certificate of Designations to the Company's Certificate of Incorporation. (Series A) (incorporated by reference to Exhibit 3.1 to our Current Report on Form 8-K filed on March 29, 2010, SEC File No. 001-31326).</u></a>

3.12	<a href="#">Certificate of Designations to the Company's Certificate of Incorporation. (0% Series C Convertible Preferred Stock) (incorporated by reference to Exhibit 3.1 of our Current Report on Form 8-K filed on May 6, 2015, SEC File No. 001-31326).</a>
3.13	<a href="#">Amended and Restated Bylaws of Eloxx Pharmaceuticals, Inc. (incorporated by reference to Exhibit 3.2 of the Company's Current Report on Form 8-K filed on December 27, 2017, SEC File No. 001-31326).</a>
4.1	<a href="#">Specimen of Common Stock Certificate (incorporated by reference to Exhibit 4.1 of our Annual Report on Form 10-K filed on March 16, 2018, SEC File No. 001-31326).</a>
10.1*	<a href="#">Research and License Agreement by and between Technion Research and Development Foundation Ltd. and Eloxx Pharmaceuticals Ltd., dated August 29, 2013 (incorporated by reference to Exhibit 10.1 of our Annual Report on Form 10-K filed on March 16, 2018, SEC File No. 001-31326).</a>
10.2*	<a href="#">First Amendment to Research and License Agreement by and between Technion Research and Development Foundation Ltd. and Eloxx Pharmaceuticals Ltd., dated November 26, 2013 (incorporated by reference to Exhibit 10.2 of our Annual Report on form 10-K filed on March 16, 2018, SEC File No. 001-31326).</a>
10.3	<a href="#">Second Amendment to Research and License Agreement by and between Technion Research and Development Foundation Ltd. and Eloxx Pharmaceuticals Ltd., dated January 14, 2014 (incorporated by reference to Exhibit 10.3 of our Annual Report on form 10-K filed on March 16, 2018, SEC File No. 001-31326).</a>
10.4	<a href="#">Third Amendment to Research and License Agreement by and between Technion Research and Development Foundation Ltd. and Eloxx Pharmaceuticals Ltd., dated June 9, 2014 (incorporated by reference to Exhibit 10.4 of our Annual Report on form 10-K filed on March 16, 2018, SEC File No. 001-31326).</a>
10.5	<a href="#">First Addendum to Research and License Agreement by and between Technion Research and Development Foundation Ltd. and Eloxx Pharmaceuticals Ltd., dated August 3, 2014 (incorporated by reference to Exhibit 10.5 of our Annual Report on form 10-K filed on March 16, 2018, SEC File No. 001-31326).</a>
10.6	<a href="#">Second Addendum to Research and License Agreement by and between Technion Research and Development Foundation Ltd. and Eloxx Pharmaceuticals Ltd., dated January 21, 2015 (incorporated by reference to Exhibit 10.6 of our Annual Report on form 10-K filed on March 16, 2018, SEC File No. 001-31326).</a>
10.7	<a href="#">Third Addendum to Research and License Agreement by and between Technion Research and Development Foundation Ltd. and Eloxx Pharmaceuticals Ltd., dated February 9, 2015 (incorporated by reference to Exhibit 10.7 of our Annual Report on form 10-K filed on March 16, 2018, SEC File No. 001-31326).</a>
10.8	<a href="#">Fourth Addendum to Research and License Agreement by and between Technion Research and Development Foundation Ltd. and Eloxx Pharmaceuticals Ltd., dated April 29, 2015 (incorporated by reference to Exhibit 10.8 of our Annual Report on form 10-K filed on March 16, 2018, SEC File No. 001-31326).</a>
10.9	<a href="#">Fifth Addendum to Research and License Agreement by and between Technion Research and Development Foundation Ltd. and Eloxx Pharmaceuticals Ltd., dated June 2, 2015 (incorporated by reference to Exhibit 10.9 of our Annual Report on form 10-K filed on March 16, 2018, SEC File No. 001-31326).</a>
10.10	<a href="#">Sixth Addendum to Research and License Agreement by and between Technion Research and Development Foundation Ltd. and Eloxx Pharmaceuticals Ltd., dated January 11, 2016 (incorporated by reference to Exhibit 10.10 of our Annual Report on form 10-K filed on March 16, 2018, SEC File No. 001-31326).</a>

Exhibit No.	Description of Exhibit
10.11	<a href="#">Seventh Addendum to Research and License Agreement by and between Technion Research and Development Foundation Ltd. and Eloxx Pharmaceuticals Ltd., dated March 6, 2016 (incorporated by reference to Exhibit 10.11 of our Annual Report on form 10-K filed on March 16, 2018, SEC File No. 001-31326).</a>
10.12	<a href="#">Eighth Addendum to Research and License Agreement by and between Technion Research and Development Foundation Ltd. and Eloxx Pharmaceuticals Ltd., dated July 16, 2017 (incorporated by reference to Exhibit 10.12 of our Annual Report on form 10-K filed on March 16, 2018, SEC File No. 001-31326).</a>
10.13	<a href="#">Ninth Addendum to Research and License Agreement by and between Technion Research and Development Foundation Ltd. and Eloxx Pharmaceuticals Ltd., dated July 16, 2017 (incorporated by reference to Exhibit 10.13 of our Annual Report on form 10-K filed on March 16, 2018, SEC File No. 001-31326).</a>
10.14	<a href="#">Amendment to Research and License Agreement, by and among Eloxx Pharmaceuticals, Inc., Eloxx Pharmaceuticals Ltd. and Technion Research &amp; Development Foundation Ltd., dated as of June 13, 2018 (incorporated by reference to Exhibit 10.1 of our Current Report on Form 8-K filed on June 14, 2018, SEC File No. 001-31326).</a>
10.15**	<a href="#">Consulting Agreement, dated December 1, 2014, by and between Eloxx Pharmaceuticals Ltd. and Dr. Silvia Noiman (incorporated by reference to Exhibit 10.1 of our Current Report on Form 8-K filed on December 22, 2017, SEC File No. 001-31326).</a>
10.16**	<a href="#">Memorandum of Understanding, dated March 13, 2018, by and between Eloxx Pharmaceuticals, Inc. and Dr. Silvia Noiman (incorporated by reference to Exhibit 10.15 of our Quarterly Report on Form 10-Q filed on May 10, 2018, SEC File No. 001-31326).</a>
10.17**	<a href="#">Employment Agreement, dated as of December 26, 2017, between Eloxx Pharmaceuticals, Inc. and Robert E. Ward (incorporated by reference to our Current Report on Form 8-K filed on December 27, 2017, SEC File No. 001-31326).</a>
10.18**	<a href="#">Employment Agreement, dated as of March 12, 2018, between Eloxx Pharmaceuticals Inc. and Gregory Weaver (incorporated by reference to Exhibit 10.19 of our Annual Report on Form 10-K filed on March 16, 2018, SEC File No. 001-31326).</a>
10.19**	<a href="#">Form of Indemnification Agreement (incorporated by reference to Exhibit 10.4 of our Current Report on Form 8-K filed on December 22, 2017, SEC File No. 001-31326).</a>
10.20**	<a href="#">Amended and Restated Senesco Technologies, Inc. 2008 Incentive Compensation Plan. (incorporated by reference to Exhibit 10.3 of our Quarterly Report on Form 10-Q for the period ended March 31, 2014, SEC File No. 001-31326)</a>
10.21**	<a href="#">Form of Stock Option Agreement under the Senesco Technologies, Inc. 2008 Stock Incentive Plan. (incorporated by reference to Exhibit 10.5 of our Quarterly Report on Form 10-Q for the period ended September 30, 2009, SEC File No. 001-31326).</a>
10.22**	<a href="#">Eloxx Pharmaceuticals Share Ownership and Option Plan (2013) (incorporated by reference to Exhibit 10.24 of our Annual Report on Form 10-K filed on March 16, 2018, SEC File No. 001-31326).</a>
10.23**	<a href="#">Forms of Option Agreement, Stock Option Grant Notice and Notice of Exercise under the Eloxx Pharmaceuticals Share Ownership and Option Plan (2013) (incorporated by reference to Exhibit 10.25 of our Annual Report on Form 10-K filed on March 16, 2018, SEC File No. 001-31326).</a>
10.24**	<a href="#">Performance Stock Option Grant Notice and Stock Option Agreement (Inducement Grant) between Eloxx Pharmaceuticals, Inc. and Robert E. Ward, dated March 5, 2018 (incorporated by reference to Exhibit 10.25 of our Annual Report on Form 10-K filed on March 16, 2018, SEC File No. 001-31326).</a>
10.25**	<a href="#">Restricted Stock Unit Grant Notice and Restricted Stock Unit Agreement (Inducement Grant) between Eloxx Pharmaceuticals, Inc. and Robert E. Ward, dated March 5, 2018 (incorporated by reference to Exhibit 10.25 of our Annual Report on Form 10-K filed on March 16, 2018, SEC File No. 001-31326).</a>
10.26**	<a href="#">Performance Restricted Stock Unit Grant Notice and Restricted Stock Unit Agreement (Inducement Grant) between Eloxx Pharmaceuticals, Inc. and Robert E. Ward, dated March 5, 2018 (incorporated by reference to Exhibit 10.25 of our Annual Report on Form 10-K filed on March 16, 2018, SEC File No. 001-31326).</a>

10.27**	<a href="#"><u>Stock Option Grant Notice and Stock Option Agreement (Inducement Grant) between Eloxx Pharmaceuticals, Inc. and Robert E. Ward, dated March 5, 2018 (incorporated by reference to Exhibit 10.25 of our Annual Report on Form 10-K filed on March 16, 2018, SEC File No. 001-31326).</u></a>
10.28**	<a href="#"><u>Retention Policy (incorporated by reference to Exhibit 10.1 of our Current Report on Form 8-K filed on October 15, 2012, SEC File No. 001-31326).</u></a>
10.29	<a href="#"><u>Lease Agreement by and between Eloxx Pharmaceuticals, Inc. and BP Bay Colony LLC, dated October 26, 2017 (incorporated by reference to Exhibit 10.31 of our Annual Report on Form 10-K filed on March 16, 2018, SEC File No. 001-31326).</u></a>
10.30	<a href="#"><u>Eloxx Pharmaceuticals, Inc. 2018 Equity Incentive Plan (incorporated by reference to Exhibit 10.1 of our Current Report on Form 8-K filed on March 30, 2018, SEC File No. 001-31326).</u></a>
10.31	<a href="#"><u>Form of Stock Option Grant Notice, Option Agreement and Notice of Exercise under the Eloxx Pharmaceuticals, Inc. 2018 Equity Incentive Plan (incorporated by reference to Exhibit 10.2 of our Current Report on Form 8-K filed on March 30, 2018, SEC File No. 001-31326).</u></a>
10.32	<a href="#"><u>Form of Restricted Stock Unit Grant Notice for non-Israeli employees (incorporated by reference to Exhibit 99.5 of our Registration Statement on Form S-3 filed on May 11, 2018, SEC File No. 333-224860).</u></a>
10.33	<a href="#"><u>Israeli Sub-Plan under the Eloxx Pharmaceuticals, Inc. 2018 Equity Incentive Plan (incorporated by reference to Exhibit 10.3 of our Current Report on Form 8-K filed on March 30, 2018, SEC File No. 001-31326).</u></a>
10.34	<a href="#"><u>Form of Israeli Stock Option Grant Package under the Israeli Sub-Plan under the Eloxx Pharmaceuticals, Inc. 2018 Equity Incentive Plan (incorporated by reference to Exhibit 10.4 of our Current Report on Form 8-K filed on March 30, 2018, SEC File No. 001-31326).</u></a>
10.35	<a href="#"><u>Form of Restricted Stock Unit Grant Notice for Israeli employees (incorporated by reference to Exhibit 10.6 of our Quarterly Report on Form 10-Q filed on August 10, 2018, SEC File No. 001-31326).</u></a>
10.36	<a href="#"><u>First Amendment to Lease Agreement by and between Eloxx Pharmaceuticals, Inc. and BP Bay Colony LLC, dated June 21, 2018 (incorporated by reference to Exhibit 10.1 of our Current Report on Form 8-K filed on June 26, 2018, SEC File No. 001-31326).</u></a>
10.37**	<a href="#"><u>Executive Employment Agreement between Eloxx Pharmaceuticals, Inc. and Gregory C. Williams dated as of June 22, 2018 (incorporated by reference to Exhibit 10.2 of our Current Report on Form 8-K filed on June 26, 2018, SEC File No. 001-31326).</u></a>
10.38	<a href="#"><u>Executive Employment Agreement between Eloxx Pharmaceuticals, Inc. and David Snow dated as of June 18, 2018 (incorporated by reference to Exhibit 10.38 of our Annual Report on Form 10-K filed on March 14, 2019, SEC File No. 001-31326).</u></a>
10.39	<a href="#"><u>Equity Distribution Agreement, dated November 16, 2018, by and between Eloxx Pharmaceuticals, Inc., Citigroup Global Markets Inc. and Cantor Fitzgerald &amp; Co. (incorporated by reference to Exhibit 10.1 of our Current Report on Form 8-K, filed on November 16, 2018, SEC File No. 001-31326).</u></a>
10.40	<a href="#"><u>Loan and Security Agreement, dated as of January 30, 2019, by and among Silicon Valley Bank, WestRiver Innovation Lending Fund VIII, L.P., Eloxx Pharmaceuticals, Inc., and Eloxx Pharmaceuticals Ltd (incorporated by reference to Exhibit 10.40 of our Annual Report on Form 10-K filed on March 14, 2019, SEC File No. 001-31326).</u></a>
10.41	<a href="#"><u>Change in Control Severance Benefit Plan, dated September 17, 2019 (incorporated by reference to Exhibit 10.1 of our Quarterly Report on Form 10-Q filed on November 6, 2019, SEC File No. 001-31326).</u></a>
10.42**	<a href="#"><u>Executive Employment Agreement between Eloxx Pharmaceuticals, Inc. and Gregory C. Williams dated as of February 25, 2020.</u></a>

Exhibit No.	Description of Exhibit
10.43**	<a href="#"><u>Executive Employment Agreement between Eloxx Pharmaceuticals, Inc. and Neil Belloff dated as of February 25, 2020.</u></a>
10.44**	<a href="#"><u>Employment offer letter between Eloxx Pharmaceuticals, Inc. and Stephen G. MacDonald dated as of September 24, 2019.</u></a>
16.1	<a href="#"><u>Letter of Kost Forer Gabbay &amp; Kasierer, a Member of Ernst &amp; Young Global, dated June 21, 2018 (incorporated by reference to Exhibit 16.1 of our Current Report on Form 8-K filed on June 21, 2018, SEC File No. 001-31326).</u></a>
21.1	<a href="#"><u>List of Subsidiaries of the Company.</u></a>
23.1	<a href="#"><u>Consent of Deloitte &amp; Touche LLP, Independent Registered Public Accounting Firm.</u></a>
23.2	<a href="#"><u>Consent of Kost Forer Gabbay &amp; Kasierer, a member of Ernst &amp; Young Global, Independent Registered Public Accounting Firm.</u></a>
31.1	<a href="#"><u>Certification of the Company's Chief 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.</u></a>
31.2	<a href="#"><u>Certification of the Company's Chief 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.</u></a>
32.1	<a href="#"><u>Certification of the Company's Chief Executive Officer pursuant to 18 U.S.C. Section 1350, as adopted pursuant to Section 906 of the Sarbanes-Oxley Act of 2002.</u></a>
32.2	<a href="#"><u>Certification of the Company's Chief Financial Officer pursuant to 18 U.S.C. Section 1350, as adopted pursuant to Section 906 of the Sarbanes-Oxley Act of 2002.</u></a>
101.INS	XBRL Instance Document
101.SCH	XBRL Taxonomy Extension Schema Document
101.CAL	XBRL Taxonomy Extension Calculation Document
101.DEF	XBRL Taxonomy Extension Definition Linkbase Document
101.LAB	XBRL Taxonomy Extension Labels Linkbase Document
101.PRE	XBRL Taxonomy Extension Presentation Link Document

- 
- \* Confidential treatment requested under 17 C.F.R. §§200.80(b)(4) and 24b-2. The confidential portions of this exhibit have been omitted and are marked accordingly. The confidential portions have been filed separately with the Securities and Exchange Commission pursuant to the confidential treatment request.
- \*\* Indicates a management contract or compensatory plan or arrangement required to be filed pursuant to Item 15(b) of Form 10-K

**SIGNATURES**

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

**ELOXX PHARMACEUTICALS, INC.**  
(Registrant)

Date: March 6, 2020

/s/ Gregory C. Williams

Gregory C. Williams

*Chief Executive Officer*

*(On behalf of the Registrant and as Principal Executive Officer)*

## POWER OF ATTORNEY

KNOW ALL PERSONS BY THESE PRESENTS, that each person whose signature appears below constitutes and appoints Gregory C. Williams and Gregory Weaver, and each of them, as his or her true and lawful attorneys-in-fact and agents, each with the full power of substitution, for him or her and in his or her name, place or stead, in any and all capacities, to sign any and all amendments to this report, with exhibits thereto and other documents in connection therewith, with the U.S. Securities and Exchange Commission, granting unto said attorneys-in-fact and agents, and each of them, full power and authority to do and perform each and every act and thing requisite and necessary to be done in and about the premises, as fully to all intents and purposes as he or she might or could do in person, hereby ratifying and confirming all that said attorneys-in-fact and agents, or their, his substitute or substitutes, may lawfully do or cause to be done by virtue hereof.

Pursuant to the requirements of the Securities Exchange Act of 1934, as amended this report has been signed below by the following persons on behalf of the Registrant and in the capacities and on the dates indicated.

<b>Signature</b>	<b>Title</b>	<b>Date</b>
/s/ Gregory C. Williams Gregory C. Williams	Chief Executive Officer (Principal Executive Officer)	March 6, 2020
/s/ Gregory Weaver Gregory Weaver	Chief Financial Officer (Principal Financial Officer)	March 6, 2020
/s/ Stephen MacDonald Stephen MacDonald	Vice President, Finance and Accounting, and Treasurer (Principal Accounting Officer)	March 6, 2020
/s/ Tomer Kariv Tomer Kariv	Chairman of the Board of Directors	March 6, 2020
/s/ Ran Nussbaum Ran Nussbaum	Director	March 6, 2020
/s/ Silvia Noiman Silvia Noiman, Ph.D.	Director	March 6, 2020
/s/ Gadi Veinrib Gadi Veinrib	Director	March 6, 2020
/s/ Zafrira Avnur Zafrira Avnur, Ph.D.	Director	March 6, 2020
/s/ Martijn Kleijwegt Martijn Kleijwegt	Director	March 6, 2020
/s/ Steven D. Rubin Steven D. Rubin	Director	March 6, 2020
/s/ Jasbir Seehra Jasbir Seehra, Ph.D.	Director	March 6, 2020

**ELOXX PHARMACEUTICALS, INC. AND SUBSIDIARIES**  
**INDEX TO CONSOLIDATED FINANCIAL STATEMENTS**

	<u>Page</u>
<u><a href="#">Report of Independent Registered Public Accounting Firm</a></u>	F-2
<u><a href="#">Consolidated Balance Sheets as of December 31, 2019 and 2018</a></u>	F-4
<u><a href="#">Consolidated Statements of Operations and Comprehensive Loss for the Years Ended December 31, 2019, 2018 and 2017</a></u>	F-5
<u><a href="#">Consolidated Statements of Convertible Preferred Stock and Stockholders' Equity for the Years Ended December 31, 2019, 2018 and 2017</a></u>	F-6
<u><a href="#">Consolidated Statements of Cash Flows for the Years Ended December 31, 2019, 2018 and 2017</a></u>	F-7
<u><a href="#">Notes to Consolidated Financial Statements</a></u>	F-9

## **REPORT OF INDEPENDENT REGISTERED PUBLIC ACCOUNTING FIRM**

To the stockholders and the Board of Directors of Eloxx Pharmaceuticals, Inc.

### **Opinion on the Financial Statements**

We have audited the accompanying consolidated balance sheets of Eloxx Pharmaceuticals, Inc. and subsidiaries (the "Company") as of December 31, 2019 and 2018, the related consolidated statements of operations and comprehensive loss, convertible preferred stock and stockholder's equity, and cash flows, for each of the two years in the period ended December 31, 2019, and the related notes (collectively referred to as the "financial statements"). In our opinion, the financial statements present fairly, in all material respects, the financial position of the Company as of December 31, 2019 and 2018, and the results of its operations and its cash flows for each of the two years in the period ended December 31, 2019, in conformity with accounting principles generally accepted in the United States of America.

We have also audited, in accordance with the standards of the Public Company Accounting Oversight Board (United States) (PCAOB), the Company's internal control over financial reporting as of December 31, 2019, based on criteria established in *Internal Control — Integrated Framework (2013)* issued by the Committee of Sponsoring Organizations of the Treadway Commission and our report dated March 6, 2020, expressed an unqualified opinion on the Company's internal control over financial reporting.

### **Basis for Opinion**

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

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

/s/ Deloitte & Touche LLP

Boston, Massachusetts  
March 6, 2020

We have served as the Company's auditor since 2018.



**Kost Forer Gabbay & Kasierer**  
144 Menachem Begin Road, Building A  
Tel-Aviv 6492102, Israel

Tel: +972-3-6232525  
Fax: +972-3-5622555  
ey.com

## REPORT OF INDEPENDENT REGISTERED PUBLIC ACCOUNTING FIRM

### Opinion on the Financial Statements

We have audited the accompanying consolidated balance sheets of Eloxx Pharmaceuticals, Inc. (the "Company") as of December 31, 2017, the related consolidated statements of operations, stockholders' equity and cash flows for each of the three years in the period ended December 31, 2017 and the related notes (collectively referred to as the "consolidated financial statements"). In our opinion, the consolidated financial statements present fairly, in all material respects, the financial position of the Company at December 31, 2017 and the results of its operations and its cash flows for each of the three years in the period ended December 31, 2017, in conformity with U.S. generally accepted accounting principles.

### Basis for Opinion

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

We conducted our audits in accordance with the standards of the PCAOB. Those standards require that we plan and perform the audit to obtain reasonable assurance about whether the financial statements are free of material misstatement, whether due to error or fraud. The Company is not required to have, nor were we engaged to perform, an audit of its internal control over financial reporting. As part of our audits we are required to obtain an understanding of internal control over financial reporting but not for the purpose of expressing an opinion on the effectiveness of the Company's internal control over financial reporting. Accordingly, we express no such opinion. Audits included performing procedures to assess the risks of material misstatement of the financial statements, whether due to error or fraud, and performing procedures that respond to those risks. Such procedures included examining, on a test basis, evidence regarding the amounts and disclosures in the financial statements. Our audits also included evaluating the accounting principles used and significant estimates made by management, as well as evaluating the overall presentation of the financial statements. We believe that our audits provide a reasonable basis for our opinion.

/s/ KOST FORER GABBAY & KASIERER  
A Member of Ernst & Young Global

Tel-Aviv, Israel  
March 16, 2018

**ELOXX PHARMACEUTICALS, INC. AND SUBSIDIARIES**

**CONSOLIDATED BALANCE SHEETS**  
**(Amounts in thousands, except share and per share data)**

	December 31,	
	2019	2018
<b>Assets</b>		
Current assets:		
Cash and cash equivalents	\$ 22,493	\$ 48,606
Marketable securities	33,783	—
Restricted cash	43	45
Prepaid expenses and other current assets	1,390	1,690
Total current assets	57,709	50,341
Property and equipment, net	201	248
Operating lease right-of-use asset	924	—
Other long-term assets	113	129
Total assets	\$ 58,947	\$ 50,718
<b>Liabilities and Stockholders' Equity</b>		
Current liabilities:		
Accounts payable	\$ 1,871	\$ 747
Accrued expenses	4,655	6,938
Current portion of long-term debt	4,336	—
Advances from collaboration partners	403	—
Current portion of operating lease liability	499	—
Taxes payable	43	122
Total current liabilities	11,807	7,807
Long-term debt	10,502	—
Operating lease liability	425	—
Total liabilities	22,734	7,807
Commitments and contingencies		
Stockholders' equity:		
Preferred stock, \$0.01 par value per share, 5,000,000 authorized, no shares issued or outstanding as of December 31, 2019 and 2018, respectively	—	—
Common stock, \$0.01 par value per share, 500,000,000 shares authorized, 40,186,469 and 35,951,537 shares issued and 40,030,763 and 35,860,114 shares outstanding as of December 31, 2019 and 2018, respectively	402	360
Common stock in treasury, at cost, 155,706 and 91,423 shares as of December 31, 2019 and 2018, respectively	(1,703)	(1,129)
Additional paid-in capital	174,515	129,825
Accumulated other comprehensive income	18	—
Accumulated deficit	(137,019)	(86,145)
Total stockholders' equity	36,213	42,911
Total liabilities and stockholders' equity	\$ 58,947	\$ 50,718

*See accompanying notes consolidated financial statements*

**ELOXX PHARMACEUTICALS, INC. AND SUBSIDIARIES**

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

	Year ended December 31,		
	2019	2018	2017
<b>Operating expenses:</b>			
Research and development, net	\$ 25,842	\$ 20,489	\$ 16,398
General and administrative	24,713	26,482	2,702
Reverse merger related expenses	—	594	1,290
Total operating expenses	<u>50,555</u>	<u>47,565</u>	<u>20,390</u>
Loss from operations	(50,555)	(47,565)	(20,390)
Other (income) expense, net	319	(502)	824
Loss before income taxes	(50,874)	(47,063)	(21,214)
Provision for income taxes	—	122	—
Net loss	(50,874)	(47,185)	(21,214)
Less: dividends accumulated for the period	—	—	(2,404)
Net loss attributable to common stockholders	<u>\$ (50,874)</u>	<u>\$ (47,185)</u>	<u>\$ (23,618)</u>
Net loss per share, basic and diluted	<u>\$ (1.34)</u>	<u>\$ (1.45)</u>	<u>\$ (4.75)</u>
Weighted average number of shares of common stock used in computing net loss per share, basic and diluted	<u>38,063,173</u>	<u>32,436,506</u>	<u>4,976,377</u>
<b>Comprehensive loss:</b>			
Net loss attributable to common stockholders	\$ (50,874)	\$ (47,185)	\$ (23,618)
Other comprehensive gain (loss):			
Unrealized gain (loss) from available-for-sale securities	18	—	—
Comprehensive loss	<u>\$ (50,856)</u>	<u>\$ (47,185)</u>	<u>\$ (23,618)</u>

*See accompanying notes consolidated financial statements*

**ELOXX PHARMACEUTICALS, INC. AND SUBSIDIARIES**

**CONSOLIDATED STATEMENTS OF CONVERTIBLE PREFERRED STOCK AND STOCKHOLDERS' EQUITY**  
**(Amounts in thousands, except share and per share data)**

	Preferred Stock		Common Stock		Additional paid-in capital	Accumulated other comprehensive income	Treasury Stock		Accumulated deficit	Total stockholders' equity
	Shares	Amount	Shares	Amount			Shares	Amount		
Balance as of December 31, 2016	7,638,263	\$ 76	4,205,278	\$ 42	\$ 18,238	\$ —	—	\$ —	\$ (17,746)	\$ 610
Issuance of Series C preferred stock, net of \$573 issuance costs	6,311,076	63	—	—	18,364	—	—	—	—	18,427
Conversion of convertible loan into Series C preferred stock of \$0.01 par value	825,213	8	—	—	3,160	—	—	—	—	3,168
Exercise of stock options	—	—	16,699	—	17	—	—	—	—	17
Stock-based compensation expense	—	—	—	—	101	—	—	—	—	101
Issuance of common stock, net of \$494 issuance costs	—	—	6,333,333	63	16,943	—	—	—	—	17,006
Conversion of Series A, B-1, B-2 and C preferred stock into common stock with respect to the Reverse Merger	(14,774,552)	(147)	14,774,552	147	—	—	—	—	—	—
Shares issued with respect to the Reverse Merger	—	—	2,197,876	22	(192)	—	—	—	—	(170)
Provision related to the Technion exit fee	—	—	—	—	3,416	—	—	—	—	3,416
Net loss	—	—	—	—	—	—	—	—	(21,214)	(21,214)
Balance as of December 31, 2017	—	—	27,527,738	274	60,047	—	—	—	(38,960)	21,361
Exercise of stock options	—	—	1,334,522	14	432	—	—	—	—	446
Issuance of common stock upon Technion settlement	—	—	569,395	6	(6)	—	—	—	—	—
Issuance of shares upon execution of warrants	—	—	60,989	1	51	—	(3,385)	(52)	—	—
Issuance of shares upon public offering	—	—	5,899,500	60	53,513	—	—	—	—	53,573
Issuance of common stock from at-the-market sales agreement	—	—	201,100	2	2,421	—	—	—	—	2,423
Repurchase of common stock	—	—	(5,076)	—	—	—	(5,000)	(31)	—	(31)
Vesting of restricted stock units	—	—	271,946	3	(3)	—	(83,038)	(1,046)	—	(1,046)
Stock-based compensation expense	—	—	—	—	13,370	—	—	—	—	13,370
Net loss	—	—	—	—	—	—	—	—	(47,185)	(47,185)
Balance as of December 31, 2018	—	—	35,860,114	360	129,825	—	(91,423)	(1,129)	(86,145)	42,911
Exercise of stock options	—	—	116,327	1	168	—	—	—	—	169
Issuance of shares upon execution of warrants	—	—	44,814	1	178	—	(14,893)	(179)	—	—
Issuance of shares upon public offering	—	—	3,833,334	38	32,134	—	—	—	—	32,172
Issuance of common stock from at-the-market sales agreement	—	—	35,362	—	455	—	—	—	—	455
Issuance of warrants	—	—	—	—	421	—	—	—	—	421
Vesting of restricted stock units	—	—	140,812	2	(2)	—	(49,390)	(395)	—	(395)
Stock-based compensation expense	—	—	—	—	11,336	—	—	—	—	11,336
Unrealized gain (loss) on investments	—	—	—	—	—	18	—	—	—	18
Net loss	—	—	—	—	—	—	—	—	(50,874)	(50,874)
Balance as of December 31, 2019	—	\$ —	40,030,763	\$ 402	\$ 174,515	\$ 18	(155,706)	\$ (1,703)	\$ (137,019)	\$ 36,213

*See accompanying notes consolidated financial statements.*

**ELOXX PHARMACEUTICALS, INC. AND SUBSIDIARIES**

**CONSOLIDATED STATEMENTS OF CASH FLOWS**  
**(Amounts in thousands)**

	Year ended December 31,		
	2019	2018	2017
<b>Cash flows from operating activities:</b>			
Net loss	\$ (50,874)	\$ (47,185)	\$ (21,214)
Adjustments to reconcile net loss to net cash used in operating activities:			
Stock-based compensation	11,336	13,370	101
Depreciation	87	216	39
Loss (gain) on sales and disposals of property and equipment	(2)	12	—
Amortization of operating lease right-of-use asset	472	—	—
Amortization of debt discount	535	—	—
Amortization of premiums and discounts on investments	(301)	—	—
Amortization and revaluation of discount in respect to convertible loan	—	—	668
Provision related to the Technion exit fee	—	—	3,416
Change in operating assets and liabilities:			
Prepaid expenses and other current assets	83	(1,080)	709
Other assets	—	(41)	—
Accounts payable	1,124	(780)	(583)
Accrued expenses	(1,300)	3,999	929
Operating lease liabilities	(472)	—	—
Taxes payable	(79)	122	—
Net cash used in operating activities	<u>(39,391)</u>	<u>(31,367)</u>	<u>(15,935)</u>
<b>Cash flows from investing activities:</b>			
Purchases of marketable securities	(67,214)	—	—
Proceeds from maturity of marketable securities	33,750	—	—
Purchase of property and equipment	(40)	(235)	(237)
Proceeds from sales of property and equipment	2	6	—
Cash paid for long-term deposits	(22)	(88)	—
Cash received upon the Reverse Merger	—	—	123
Net cash used in investing activities	<u>(33,524)</u>	<u>(317)</u>	<u>(114)</u>
<b>Cash flows from financing activities:</b>			
Proceeds from underwritten public offerings, net of issuance costs	32,172	53,573	—
Proceeds from debt financing obligation	15,000	—	—
Payment of debt issuance costs	(276)	—	—
Proceeds from share-based compensation arrangements	169	446	17
Proceeds from sale of common stock under equity sales agreement	710	2,165	—
Payment for settlement of taxes upon vesting of restricted stock securities	(1,378)	—	—
Proceeds from issuance of Series C preferred stock	—	—	18,427
Proceeds from issuance of common stock	—	—	17,006
Proceeds from convertible loan and related financial derivative into Series C preferred stock	—	—	2,500
Proceeds from advances from collaboration partners	403	—	—
Net cash provided by financing activities	<u>46,800</u>	<u>56,184</u>	<u>37,950</u>
(Decrease) increase in cash, cash equivalents and restricted cash	(26,115)	24,500	21,901
Cash, cash equivalents and restricted cash at the beginning of the year	<u>48,651</u>	<u>24,151</u>	<u>2,250</u>
Cash, cash equivalents and restricted cash at the end of the year	<u>\$ 22,536</u>	<u>\$ 48,651</u>	<u>\$ 24,151</u>
Reconciliation of cash, cash equivalents and restricted cash to consolidated balance sheet:			
Cash and cash equivalents	\$ 22,493	\$ 48,606	\$ 24,049
Restricted cash	43	45	102
Total cash, cash equivalents and restricted cash	<u>\$ 22,536</u>	<u>\$ 48,651</u>	<u>\$ 24,151</u>
<b>Supplemental disclosure of cash flow activities:</b>			
Cash paid for interest	\$ 986	\$ —	\$ —
Cash paid for income taxes, net of refunds received	<u>\$ 36</u>	<u>\$ —</u>	<u>\$ —</u>
<b>Supplemental disclosure of non-cash financing activities:</b>			
Non-cash acquisition of treasury stock	\$ —	\$ 1,129	\$ —
Non-cash issuance of common stock upon exercise of warrants	<u>\$ 179</u>	<u>\$ —</u>	<u>\$ —</u>

Fair value of warrants issued in connection with long-term debt	\$ 421	\$ —	\$ —
Remeasurement of operating leases	\$ 176	\$ —	\$ —
Conversion of convertible loan into Series C preferred stock	\$ —	\$ —	\$ 3,168
Issuance expenses of Series C Preferred Shares	\$ —	\$ —	\$ 242
Proceeds receivable from equity sales agreement	\$ —	\$ 255	\$ —

*See accompanying notes consolidated financial statements*

**ELOXX PHARMACEUTICALS, INC. AND SUBSIDIARIES**  
**NOTES TO 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 mRNA stability and enabling functional protein synthesis. 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 and nephropathic cystinosis. ELX-02 is an investigational drug that has not been approved by any global regulatory body. In addition, during 2019 the Company announced a new program studying intravitreal administration of ERSG compounds for rare inherited retinal disorders (IRDs) with an initial focus on Usher Syndrome.

During the year, the Company advanced its clinical program for ELX-02 into Phase 2 studies in cystic fibrosis and nephropathic cystinosis. The Company 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 provide support for both continuing the Company’s clinical development programs and evaluating the suitability of its ERSG library for development in additional renal disorders, including autosomal dominant polycystic kidney disease and cystinuria. 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, MA, with additional offices in New Jersey, Pennsylvania 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 studies in both cystic fibrosis and nephropathic cystinosis.

During the third quarter of 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 and that it will form 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.

Eloxx Pharmaceuticals, Ltd. (“Eloxx Limited”) was incorporated in Israel on September 17, 2013 and acquired by the Company in a reverse merger described below. The Company focuses its activity on the discovery, development and commercialization of compounds for the treatment of genetic diseases caused by nonsense mutations primarily through a license agreement (the “Technion Agreement”) with the Technion Research and Development Foundation Ltd. (“TRDF”) entered into in 2013. For more information relating to the Technion Agreement, see Note 9, “Legal and Other Contingencies”.

### ***Reverse Merger***

On December 19, 2017, Sevion Therapeutics, Inc. (“Sevion”) acquired Eloxx Limited pursuant to a merger between the companies (the “Transaction” or “Reverse Merger”). Upon consummation of the Transaction (the “Closing”), Sevion adopted the business plan of Eloxx Limited and discontinued the pursuit of Sevion’s business plan. In connection with the Transaction, Sevion acquired all of the outstanding capital stock of Eloxx Limited in exchange for the issuance of an aggregate 20,316,656 shares of Sevion’s common stock, par value \$0.01 per share (the “Common Stock”), after giving effect to a 1-for-20 reverse split immediately prior to the Transaction. As a result of the Transaction, Eloxx Limited became a wholly-owned subsidiary of Sevion. While Sevion was the legal acquirer in the transaction, Eloxx Limited was deemed the accounting acquirer. Immediately after giving effect to the Transaction, on December 19, 2017, Sevion changed its name to Eloxx Pharmaceuticals, Inc.

The annual consolidated financial statements of the Company reflect the operations of the acquirer for accounting purposes, together with a deemed issuance of shares, equivalent to the shares held by the former stockholders of the legal acquirer and a recapitalization of the equity of the accounting acquirer. The annual consolidated financial statements include the accounts of the Company since the effective date of the reverse capitalization and the accounts of Eloxx Limited since inception.

### ***Liquidity***

The Company has a history of net losses and negative cash flows from operating activities since inception, and as of December 31, 2019, had an accumulated deficit of \$(137.0) 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 17, on February 24, 2020, the Company’ 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, cash equivalents and marketable securities of \$56.3 million at December 31, 2019 will enable it to meet anticipated cash needs required to maintain the Company’s current and planned operations through at least the next 12 months from the issuance of the financial statements for the year ended December 31, 2019. 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 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. Summary of Significant Accounting Policies**

### ***Basis of Presentation***

The Company has prepared its 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”).

### ***Principles of Consolidation***

The consolidated financial statements include the accounts of the Company and its subsidiaries. Intercompany balances and transactions have been eliminated upon consolidation.

### ***Use of Estimates***

The preparation of the consolidated financial statements in conformity with U.S. GAAP requires management to make estimates, judgments and assumptions. The Company's management believes that the estimates, judgments and assumptions used are reasonable based upon information available at the time they are made. These estimates, judgments and assumptions can affect the reported amounts of assets and liabilities, the disclosure of contingent assets and liabilities at the dates of the consolidated financial statements, and the reported amounts of expenses during the reporting period. The Company evaluates estimates on an ongoing basis in light of changes in circumstances, facts and experiences. Actual results could materially differ from those estimates.

### ***Foreign Currency***

The functional currency of the Company is the U.S. dollar. Accordingly, monetary accounts maintained in other currencies are re-measured into U.S. dollars in accordance with ASC Topic 830, "Foreign Currency Matters". All foreign currency transaction gains and losses arising from transactions denominated in foreign currencies, whether realized or unrealized, are recorded in the statements of operation as other income or expenses.

### ***Comprehensive Loss***

Comprehensive loss is defined as the change in equity of a business enterprise during a period from transactions and other events and circumstances from non-owner sources. Comprehensive loss consists of net loss and other comprehensive loss. Other comprehensive loss consists of unrealized gains and losses from available-for-sale securities.

### ***Cash and Cash Equivalents***

The Company considers all highly liquid investments purchased with an original maturity of three months or less to be cash equivalents. The Company's cash and cash equivalents include holdings in checking and overnight sweep accounts. The Company's cash equivalents, which are money market funds held in a sweep account, are measured at fair value on a recurring basis. As of December 31, 2019 and 2018 the balance of cash and cash equivalents was \$22.5 million and \$48.6 million, respectively, which approximates fair value and was determined based upon Level 1 inputs. The sweep account is valued using quoted market prices with no valuation adjustments applied. Accordingly, these securities are categorized as Level 1.

### ***Marketable Securities***

All investment instruments with an original maturity date, when purchased, in excess of three months but less than one year have been classified as current marketable securities. The Company classifies securities that are available to fund current operations as current assets. These marketable securities are classified as available-for-sale and are carried at fair value. The Company records unrealized gains and losses on available-for-sale debt securities as a component of accumulated other comprehensive income, which is a separate component of stockholders' equity on its consolidated balance sheet, until such gains and losses are realized. Realized gains and losses on available-for-sale securities are included in other income. The cost of securities sold is based on the specific identification method. The Company periodically reviews its portfolio of securities to determine whether an other-than-temporary impairment loss has occurred. No such losses have occurred to date. There were no realized gains or losses on sales of securities for the year ended December 31, 2019.

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

	Amortized Cost	Unrealized		Unrealized Losses	Fair Value
		Gains	—		
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	<u>\$ 56,258</u>	<u>\$ 19</u>	<u>—</u>	<u>\$ (1)</u>	<u>\$ 56,276</u>

### ***Restricted Cash***

At December 31, 2019, and 2018, restricted cash consisted of bank guarantees related to Eloxx Limited's corporate facilities lease and credit card program.

### ***Concentrations of Credit Risk and Off-Balance Sheet Risk***

Financial instruments that potentially subject the Company to credit risk consist primarily of cash and cash equivalents and marketable securities. The Company mitigates its risk with respect to cash and cash equivalents and marketable securities by maintaining its deposits and investments at high-quality financial institutions. The Company invests any excess cash in money market funds and U.S. treasuries, and the management of these investments is not discretionary on the part of the financial institution.

The Company has no off-balance-sheet concentration of credit risk such as foreign exchange contracts, option contracts or other foreign hedging arrangements.

### ***Property and Equipment***

Property and equipment are recorded at cost, less accumulated depreciation. Leasehold improvements are amortized over the lesser of the estimated useful life or the expected term of the related lease. Costs associated with maintenance and repairs are expensed as incurred. Depreciation expense is computed on a straight-line method over the estimated useful lives of the respective assets, as follows:

	<b>Useful Life (Years)</b>
Computers and software	3 years
Office furniture and equipment	5 to 12 years
Laboratory equipment	5 years
Leasehold improvement	Over the shorter of the expected lease term or estimated useful life

Upon sale or disposition of property and equipment, the cost and related accumulated depreciation are eliminated from the accounts and any resultant gain or loss is credited or charged to operations.

### ***Impairment of Long-Lived Assets***

Property and equipment subject to depreciation are reviewed for impairment in accordance with ASC Topic 360, "Accounting for the Impairment or Disposal of Long-Lived Assets," whenever events or changes in circumstances indicate that the carrying amount of an asset may not be recoverable. If such assets are considered to be impaired, the impairment to be recognized is measured as the amount by which the carrying amount of the assets exceeds their fair value. The Company continually evaluates whether events or circumstances have occurred that indicate that the remaining useful life of its long-lived assets may warrant revision or that the carrying value of these assets may be impaired. Factors that the Company considers in deciding when to perform an impairment review include significant underperformance of the business in relation to expectations, significant negative industry or economic trends, and significant changes or planned changes in the use of the assets.

Evaluation of recoverability of the asset or asset group is based on an estimate of undiscounted future cash flows resulting from the use of the asset or asset group and its eventual disposition. In the event that such cash flows are not expected to be sufficient to recover the carrying amount of the asset or asset group, the assets are written down to their estimated fair value. The impairment loss would be based on the excess of the carrying value of the impaired asset over its fair value, determined based on discounted cash flows. As of each balance sheet date presented, none of the Company's long-lived assets were impaired. The Company has not recorded any impairment losses to date.

### ***Legal and Other Contingencies***

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 years ended December 31, 2019, 2018 and 2017 the Company was not a party to any litigation that could have a material adverse effect on the Company's business, financial position, results of operations or cash flows (see also Note 9, "Legal and Other Contingencies"). Legal costs incurred in connection with loss contingencies are expensed as incurred.

### ***Research and Development Expenses***

Research and development expenses are comprised of costs incurred in performing research and development activities, including salaries, stock-based compensation and benefits for employees performing such activities, certain facilities costs, depreciation, third-party license fees, and external costs of vendors engaged to conduct preclinical development activities and clinical trials. Research and development expenses are expensed as incurred and include the Company's costs of performing services in connection with its collaboration agreements and research grants.

Non-refundable prepayments for goods or services that will be used or rendered for future research and development activities are deferred and capitalized in prepaid expenses and other current assets. Such amounts are recognized as an expense as the goods are delivered or the related services are performed, or until it is no longer expected that the goods will be delivered, or the services rendered.

The Company enters into arrangements with contract research organizations in connection with clinical trials. Such arrangements often provide for payment prior to commencing the project or based upon predetermined milestones throughout the period during which services are expected to be performed. As part of the process of preparing the Company's financial statements, management is required to estimate accrued expenses. The date on which services commence, the level of services performed on or before a given date, and the cost of such services are often determined based on subjective judgments informed by the facts and circumstances known to management from the terms of the contract and the Company's ongoing monitoring of service performance. The Company makes these judgments based upon the facts and circumstances known to management based on the terms of the contract and the Company's ongoing monitoring of service performance.

### ***Government Grants***

Eloxx Limited has received royalty-bearing grants for the years ended December 31, 2015, 2016 and 2017 totaling \$2.6 million, which represents participation in approved research and development programs of the Israeli Innovation Authority ("IIA", previously known as the Office of the Chief Scientist of the Ministry of Economy). These amounts are recognized on the accrual basis as a reduction of research and development expenses as such expenses are incurred.

### ***Fair Value of Financial Instruments***

Fair value is determined based on the exchange price that would be received for an asset or paid to transfer a liability (an exit price) in the principal market for the asset or liability in an orderly transaction between market participants. U.S. GAAP specifies a hierarchy of valuation techniques based upon whether the inputs to those valuation techniques reflect assumptions other market participants would use based upon market data obtained from independent sources (observable inputs) or reflect the Company's own assumptions of market participant valuation (unobservable inputs).

The fair value hierarchy consists of three levels:

- Level 1 - Quoted prices (unadjusted) in active markets that are unadjusted and accessible at the measurement date for identical, unrestricted assets or liabilities.
- Level 2 - Observable inputs other than Level 1 prices, such as quoted prices for similar assets or liabilities, quoted prices in markets that are not active, or inputs that are observable or can be corroborated by observable market data for substantially the full term of the assets or liabilities.
- Level 3 - Unobservable inputs that are supported by little or no market activity and that are significant to the fair value of the assets or liabilities.

Financial assets and liabilities are classified within the fair value hierarchy based on the lowest level of input that is significant to the fair value measurement. The authoritative guidance requires the use of observable market data if such data is available without undue cost and effort. When available, the Company uses unadjusted quoted market prices to measure fair value and classify such items within Level 1. If quoted market prices are not available, fair value is based upon internally developed models that use, where possible, current market-based or independently-sourced market parameters, such as interest and currency rates and comparable transactions. Items valued using internally generated models are classified according to the lowest level input or value driver that is significant to the valuation. Thus, items may be classified in Level 3 even though there may be inputs that are readily observable. If quoted market prices are not available, the valuation model used generally depends on the specific asset or liability being valued. At December 31, 2019 and 2018, the Company's financial assets valued based on Level 1 inputs consisted of cash, cash equivalents and marketable securities (U.S. treasuries). The Company did not have any transfers of financial assets between Level 2 and 3 during 2019.

The carrying amounts of cash and cash equivalents, restricted bank deposits, prepaids and other current assets, accounts payables and accrued expenses approximate their fair value due to the short-term maturities of such instruments.

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.

### ***Stock-Based Compensation***

The Company accounts for stock-based compensation in accordance with ASC Topic 718, "Compensation-Stock Compensation" ("ASC 718"), which requires companies to estimate the fair value of equity-based payment awards on the date of grant using an option-pricing model. The Company recognizes compensation expenses for the value of its awards granted based on the straight-line method over the requisite service period of each of the awards or over the implicit service period when a performance condition affects the vesting, and it is considered probable that the performance condition will be achieved.

The Company determines the fair value of each stock option award at its grant date using the Black-Scholes option pricing model for options awarded in 2019, and using the binomial lattice-based option valuation model for options awarded in 2018 and 2017. The Company determines the fair value of each restricted stock unit, or RSU, at its grant date based on the closing market price of the Company's common stock on that date.

### ***Income Taxes***

The Company accounts for income taxes in accordance with ASC Topic 740, "Income Taxes" ("ASC 740"), which prescribes the use of the asset and liability method whereby deferred tax assets and liability account balances are determined based on differences between financial reporting and tax bases of assets and liabilities and are measured using the enacted tax rates and laws that will be in effect when the differences are expected to be recovered or settled. The Company provides a valuation allowance, if necessary, to reduce deferred tax assets to their estimated realizable value if it is more likely than not that a portion or all of the deferred tax assets will not be realized. The Company considers many factors when assessing the likelihood of future realization of deferred tax assets, including recent earnings results, expectations of future taxable income, carryforward periods available and other relevant factors. The Company records changes in the required valuation allowance in the period that the determination is made.

Based on ASC 740, a two-step approach is used to recognize and measure uncertain tax positions. The first step is to evaluate the tax position taken or expected to be taken in a tax return by determining if the weight of available evidence indicates that it is more likely than not that, on an evaluation of the technical merits, the tax position will be sustained on audit, including resolution of any related appeals or litigation processes. The second step is to measure the tax benefit as the largest amount that is more than 50% likely to be realized upon ultimate settlement. As of December 31, 2019 and 2018, no liability for unrecognized tax positions has been recorded. Accordingly, no interest or penalties related to uncertain tax positions are recorded, either. The Company's policy is to record any interest or penalties associated with unrecognized tax positions as a component of income tax expense.

#### ***Net Loss per Share***

For the year ended December 31, 2017, the Company applied the two-class method as required by ASC Topic 260-10, "Earnings Per Share" ("ASC 260-10"), which requires the income or loss per share for each class of shares (ordinary and preferred shares) to be calculated assuming 100% of the Company's earnings are distributed as dividends to each class of shares based on their contractual rights. For the years ended December 31, 2019 and 2018, the Company had no outstanding preferred shares and was not required to apply the two-class method under ASC 260-10.

No dividends were declared or paid during the reported periods. According to the provisions of ASC 260-10, the Company's preferred shares are not participating securities in losses and, therefore, are not included in the computation of net loss per share.

Basic loss per share is computed by dividing the loss for the period applicable to ordinary shareholders by the weighted average number of ordinary shares outstanding during the period. In computing diluted income per share, weighted average shares used in computing basic earnings per share are adjusted to reflect the potential dilution that could occur upon the exercise of outstanding stock options and upon conversion of restricted stock units and warrants issued to investors and service providers using the "treasury stock method".

#### ***Recent Accounting Pronouncements - Adopted***

In February 2016, the FASB issued ASU 2016-02, Leases ("ASU 2016-02"). ASU 2016-02 establishes ASC Topic 842 ("Topic 842") which replaces ASC 840, Leases, by introducing a lessee model that requires balance sheet recognition for most leases and the disclosure of key information about leasing arrangements. Leases will be classified as finance or operating, with classification affecting the pattern and classification of expense recognition in the income statement. Topic 842 provides several optional practical expedients in transition. The Company elected the package of practical expedients which allowed the Company to not reassess its existing conclusions on lease identification, classification and initial direct costs. Further, the Company elected the hindsight practical expedient and utilized the short-term lease exemption for all leases with an original term of 12 months or less for purposes of applying the recognition and measurement requirements of the new standard. The Company also elected the practical expedient which allowed it to not separate lease and non-lease components for all its leases. The Company adopted the new standard on January 1, 2019 and applied the effective date as its date of initial application and has not updated disclosures required under the new standard for dates and periods prior to January 1, 2019. Also see Note 6, "Leases".

#### ***Recent Accounting Pronouncements - Pending Adoption***

In June 2016, the FASB issued ASU 2016-13, Measurement of Credit Losses on Financial Instruments ("ASU 2016-13"). 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. This standard is effective for annual periods beginning after December 15, 2019 and interim periods within those annual periods, with early adoption permitted for annual periods beginning after December 15, 2018. The Company is assessing the impact the adoption of ASU 2016-13 may have on its consolidated financial statements.

### **3. Prepaid Expenses and Other Current Assets**

Prepaid expenses and other current assets consisted of the following (in thousands):

	<u>December 31,</u>	
	<u>2019</u>	<u>2018</u>
Research and development	\$ 448	\$ 864
Other	699	555
Insurance	217	251
Other governmental agency receivables	26	20
	<u>\$ 1,390</u>	<u>\$ 1,690</u>

### **4. Property and Equipment, net**

Property and equipment, net consisted of the following (in thousands):

	<u>December 31,</u>	
	<u>2019</u>	<u>2018</u>
Computers and software	\$ 166	\$ 146
Office furniture and equipment	164	165
Leasehold improvements	158	141
	<u>488</u>	<u>452</u>
Less: Accumulated depreciation	(287)	(204)
Property and equipment, net	<u>\$ 201</u>	<u>\$ 248</u>

Depreciation expense was \$87 thousand, \$216 thousand and \$39 thousand for the years ended December 31, 2019, 2018 and 2017, respectively.

### **5. Accrued Expenses**

Accrued expenses consisted of the following (in thousands):

	<u>December 31,</u>	
	<u>2019</u>	<u>2018</u>
Research and development expenses	\$ 1,560	\$ 3,086
Payroll, bonus and other employee-related expenses	2,200	2,562
Professional services	664	985
Other	137	305
Interest on debt	94	—
	<u>\$ 4,655</u>	<u>\$ 6,938</u>

## **6. Leases**

The Company adopted the new lease accounting standard, Topic 842, effective January 1, 2019. The Company has elected the ‘package of practical expedients’, which permits it not to reassess under the new standard its prior conclusions about lease identification, lease classification and initial direct costs. In addition, the Company elected not to apply Topic 842 to arrangements with lease terms of 12 months or less. The Company has operating leases for its principal offices in the U.S. and Israel.

Operating lease assets represent the Company’s right to use an underlying asset for the lease term and operating lease liabilities represent the Company’s obligation to make lease payments arising from its operating leases. In determining the length of the lease term to its long-term lease, the Company determined not to consider an embedded renewal option for one operating lease primarily due to the facts that (i) the renewal rate is at a future market rate to be determined, and (ii) the Company does not have significant leasehold improvements that would restrict its ability to consider relocation. The Company applied its incremental borrowing rate based upon information available in determining the present value of the lease payments. The Company’s incremental borrowing rate is determined using a secured borrowing rate for the same currency and term as the associated lease.

Operating lease costs under the leases for the year ended December 31, 2019 were approximately \$0.5 million. Lease expense for these leases is recognized on a straight-line basis over the lease term, with variable lease payments recognized in the period those payments are incurred. At lease commencement date, the Company estimated the lease liabilities and the right-of-use assets at present value using the Company’s estimated incremental borrowing rate of 8% and determined the initial present value, at inception, of \$1.5 million. On January 1, 2019, upon adoption of Topic 842, the Company recorded right-of-use assets of \$1.2 million and lease liabilities of \$1.2 million. These assets and obligations are reflected within ‘Operating lease right-of-use asset’, and ‘Operating lease liability’ (current and noncurrent), respectively, on the Consolidated Balance Sheet. The weighted average remaining lease term at December 31, 2019 was 1.81 years. The Company made payments of \$0.5 million for the year ended December 31, 2019, and recorded a \$0.2 million remeasurement, which increased the respective assets and obligations balances.

The following table summarizes the Company’s maturities of operating lease liabilities as of December 31, 2019 (in thousands):

2020	\$	556
2021		404
2022		32
Total lease payments		992
Less: present value discount		(68)
Total	\$	924

The Company’s aggregate future minimum non-cancellable commitments under operating leases as of December 31, 2018 were as follows (in thousands):

2019	\$	541
2020		490
2021		310
Total minimum lease payments	\$	1,341

## **7. Debt**

On January 30, 2019, the Company entered into a Loan and Security Agreement (the “SVB Loan Agreement”) with Silicon Valley Bank (“SVB”) and WestRiver Innovation Lending Fund VIII, L.P. (“WestRiver”, and together with SVB, the “Lenders”). Under the SVB Loan Agreement, the Lenders agreed to extend term loans to the Company in an aggregate principal amount of \$25.0 million, comprised of (i) an initial loan advance of \$15.0

million; and (ii) a subsequent loan advance of \$10.0 million, subject to first achieving certain conditions (collectively, the “Term Loan Advances”). The initial term loan was funded on January 30, 2019. The subsequent term loan advance was available until December 31, 2019 at the Company’s election after the occurrence of certain milestone events relating to data from the Company’s clinical trials and receipt by the Company of certain minimum cash proceeds of at least \$75 million from an additional equity offering through a private placement or a public offering. These events did not occur, and thus the Company did not draw the second advance.

Outstanding principal on the Term Loan Advances 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 a Term Loan advance. On December 31, 2019, the rate was 7.25%. The Company will be required to make principal payments on the outstanding balance of the Term Loan Advances commencing on February 1, 2020 in 36 equal monthly installments, plus interest. Any amounts outstanding under the Term Loan Advances, if not repaid sooner, are due and payable on January 1, 2023 (the “Maturity Date”).

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 term 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 term loans advanced, due upon the earliest 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 December 31, 2019, the carrying value of the term loan consists of \$15.0 million principal outstanding less the unamortized debt issuance costs of approximately \$1.1 million. The debt issuance costs have been recorded as a debt discount and are being accreted to interest expense through the maturity date of the term loan. Interest expense relating to the term loan for the period ended December 31, 2019 was \$1.6 million. Interest expense is calculated using the effective interest method and is inclusive of non-cash amortization of capitalized loan costs. At December 31, 2019, the effective interest rate was 12.40%. The final maturity payment of \$0.9 million is recognized over the life of the term loan through interest expense using the effective interest method.

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

	December 31, 2019
2020	\$ 4,583
2021	5,000
2022	5,000
2023	417
Total future principal payments	<u>15,000</u>
Less: unamortized discount	(1,062)
Carrying value of long-term debt	13,938
Less: current portion	(4,336)
Add: final fee due at maturity in 2023	900
Long-term portion	<u><u>\$ 10,502</u></u>

## **8. Related Parties**

On August 29, 2013, the Company entered into the Technion Agreement with TRDF, with respect to certain technology relating to aminoglycosides and the redesign of aminoglycosides for the treatment of human genetic diseases caused by premature stop mutations and further results of the research of the technology, in order to develop and commercialize products based on such technology. Under the Technion Agreement, TRDF is obligated to provide the Company with research services for an estimated annual payment of \$0.1 million, the precise amount to be agreed by the parties prior to the beginning of each year of the research period. During the year ended December 31, 2017, the Company recorded general and administrative expenses of \$7 thousand in relation to the Technion Agreement. No such expenses were incurred during the years ended December 31, 2019 and 2018. In addition, during the years ended December 31, 2019, 2018 and 2017, the Company recorded research and development expenses of \$0.2 million, \$0.1 million and \$3.5 million, respectively, in relation to the Technion Agreement for the reimbursement of costs incurred during the preparation, filing, prosecution and maintenance of the TRDF patents rights related to Eloxx Limited. As of December 31, 2019 and 2018, amounts recorded in accrued expenses were \$142 thousand and \$25 thousand, respectively.

In addition, TRDF granted the Company a license to use, market, sell or sub-license the rights of the product developed under the TRDF research results (the “Licensed Product”), as fully defined in the Technion Agreement, for the following considerations: (i) milestone payments up to total consideration of \$6.1 million, to be transferred upon meeting certain milestones as defined in the Technion Agreement; (ii) certain royalties in the low- to mid-single-digit percentage of net sales (subject to change in the case of (a) sublicensing to a big pharmaceutical or biotechnology company, or (b) payment of royalties to third parties, or (c) commercialization by a third party of an authorized generic to a licensed product), for a period until the later of (i) the expiration of a valid claim on the Licensed Product in each country the Licensed Product is sold to, or (ii) a certain amount of years from the date of the first commercial sale of the Licensed Product in such country; and (iii) a low- to mid-double-digit percentage of any non-royalty sub-license income received by the Company from a sub-licensed entity. In addition, the Company shall pay certain fee to TRDF upon an exit event as described in the Technion Agreement.

Moreover, upon the closing of an exit event which is not an Initial Public Offering (“IPO”), as defined in the Technion Agreement, TRDF shall be entitled to an amount equal to 3% of all non-refundable, non-contingent consideration, whether in cash or in kind, actually received by the Company and/or its shareholders. Upon the closing of an exit event which is an IPO, as defined in the Technion Agreement, TRDF shall be entitled to a number of Ordinary Shares of the Company representing 3% of the Company’s outstanding shares on a fully diluted basis immediately prior to the closing of such IPO.

On August 9, 2017 the Company received a letter from TRDF regarding TRDF’s alleged entitlement to an exit fee in accordance with the Technion Agreement. The Company recorded \$3.4 million in research and development expense with an offsetting adjustment to additional paid-in capital for the year ended December 31, 2017 related to the planned issuance of shares to TRDF at fair market value on the purported date of the exit event. On June 13, 2018, the Company entered into an amendment to the license agreement providing that in exchange for the issuance by the Company to TRDF of 569,395 shares, the Company and TRDF agreed to terminate (i) TRDF’s rights to receive payments from the Company upon the Company’s consummation of certain business transactions such as a merger or an initial public offering, (ii) certain preemptive rights provided by the Articles of Association of Eloxx Limited, and (iii) TRDF’s right to designate an observer to the Company’s board of directors. On June 30, 2018, the Company issued 569,395 shares to TRDF in satisfaction of this claim.

## **9. Commitments and Contingencies**

### ***Royalty Commitments to the IIA***

To date, the Company has received research and development grants from the IIA totaling \$2.6 million, including \$0.9 million for the year ended December 31, 2017. No grants were received for the years ended December 31, 2019 and 2018.

Under the research and development agreements with the IIA and pursuant to applicable law, the Company is required to pay royalties at the rate of 3% on sales to end customers of products candidates developed with funds provided by the IIA, up to an amount equal to 100% of the IIA research and development grants received, plus

interest based on the 12-month LIBOR rate. If the Company does not generate sales of products developed with funds provided by the IIA, the Company is not obligated to pay royalties or repay the grants.

As of December 31, 2019, the Company has not commenced the payment obligation of the royalties and has a contingent obligation with respect to royalty-bearing participation received or accrued, amounting to \$2.7 million, including accrued LIBOR interest.

#### ***Commitments to TRDF***

Since August 29, 2013, the Company has had an ongoing agreement with TRDF. Refer to Note 8, “Related Parties”, for further information.

#### ***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, 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.

The Company accounts for 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. As of December 31, 2019 and 2018, 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.

During 2019, the Company received a funding award from the Cystic Fibrosis Foundation and entered into an agreement relating to the award and provision of other services. Payment of award amounts are subject to the achievement of certain milestones in connection with the Company’s cystic fibrosis development program in the U.S. The funding provided to the Company is accounted for as an advance from a collaboration partner within the scope of ASC Topic 730, “Research and Development.” The Company recorded a liability for advances from collaboration partners of \$0.4 million as of December 31, 2019.

#### **10. Stockholders’ Equity**

For accounting purposes, all common stock, preferred stock, warrants, options to purchase common stock and loss per share amounts have been adjusted to give retroactive effect to the exchange ratio and reverse stock split for all periods presented in these consolidated financial statements.

As of December 31, 2019, the Company had 500,000,000 shares authorized of common stock, \$0.01 par value, of which 40,030,763 shares were outstanding and 5,000,000 shares authorized of preferred stock, \$0.01 par value, of which no shares were issued or outstanding.

#### ***Common Stock***

##### ***Public Offering***

On April 30, 2018, the Company closed an underwritten public offering of 5,899,500 shares of its common stock, including the exercise in full by the underwriter of its overallotment option to purchase an additional 769,500 shares, at the public offering price of \$9.75 per share for gross proceeds of approximately \$57.5 million, before deducting the underwriting discounts and commissions and offering expenses of approximately \$3.9 million. The shares of common stock were offered by the Company pursuant to a shelf registration statement on Form S-3 (File

No. 333-224207) that was filed with the U.S. Securities and Exchange Commission (the “SEC”) on April 10, 2018 and declared effective on April 20, 2018, which covers the offering, issuance and sale of up to \$125 million of its common stock, preferred stock, debt securities or warrants and other securities, either individually or in combination (the “April 2018 Shelf”).

On June 24, 2019, the Company completed an underwritten public offering of 3,833,334 shares of common stock of the Company at the public offering price of \$9.00 per share and received net proceeds of approximately \$32.2 million after deducting underwriting discounts and commissions of \$2.1 million and estimated offering expenses of \$0.2 million.

#### *Form S-3 and Equity Sales Agreement*

In November 2018, the Company entered into an Equity Distribution Agreement (the “Agreement”) with Citigroup Global Markets Inc. and Cantor Fitzgerald & Co. (collectively, the “Sales Agents”), pursuant to which the Company may sell and issue shares of its common stock up to an aggregate of \$50 million through the Sales Agents. The shares were offered pursuant to the April 2018 Shelf. The Company agreed to pay the Sales Agents a commission of up to 3% of the gross proceeds of any sales of common stock pursuant to the Agreement. The Company incurred approximately \$0.3 million related to legal, accounting and other fees in connection with the Agreement. For the year ended December 31, 2018, under the Agreement, the Company sold 201,100 shares of common stock and received net proceeds of \$2.2 million. In January 2019, the Company sold 35,362 shares of common stock and received net proceeds of \$0.7 million.

On November 16, 2018, the Company filed a shelf registration statement (the “November 2018 Shelf”) on Form S-3 with the SEC. The November 2018 Shelf (File No. 333-228430) was declared effective on November 26, 2018 and covers the offering, issuance and sale of up to \$200 million of the Company’s common stock, preferred stock, debt securities or warrants and other securities, either individually or in combination.

#### ***Preferred and Common Stock***

In February 2016, Eloxx Limited issued shares of preferred stock to purchase shares of preferred stock for an aggregate gross amount of \$6.0 million.

In August 2016, Technion Investment Opportunities Fund L.P (the “TIOF”) and TRDF exercised 124,786 and 311,964 warrants, respectively, to purchase shares of preferred stock for total consideration of \$0.4 million.

In September 2016, Eloxx Limited achieved a milestone in connection with the prior issuance of securities, pursuant to which Eloxx Limited paid a \$0.1 million milestone payment and issued to investors additional shares of preferred stock and warrants to purchase preferred stock for an aggregate amount of \$3.7 million.

In May 2017, Eloxx Limited entered into a Share Purchase Agreement (the “2017 SPA”) with certain existing and new investors, whereby an aggregate gross amount of \$21.5 million, which included the conversion of the loan (as detailed in Note 16), was received by Eloxx Limited in exchange for the issuance of preferred stock including shares of preferred stock that were issued as a result of the anti-dilution effect of the Reverse Merger. The related issuance costs recorded in 2017 were \$0.6 million.

Upon the closing of the Reverse Merger in December 2017, the Company issued 6,333,333 shares of common stock related to the 2017 SPA for an aggregate gross amount of \$17.5 million. Additionally, Sevion raised \$1.5 million prior to the Reverse Merger. The related issuance costs for these transactions recorded in 2017 were \$0.5 million.

### ***Warrants***

Eloxx Limited issued warrants to purchase shares in conjunction with the Share Purchase Agreements prior to the Transaction. During the year ended December 31, 2019, transactions related to warrants were as follows:

	Shares	Weighted average exercise price	Weighted average remaining contractual life (years)
Warrants outstanding at December 31, 2018	347,241	\$ 3.77	3.92
Granted	40,834		
Exercised	(59,707)		
Forfeited	(4,474)		
Warrants outstanding at December 31, 2019	<u>323,894</u>	<u>\$ 4.32</u>	<u>3.74</u>
Warrants exercisable at December 31, 2019	<u>323,894</u>	<u>\$ 4.32</u>	<u>3.74</u>

## **11. Stock-Based Compensation**

### ***Stock Incentive Plans***

Prior to April 20, 2018, the Company had two equity compensation plans which provided that options to purchase ordinary shares of Eloxx Limited, or ordinary shares of Eloxx Limited, may be granted to employees, officers, directors, service providers and consultants of Eloxx Limited: the Sevion 2008 Incentive Plan (the “2008 Plan”) and the Eloxx Limited 2013 Share Ownership and Option Plan (the “2013 Plan”).

On March 12, 2018, the Company’s Board of Directors (the “Board”) adopted the 2018 Equity Incentive Plan (the “2018 Plan”). The 2018 Plan became effective on April 20, 2018 upon approval by the stockholders of the Company with the outstanding options and shares available for future grant under the 2008 Plan and the 2013 Plan being assumed by the 2018 Plan. The initial total number of shares available under the 2018 Plan for awards to employees, non-employee directors and other key personnel was 5,000,000 shares. Upon the 2018 Plan becoming effective, the Company ceased granting awards under each of the 2008 Plan and the 2013 Plan.

Stock options granted have a ten-year contractual life and, upon termination of service, vested options are generally exercisable between one and three months following the termination date, while unvested options are forfeited immediately.

In 2017, the Company issued an inducement award outside of its stock plans to its Chief Executive Officer in the form of an option to purchase 22,427 shares of the Company’s common stock with an exercise price per share equal to \$8.00, and an award of restricted stock units for 22,427 shares of the Company’s common stock (collectively, the “Performance Awards”). Subject to continued service through the vesting date, the Performance Awards will vest and become immediately exercisable upon the date that marks the first successful completion of a Phase 2B study with respect to any indication. The Company recognized \$0.1 million of expense associated with these awards during each of the years ended December 31, 2019 and 2018.

In addition, the Company issued an inducement award outside of its stock plans to its Chief Executive Officer in the form of an option to purchase 640,785 shares of the Company's common stock with an exercise price per share equal to \$8.00, and an award of restricted stock units for 640,785 shares of the Company's common stock (collectively, the "Time-Vesting Awards"). Subject to continued service through the vesting date, one-third of the Time-Vesting Awards will vest and become immediately exercisable on the first anniversary of the effective date, with an additional one-twelfth of the Time-Vesting Awards vesting on each quarterly anniversary of the effective date, provided that vesting of the Time-Vesting Awards shall be subject to acceleration following the achievement of certain milestones.

#### ***Summary of Stock Option Activity***

Transactions related to stock options granted to employees and directors during the year ended December 31, 2019, were as follows:

	<u>Amount</u>	<u>Weighted average exercise price</u>	<u>Weighted average remaining contractual life (years)</u>	<u>Aggregate intrinsic value</u>
Options outstanding as of December 31, 2018	3,261,719	\$ 13.09	8.56	\$ 11,650,373
Granted	1,842,990	9.31		
Exercised	(116,327)	4.27		
Forfeited	(250,712)	10.35		
Options outstanding as of December 31, 2019	<u>4,737,670</u>	<u>\$ 11.17</u>	<u>8.54</u>	<u>\$ 3,629,073</u>
Options exercisable at December 31, 2019	<u>1,785,477</u>	<u>\$ 11.78</u>	<u>7.55</u>	<u>\$ 2,498,968</u>

As of December 31, 2019, the unrecognized compensation cost related to the outstanding options was \$18.4 million and is expected to be recognized over a weighted-average period of 2.68 years.

The weighted average grant date fair values of stock options granted during the years ended December 31, 2019, 2018, and 2017 were \$6.28, \$18.06 and \$3.68 per share respectively.

The following table presents the assumptions used to estimate the fair values of stock options granted in the periods presented:

	<u>Year ended December 31,</u>		
	<u>2019</u>	<u>2018</u>	<u>2017</u>
Dividend yield	0%	0%	0%
Volatility	76%-92%	90%-92%	87%-117%
Risk-free interest rate	1.51%-2.62%	2.55%-3.20%	1.22%-2.50%
Expected term (years)	5.5-6.0	—	—
Contractual term (years)	10	10	10
Forfeiture rate post-vesting	—	10%	10%
Suboptimal exercise	—	2.3	2.3

### **Summary of Restricted Stock Unit Activity**

Transactions related to restricted stock units granted to employees during the year ended December 31, 2019, were as follows:

	Shares	Weighted average grant date fair value per share
Unvested at December 31, 2018	554,147	\$ 9.34
Granted	100,000	11.53
Vested	(190,202)	8.09
Forfeited	—	—
Unvested at December 31, 2019	<u>463,945</u>	<u>\$ 9.45</u>

As of December 31, 2019, the unrecognized compensation cost related to the outstanding options was \$4.0 million and is expected to be recognized over a weighted-average period of 2.27 years.

### **Stock-based Compensation Expense**

Stock-based compensation expense 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. The total equity-based compensation expense related to all of the Company's equity-based awards was recognized as follows (in thousands):

	Year ended December 31,		
	2019	2018	2017
Research and development	\$ 2,458	\$ 1,745	\$ 39
General and administrative	8,878	11,625	62
Total stock-based compensation expense	<u>\$ 11,336</u>	<u>\$ 13,370</u>	<u>\$ 101</u>

### **12. Income Taxes**

The components of income (loss) before taxes on income are as follows (in thousands):

	Year ended December 31,		
	2019	2018	2017
U.S.	\$ (22,242)	\$ (24,439)	\$ (21)
Israel	(28,632)	(22,624)	(21,145)
Loss before taxes on income	<u>\$ (50,874)</u>	<u>\$ (47,063)</u>	<u>\$ (21,166)</u>

There were no taxes on income during the year ended December 31, 2019. Taxes on income during the year ended December 31, 2018 resulted primarily from subsidiary income as a result of the implementation of an intercompany cost-plus arrangement. The current income tax provision consisted of the following (in thousands):

	Year ended December 31,		
	2019	2018	2017
Federal	\$ —	\$ 94	\$ —
State and local	—	28	—
Foreign	—	—	—
Income tax provision	<u>\$ —</u>	<u>\$ 122</u>	<u>\$ —</u>

The significant components of the Company's deferred tax assets were comprised of the following (in thousands):

	December 31,	
	2019	2018
Deferred tax assets:		
Net operating loss carryforwards	\$ 38,357	\$ 29,521
Stock-based compensation	2,801	1,001
Reserves and allowances	615	377
U.S. tax credits and other credits	2,980	714
Research and development credits	1,831	4,216
Operating lease right-of-use assets	(169)	—
Operating lease liabilities	169	—
Other	98	40
Total deferred tax assets	<u>46,682</u>	<u>35,869</u>
Valuation allowance	(46,682)	(35,869)
Net deferred tax assets	<u>\$ —</u>	<u>\$ —</u>

Deferred taxes are recognized for temporary differences between the basis of assets and liabilities for financial statements and income tax purposes. The Company has evaluated the positive and negative evidence bearing upon the realizability of its deferred tax assets. As of December 31, 2019 and 2018, based on the Company's history of operating losses, the Company has concluded that it is not more likely than not that the benefit of the deferred tax assets will not be realized. Accordingly, the Company has provided a full valuation allowance for deferred tax assets as of December 31, 2019 and 2018.

As of December 31, 2019 and 2018, the Company provided valuation allowances of approximately \$46.7 million and \$35.9 million, respectively, on U.S. federal, U.S. state and Israeli tax jurisdiction deferred tax assets to reduce the carrying amounts of these assets to zero. The net changes in the Company's valuation allowances were increases of \$10.8 million and \$7.7 million during the years ended December 31, 2019 and 2018, respectively. For the year ended December 31, 2019, the increase in the valuation allowance was primarily related to losses generated during the period and an increase in the stock-based compensation deferred tax asset. For the year ended December 31, 2018, the increase in the valuation allowance was primarily related to losses generated during the period, partially offset by a reduction in the stock-based compensation deferred tax asset associated with cancelled awards.

For the year ended December 31, 2019, the expected tax expense based on the federal statutory rate reconciled with the actual tax expense as follows:

	Year ended December 31,	
	2019	2018
U.S. federal statutory rate	21.0%	21.0%
State tax rate, net of federal benefit	0.9	2.4
Permanent differences	(1.5)	(3.0)
Adjustments to deferred taxes	—	(1.8)
Effect of rate differences from statutory	1.1	0.8
Tax reform - federal tax rate change	—	0.1
Tax reform - change in valuation allowance	—	(0.1)
Change in valuation allowance	(21.5)	(19.5)
Other	—	(0.2)
Income tax provision	0.0%	-0.3%

The main reconciling item between the statutory tax rate and the Company's effective tax rate is the recognition of valuation allowances in respect to deferred taxes related to accumulated net operating losses carried forward and temporary differences due to the uncertainty of the realization of such deferred taxes.

For the years ended December 31, 2019 and 2018, the Company had U.S. federal net operating loss (“NOL”) carryforwards of \$99.4 million and \$89.8 million. U.S. federal NOL carryforwards will begin to expire, if not utilized, beginning in 2020 through 2037. Included in the U.S. federal NOL carryforward are \$23.5 million of NOLs generated after the effective date of the Tax Cuts and Jobs Act of 2017 (the “Tax Act”), which are not subject to expiration 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 Tax Act.

For the years ended December 31, 2019 and 2018, the Company had U.S. state NOL carryforwards of \$31.9 million and \$40.0 million, respectively, which may be available to offset future income tax liabilities.

As of December 31, 2019 and 2018, the Company had federal research tax credit carryforwards of \$3.0 million and \$0.7 million, respectively, available to reduce future tax liabilities and which expire at dates beginning in 2027 through 2037.

As of December 31, 2019 and 2018, the Company had Israeli NOL carryforwards of \$73.7 million, and \$34.6 million, respectively, which carry forward indefinitely.

The enactment of the Tax Act in December 2017, as further discussed below, resulted in the remeasurement of the Company’s net deferred tax assets due to the reduction in the corporate statutory rate from 35% to 21%. The effect on deferred tax assets and liabilities of a change in law or tax rates is recognized in income in the period that includes the enactment date. The Tax Act also includes a provision designed to currently tax global intangible low-taxed income (“GILTI”). The Company will record the U.S. income tax effect of future GILTI inclusions in the period in which they arise, if ever. After the enactment of the Tax Act, the SEC issued Staff Accounting Bulletin No. 118 (“SAB 118”) which provided guidance on accounting for the enactment effect of the Tax Act. SAB 118 addressed the application of U.S. GAAP in situations when a registrant does not have the necessary information available, prepared, or analyzed (including computations) in reasonable detail to complete the accounting for certain income tax effects of the Tax Act. SAB 118 provided for a measurement period of up to one year from the Tax Act enactment date for companies to complete their accounting under ASC 740. The Company had calculated a provisional estimate of deferred tax expense of \$10.2 million related to the remeasurement of its U.S. deferred tax assets in the future, which was fully and equally offset by a corresponding reduction in the valuation allowance. During the quarter ended December 31, 2018, the Company completed the accounting for the income tax effects of the Tax Act, which resulted in an immaterial change in the net deferred tax asset, before valuation allowance, as of the enactment date.

Under the provisions of the Internal Revenue Code (“IRC”), the net operating loss and tax credit carryforwards are subject to review and potential adjustments by the Internal Revenue Service and state tax authorities. Under Section 382 of the Internal Revenue Code and corresponding provisions of state law, if a corporation undergoes an “ownership change,” which is generally defined as a greater than 50 percent change, by value, in its equity ownership over a three-year period, the corporation’s ability to use its pre-change NOL carryforwards and other pre-change tax attributes to offset its post-change income or taxes may be limited. The Company may have experienced ownership changes in the past, including the Reverse Merger of Sevion Therapeutics, Inc. on December 19, 2017 at which time the Company’s pre-change U.S. federal NOL carryforward was \$77.2 million, and its research tax credit was \$0.7 million. The Company may experience additional ownership changes in the future as a result of subsequent shifts in its stock ownership, some of which may be outside of its control. Although the Company has not completed its analysis, it is reasonably possible that its federal NOLs available to offset future taxable income could materially decrease. This reduction would be offset by an adjustment to the existing valuation allowance for an equal and offsetting amount. Additionally, the 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 the Company’s Consolidated Financial Statements.

The Company is subject to income taxes in the United States and Israel. The Company files income tax returns in the U.S. and in several states. The federal and state tax returns are generally subject to tax examination by taxing authorities for tax years beginning in June 30, 2015 to present. To the extent the Company has tax attribute carryforwards, the tax years in which the attribute was generated may still be adjusted upon examination by the Internal Revenue Service or state tax authorities to the extent utilized in a future period. The Israeli income tax returns remain open to examination beginning in 2013 to present. If and when the Company claims NOL carryforwards from any prior years against future taxable income, those losses may be examined by the taxing authorities.

### 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 years ended December 31, 2019, 2018, and 2017, are as follows (in thousands, except share and per share data):

	Year ended December 31,		
	2019	2018	2017
<b>Numerator:</b>			
Net loss	\$ (50,874)	\$ (47,185)	\$ (21,214)
Less: Dividends accumulated for the period(1)	—	—	(2,404)
Net loss attributable to common stockholders	<u>\$ (50,874)</u>	<u>\$ (47,185)</u>	<u>\$ (23,618)</u>
<b>Denominator:</b>			
Weighted average number of shares common stock used in computing net loss per share, basic and diluted	38,063,173	32,436,506	4,976,377
Net loss per share, basic and diluted	<u>\$ (1.34)</u>	<u>\$ (1.45)</u>	<u>\$ (4.75)</u>

- (1) The net loss used for the computation of basic and diluted net loss per share includes 8% per share per annum compounded annually which was related to distributions for preferred stockholders of Eloxx Limited. On December 19, 2017 in conjunction with the Reverse Merger, all preferred shares were converted to common shares.

For the years ended December 31, 2019, 2018, and 2017, the totals of outstanding preferred stock, stock options, stock warrants and restricted stock units, as applicable, excluded from the calculation of the diluted net loss per share due to their anti-dilutive effect were 5,525,509, 4,163,107 and 19,027,306 respectively.

### 14. Other (Income) Expense, net

Other (income) expense consisted of the following (in thousands):

	Year ended December 31,		
	2019	2018	2017
Interest and other income	\$ (1,072)	\$ (601)	\$ —
Foreign currency exchange gains	76	92	156
Amortization and revaluation of embedded conversion feature in respect to convertible loan	—	—	625
Investment income, net	(301)	—	—
Interest and other expense	1,616	7	43
Total other expense (income), net	<u>\$ 319</u>	<u>\$ (502)</u>	<u>\$ 824</u>

### 15. Segment and Geographic Information

Operating segments are defined as components of an enterprise (business activity from which it earns revenue and incurs expenses) about which discrete financial information is available and regularly reviewed by the chief operating decision maker in deciding how to allocate resources and in assessing performance. The Company's chief operating decision maker is its Chief Executive Officer. The chief operating decision maker reviews consolidated operating results to make decisions about allocating resources and assessing performance for the entire company. The Company views its operations and manages its business as one operating segment; however, it operates in two geographic regions: the U.S. and Israel. Substantially all of the Company's assets are located in the U.S.

### 16. Reverse Merger

As described in Note 1, "Nature of the Business," the Reverse Merger was accounted for as a reverse recapitalization which is outside the scope of ASC Topic 805, Business Combinations ("ASC 805"). Under reverse capitalization accounting, Eloxx Limited is considered the acquirer for accounting and financial reporting purposes

and acquired the assets and assumed the liabilities of the Company. The assets acquired and liabilities assumed are reported at their historical amounts. The annual consolidated financial statements of the Company reflect the operations of the acquirer for accounting purposes together with a deemed issuance of shares, equivalent to the shares held by the former stockholders of the legal acquirer and a recapitalization of the equity of the accounting acquirer. The annual consolidated financial statements include the accounts of the Company since the effective date of the reverse merger and the accounts of Eloxx Limited since inception.

The following table summarizes the assets and liabilities assumed at the date of the Reverse Merger (in thousands):

	<b>December 19, 2017</b>
Cash and cash equivalents	\$ 123
Prepaid expenses and other current assets	220
Property, plant and equipment, net	39
Restricted bank deposits	6
Total assets acquired	388
Accounts payable	(215)
Accrued expenses	(343)
Total liabilities acquired	(558)
Total net liabilities acquired	\$ (170)

The Company incurred approximately \$0.6 million and \$1.3 million for the years ended December 31, 2018 and 2017, respectively, in professional fees related to the Reverse Merger. No fees were incurred related to the Reverse Merger for the year ended December 31, 2019.

## 17. Subsequent Events

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 organizational realignment reduces managerial layers and consolidates roles across the organization, resulting in the elimination of 13 full-time positions. These changes will take place throughout March 2020.

The Company expects to incur a one-time pre-tax charge, during the first quarter of 2020, consisting of employee separation costs of approximately \$1.7 million, primarily relating to severance and benefits costs. The Company also expects to incur a one-time non-cash stock compensation charge, during the first quarter of 2020, relating to accelerated vesting of executive stock awards, of approximately \$2.5 million.