UNITED STATES
SECURITIES AND EXCHANGE COMMISSION
WASHINGTON, DC 20549

FORM 10-K

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

For the fiscal year ended December 31, 2017

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: 001-35112

Aevi Genomic Medicine, Inc.

(Exact name of registrant as specified in its charter)

Delaware	**98-0217544**
(State or other jurisdiction of incorporation or organization)	**(I.R.S. Employer Identification No.)**
435 Devon Park Drive, Suite 715 Wayne, Pennsylvania	**19087**
(Address of Principal Executive Offices)	**(Zip Code)**

(610) 254-4201
(Registrant's telephone number, including area code)

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

Title of each class	**Name of exchange on which registered**
Common stock, par value $0.0001 per share	Nasdaq Global Market

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 Section 15(d) of the Act. Yes ☐ No ☒

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

Indicate by check mark whether the registrant has submitted electronically and posted on its corporate Web site, if any, every Interactive Data File required to be submitted and posted 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 and post such files). Yes ☒ No ☐

Indicate by check mark if disclosure of delinquent filers pursuant to Item 405 of Regulation S-K (§229.405) is not contained herein, and will not be contained, to the best of registrant's knowledge, in definitive proxy or information statements incorporated by reference in Part III of this Form 10-K or any amendment to this Form 10-K. ☐

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

Large accelerated filer	☐	Accelerated filer	☐
Non-accelerated filer	☐ (Do not check if a smaller reporting company)	Smaller reporting company	☒
		Emerging growth company	☐

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 common stock held by non-affiliates of the registrant, computed by reference to the closing price of the registrant's common stock on The NASDAQ Global Market on June 30, 2017, as of the last business day of the registrant's most recently completed second fiscal quarter was approximately $40.5 million.

As of March 9, 2018, the registrant had 59,337,265 shares of common stock outstanding.

DOCUMENTS INCORPORATED BY REFERENCE

Portions of the registrant's definitive proxy statement to be issued in conjunction with the registrant's annual meeting of stockholders to be held in 2018 are incorporated by reference into Part III of this Annual Report on Form 10-K. The proxy statement will be filed by the registrant with the Securities and Exchange Commission not later than 120 days after the end of the registrant's fiscal year ended December 31, 2017.

AEVI GENOMIC MEDICINE, INC.

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Cautionary Note Regarding Forward-Looking Statements

This Annual Report on Form 10-K contains "forward-looking statements" within the meaning of the safe harbor provisions of the U.S. Private Securities Litigation Reform Act of 1995, including statements regarding the progress and timing of clinical trials, the safety and efficacy of our product candidates, the goals of our development activities, estimates of the potential markets for our product candidates, estimates of the capacity of manufacturing and other facilities to support our products, our expected future revenues, operations and expenditures and projected cash needs. These statements relate to future events of our financial performance and involve known and unknown risks, uncertainties and other factors that could cause our actual results, levels of activity, performance or achievement to differ materially from those expressed or implied by these forward-looking statements. Those risks and uncertainties include, among others:

- our ability to obtain additional funding to develop our product candidates;
- the need to obtain regulatory approval of our product candidates and companion products;
- the success of our clinical trials through all phases of clinical development;
- any delays in regulatory review and approval of product candidates in clinical development;
- our ability to commercialize our product candidates;
- market acceptance of our product candidates;
- competition from existing products or new products that may emerge;
- regulatory difficulties relating to products that have already received regulatory approval;
- potential product liability claims;
- our dependency on third-party manufacturers to supply or manufacture our products;
- our ability to establish or maintain collaborations, licensing or other arrangements;
- our ability and third parties' abilities to protect intellectual property rights;
- compliance with obligations under intellectual property licenses with third parties;
- our ability to adequately support future growth; and
- our ability to attract and retain key personnel to manage our business effectively.

Forward-looking statements include all statements that are not historical facts. In some cases, you can identify forward-looking statements by terms such as "can," "may," "will," "should," "could," "would," "expects," "plans," "continues," "anticipates," "intends," "seeks," "targets," "believes," "estimates," "projects," "predicts," "potential," or the negative of those terms, and similar expressions and comparable terminology intended to identify forward-looking statements. These statements reflect our current views with respect to future events and are based on assumptions and subject to risks and uncertainties, including, but not limited to, those discussed in the section titled "Risk Factors" included in Part I, Item 1A of this Annual Report on Form 10-K. Given these uncertainties, you should not place undue reliance on these forward-looking statements. Any forward-looking statement speaks only as of the date of this report and, except as required by law, we undertake no obligation to update or review publicly any forward-looking statements, whether as a result of new information, future events or otherwise. We qualify all of our forward-looking statements by these cautionary statements.

Unless the context otherwise requires, all references in this Annual Report on Form 10-K to the "Company", "Aevi Genomic Medicine", "we," "us" and "our" refer to Aevi Genomic Medicine, Inc., a Delaware corporation organized on January 27, 2000, and its wholly-owned subsidiaries, Medgenics Medical (Israel) Ltd. and neuroFix, LLC. We use the Aevi Genomic Medicine logo as trademarks in the United States and elsewhere. All other trademarks or trade names referred to in this document are the property of their respective owners.

PART I

ITEM 1 Business.

Overview

We are a clinical stage biopharmaceutical company with an emphasis on identifying the genetic drivers of disease and applying this understanding to the pursuit of differentiated novel therapies primarily for pediatric onset, life-altering diseases, including rare and orphan diseases. We look to find treatments for genetically defined diseases for which there are limited therapeutic options currently available, with a primary focus on pediatric patients. This strategy begins with identifying and genetically validating a therapeutic target and using genomics to guide product development. The strategy also involves identifying and acquiring otherwise abandoned or overlooked drug candidates and matching targets and mechanisms of action to novel genetic discoveries.

We have partnered with the Center for Applied Genomics, or CAG, at The Children's Hospital of Philadelphia, or CHOP, to implement a genomic medicine driven approach to drug development. Included in the assets at CAG is a fully automated biorepository containing specimens from more than 75,000 pediatric patients and 150,000 relatives of those patients. The sample is highly enriched for rare and orphan diseases and the large majority of patients have been genotyped. Their phenotypes are recorded in a modern electronic health record that is linked to the genomics database and biorepository. The patients in the database have consented to anonymized use of their data for research and follow up contact if needed.

CAG continues to discover important and novel genetic biomarkers by both genome-wide association studies and exome sequencing and analysis of affected individuals and their family members. Such markers not only identify patients with the disease but frequently point to the cause of the disease and suggest targets and feasible intervention strategies that include protein or peptide therapy, monoclonal antibodies, drugs or gene therapy. By working initially in pediatric populations of specific diseases, we can minimize the confounding environmental factors seen in older patients. In addition, the availability of robust genetic biomarkers allows us to design trials that focus on a highly-enriched patient population that we believe is more likely to respond to targeted therapies and further enhance the likelihood of clinical and regulatory success. We believe this will allow us to implement clinical development programs that will lead to higher value medicines that can address critical needs in patients suffering from rare and orphan diseases.

Our Product Pipeline

The following table summarizes the status of our development programs as of the date of this Annual Report:

Compound	Indication	Preclinical	Phase 1	Phase 2	Phase 3
AEVI-001	mGluR+ Genetic Subset in ADHD (pediatric, age 6-17)				Top-Line Data Mid-2018
	mGluR+ Genetic Subset in ASD*				*Plan to Initiate H2 2018
AEVI-002 (anti-LIGHT mAb)	Severe Pediatric Onset Crohn's Disease				Initial Data Year-end 2018
AEVI-005	Undisclosed pediatric rare disease				Initiating in vitro POC work

AEVI-001 ***(mGluR+ Genetic Subset ADHD)***

The lead program from our genomic research collaboration with CHOP is the development candidate AEVI-001, an oral, non-stimulant glutamatergic neuromodulator. Through our acquisition of neuroFix, LLC, or neuroFix, in September 2015, we acquired the rights to develop AEVI-001 (then known as NFC-1), as well as the rights to certain data derived from a clinical trial and other studies of AEVI-001.

The selection of AEVI-001 for development in the mGluR+ ADHD patients was the result of a rational search process conducted to specifically identify therapeutic candidates with a demonstrated ability to modulate glutamate signaling via the mGluR network. The role of glutamate in ADHD and other CNS disorders is supported by recent neuroimaging studies that suggest glutamate levels are abnormal in children with ADHD. These abnormalities appear to be concentrated in the anterior singular cortex region of the brain, as evidenced by volumetric and functional magnetic resonance imagery studies, as well as targeted studies of magnetic resonance spectroscopy. Additional supportive evidence for targeting glutamate modulation is provided by genetic studies that have identified mutations in glutamatergic genes that are enriched in children with ADHD.

Our ADHD Opportunity

We are developing AEVI-001 to treat a sub-population of ADHD patients who have genetic mutations that disrupt the mGluR network, resulting in glutamate imbalance. ADHD is one of the most common childhood neurodevelopmental disorders of childhood. In the United States, the Center for Disease Control estimates that 6.4 million children 4-17 years of age (11%) have been diagnosed with ADHD. It is usually first diagnosed in childhood and often lasts into adulthood. Approximately 25% of ADHD patients are mGluR mutation positive, thereby representing approximately 1.5 million pediatric and 2.5 million adult patients in the United States. Based on pricing assumptions of currently available ADHD therapies, as well as established compliance and adherence rates, this equates to a potential $2 billion to $3 billion market opportunity for the drug.

ADHD is defined as a persistent pattern of inattention and/or hyperactivity-impulsivity that interferes with functioning or development. ADHD causes significant impairment in childhood and throughout the lifespan, as well as increased mortality and psychosocial adversity. There is no definitive management for ADHD; current management frequently includes a combination of educational support, behavioral interventions, and pharmacotherapy. Current standard of care is the stimulant class of medications including immediate- and extended-release methylphenidate and amphetamine; these products represent 90% of sales in the United States. In 2016, ADHD pharmaceutical product sales in the United States were approximately $11 billion, and grew at a compounded annual growth rate of approximately 2% from 2012 to 2016. However, while conferring great benefit for many individuals, currently available ADHD medications also have significant limitations including decreased appetite, weight loss, and insomnia.

Prevalence of mGluR Network Mutations

To examine the prevalence of mGluR network mutations in the broader pediatric and adolescent ADHD populations, we conducted a large-scale non-interventional phenotype/genotype study at 32 sites across the United States. The study genotyped 1,876 ADHD patients aged 6-17 years, with 420 children and adolescents being mGluR+ (22.4%). A higher prevalence (75/292, 26%) was seen in patients aged 6-12 years than patients aged 13-17 years (344/1584, 21%). The data also showed that patients with the mGluR mutations had significantly higher prevalence of symptoms associated with inappropriate movements, disruptive behavior, and anger control.

mGluR Network Mutations Highly Predictive of ADHD

A study genotyped 3,445 ADHD patients from the CHOP Psychiatry and Behavioral Sciences Clinics to classify the prevalence of copy number variation mGluR+ mutations and the proportion of those patients who had already been diagnosed with ADHD. The research demonstrated the association between the excitatory glutamate neurotransmitter in the brain, mutations in the mGluR pathway, and ADHD in pediatric patients who possess these mutations. The study also clearly demonstrated the highly predictive capabilities of the genetic biomarker, as demonstrated by the fact that 98% of the patients with the identified mGluR network mutations had a positive diagnosis of ADHD (the study was conducted on a blinded basis). We believe the genomic validation for AEVI-001 addresses a key inefficiency in the current ADHD diagnosis and treatment paradigm and may lead to improved safety and ultimately a personalized approach to treatment.

Development of AEVI-001 in mGluR+ Genetic Subset ADHD

AEVI-001 completed a Phase 2/3 trial (which we refer to as the SAGA trial) in adolescent ADHD patients with specific mutations in their mGluR gene network, which we refer to as mGluR+ ADHD, in the first quarter of 2017. Although AEVI-001 did not meet the primary endpoint of reduction on the ADHD rating scale (ADHD-RS) compared to placebo, in the SAGA trial, the drug did demonstrate statistically significant and clinically meaningful improvement compared to placebo in a pre-specified responder analysis of ADHD-RS improvement of 30% or more [ADHD-RS reduction of 17.6, $p < .005$]. In a second pre-specified responder analysis of Clinical Global Impression of Improvement scale (CGI-I), a key secondary endpoint, AEVI-001 demonstrated a statistically significant and clinically meaningful improvement compared to placebo [57% of patients treated with AEVI-001 achieved a score of much improved or very much improved compared to 33% on placebo, $p=0.0155$]. Additionally, the safety analysis demonstrated that AEVI-001 was well tolerated at all doses and the majority of adverse events were generally mild to moderate in severity. There were no serious adverse events.

Subsequent analysis of responder data from a subset of genomically identified patients in the SAGA trial identified nine genes (genetic subset) that appear to be predictive of clinically meaningful and statistically significant response on the ADHD-RS scales and CGI-I scales. These genes include certain glutamate metabotropic receptors and neurodevelopmental genes that are found in approximately 10% of pediatric ADHD patients.

One of the neurodevelopmental genes, contactin-4 (CNTN4), has been previously identified as being important in Autism Spectrum Disorder (ASD) representing approximately 5% of the overall pediatric ADHD patient population. The CNTN4 mutation phenotype is relatively severe, with an increased prevalence of emotional dysregulation, which includes issues related to anger control, risk taking, and inappropriate movements and sounds. All of the CNTN4 mutation positive (CNTN4+) patients on treatment (n=6, 100%) had clinically meaningful and statistically significant response to therapy with AEVI-001 [ADHD-RS reduction of 20.8, $p=0.03$].

Importantly, these results clarify a path forward for the continued development of AEVI-001 in ADHD, as well as in other potential neurodevelopmental disorders, including but not limited to ASD and Pediatric Generalized Anxiety Disorder. We have initiated a Phase 2 trial in the mGluR mutation positive genetic subset ADHD ("mGluR+ Genetic Subset ADHD") to confirm genetic responders to AEVI-001. Patient screening began in the third quarter of 2017 and data is expected by mid-2018.

In the United States, mGluR+ Genetic Subset ADHD represents approximately 10% of ADHD patients, estimated at 600,000 pediatric and 1.5 million adult. Based on pricing assumptions of currently available ADHD therapies, as well as established compliance and adherence rates, this equates to a potential $2 billion to $3 billion market opportunity for AEVI-001.

Diagnostic Development in ADHD

As part of our precision medicine strategy, Aevi is looking to develop and commercialize novel diagnostic tests to support therapies in development. For AEVI-001, Aevi is developing a stand-alone diagnostic to be used as an aid in the diagnosis of ADHD in patients aged 6-17, based on the discovery that mutations in the mGluR network are highly associated with ADHD. Aevi has engaged the US FDA on seeking a path to clearance for the diagnostic test. In addition to providing valuable information for the diagnosis of pediatric patients with mGluR+ ADHD, the diagnostic would support pre-identification of patients for future clinical trials in ADHD.

Previous Study of AEVI-001

The originator company for AEVI-001, Nippon Shinyaku, conducted research showing the ability of AEVI-001 to cross the blood-brain barrier and ameliorate cognitive impairment in animal behavioral models, at concentrations achievable in humans. AEVI-001 was shown to have a compelling pharmacokinetic and metabolic profile and to be a pan-selective activator and modulator of multiple mGluRs. Nippon Shinyaku studied AEVI-001 in vascular dementia, where approximately 1,000 adult patients were exposed to AEVI-001 for periods up to 12 months, in a development program that progressed to Phase 3. AEVI-001 was shown to be well tolerated with no treatment-emergent serious adverse events in this patient population, but was not effective for the treatment of vascular dementia.

The GREAT Study

A Phase Ib proof of concept trial (which we refer to as the GREAT trial) of AEVI-001 in adolescent patients with ADHD was completed in 2015. The study enrolled 30 adolescents aged 12-17 with severe and genetically confirmed mGluR+ ADHD. Of the 30 enrolled patients, 17 had Tier 1 mGluR mutations, which are mutations in genes in the mGluR receptors or in genes that directly influence mGluR signaling. Seven patients had Tier 2 mutations, which are mutations in genes that encode proteins that influence mGluR. The remaining six patients had more distal Tier 3 mutations, which are mutations in genes that encode proteins that influence Tier 1 and Tier 2 genes.

Part 1 of the study measured safety and the pharmacokinetic profile of single ascending doses of 50-800mg of AEVI- 001. Part 2 of the study was single-blinded to patients and caregivers. Dosing was one week with placebo followed by four weeks of ascending doses from 50mg BID to 400mg BID of AEVI-001. The study used the Clinical Global Impression of Symptom Improvement (CGI-I) and the Vanderbilt Parent Rating Score (similar to the ADHD Rating Scale) to assess efficacy. Despite not being powered to show efficacy, the study demonstrated dose and duration-dependent improvements and response rates comparable to best-in-class ADHD therapies.

The treatment effect was more robust over time and at higher doses. In all patients, AEVI-001 showed weekly improvements in mean CGI-I from 3.79 during week 1 on placebo (baseline), 3.13 during week 2 (50mg BID), 2.79 during week 3 (100mg BID), 2.79 during week 4 (200mg BID) and 2.21 during week 5 (400mg BID). In all patients, AEVI-001 likewise showed weekly improvements in mean Vanderbilt scores from 29.1 during week 1 on placebo (baseline), 26.4 during week 2 (50mg BID), 24.0 during week 3 (100mg BID), 23.3 during week 4 (200mg BID) and 22.5 during week 5 (400mg BID).

The GREAT study also confirmed the previously observed pharmacokinetic profile of AEVI-001, showing the therapy to be well tolerated with no treatment-related SAEs. Following the conclusion of the study, a majority of patients enrolled in an open label long-term safety study. Full data from the study was presented at the American Academy of Child and Adolescent Psychiatry meeting in October 2015.

Development of AEVI-001 in 22q Deletion Syndrome (22q DS)

We completed work on a signal-finding trial for the treatment of the psychiatric symptoms of 22q Deletion Syndrome (22q DS) in 2017. 22q DS is an orphan, severe autism spectrum disorder with significant co-morbidities. The disease has a prevalence of between 1:2000-1:4000, roughly equivalent with the more recognized Down's Syndrome. Enrolling patients into the signal-finding study was difficult, with only two patients enrolled by the time the study ended. Due to the limited enrollment, it was not feasible to meaningfully interpret the resulting data, and the program was terminated.

Future Development of AEVI-001 in ASD

We are exploring a development opportunity for AEVI-001 for the treatment of mGluR+ patients with ASD to better define the patient phenotype and intend to initiate work on a proof-of-concept study to begin in the second half of 2018. In 2012, 1 in 68 children were diagnosed with ASD in the United States, increasing from 1 in 150 in 2000. There is a high unmet need for pharmaceutical treatments for ASD as currently approved medications are indicated only for the symptoms of irritability in ASD patients. There are currently limited pharmacotherapy options available to treat ASD.

AEVI-002 ***(Anti-LIGHT Monoclonal Antibody)***

The second program arising out of our genomic research collaboration with CHOP is the development candidate AEVI-002, a first-in-class anti-LIGHT monoclonal antibody, or the Antibody, being developed for use in Pediatric Onset Crohn's disease. Pediatric Onset Crohn's disease has a more aggressive phenotype at younger ages. The genomic rationale for the use of anti-LIGHT antibody in Crohn's disease was validated by CAG research showing the association to a loss of function mutation in decoy receptor 3 (DcR3).

In June 2016, we entered into a Clinical Development and Option Agreement, or the Development and Option Agreement, with Kyowa Hakko Kirin Co., Ltd., or KHK, pursuant to which we acquired certain rights with respect to the development and potential commercialization of the Antibody. Under the Development and Option Agreement, we received an exclusive option for exclusive rights to develop products containing the Antibody, or an Antibody Licensed Product, exclusive rights to commercialize Antibody Licensed Product in various countries and to conduct various development activities with respect to the Antibody Licensed Product, including the conduct of a signal finding study testing the Antibody in Severe Pediatric Onset Inflammatory Bowel Disease, or the Study. The terms of the Development and Option Agreement with KHK are more fully described under the section entitled "Licenses."

An 8-week Phase Ib proof-of-concept study has been initiated at CHOP with the goal of enrolling up to 12 patients with a Pediatric Onset Crohn's disease diagnosis with most patients being refractory to treatment with TNF-α inhibitors, with or without a DcR3 mutation. The endpoints of the trial will include endoscopic evaluation, Crohn's Disease Activity Index ratings and safety. Initial data from the proof-of-concept study is expected by year-end 2018, at which point we will make a determination on our option to license exclusive rights to the Antibody for further development. Active recruitment for the trial is underway, although the identification and recruitment of patients into the proof-of-concept study has been extremely challenging, and to date no patients have been enrolled. The ability to produce initial data by year-end 2018 is highly dependent on timely recruiting; thus, continued difficulties in recruitment could cause a delay in the delivery of initial data for the program. In an effort to address the recruitment challenges, we are currently initiating three additional trial sites for the program.

Business Strategy

Our goal is to translate key scientific insights relating to underlying genomic drivers of disease into the development of effective and highly selective therapeutics. To execute our strategy, we intend to:

- *Advance our lead product candidate AEVI-001 through clinical development.* AEVI-001, a first-in-class non-stimulant mGluR modulator, is being developed for the treatment of mGluR+ Genetic Subset ADHD. AEVI-001 is currently being studied in a Phase 2 trial to confirm genetic responders to AEVI-001. Patient screening began in the third quarter of 2017 and data is expected by mid-2018.

- *Pursue development of AEVI-001 for various other diseases where our genomic insights suggest it may be an effective therapy.* In addition to mGluR+ Genetic Subset ADHD, we intend to develop AEVI-001 for the treatment of certain genetically defined patient subsets with other neurological and neuropsychological indications, including but not limited to ASD.

- *Advance our second clinical candidate AEVI-002 through clinical development.* The second program arising out of our genomic research collaboration with CHOP is the development candidate AEVI-002, a first-in-class anti-LIGHT monoclonal antibody being developed for use in Pediatric Onset Crohn's disease. An 8-week signal finding study at CHOP has been initiated and will enroll up to 12 patients with the DcR3 mutation and a Pediatric Onset Crohn's disease diagnosis, with most subjects being refractory to treatment with TNF-α inhibitors. Initial data from the proof-of-concept study is expected by year-end 2018, at which point we will make a determination on our option to license exclusive rights to the antibody for further development.

- *Leverage our strategic collaborations to continue to implement a genomic medicine driven approach to drug development.* Our strategy is to work closely with our collaborators at CAG to identify populations of need with well-characterized, novel, genetically-defined targets. We then designate an actionable therapeutic development approach based upon the target and the biology and human pathophysiology of the relevant disease and likely clinical and regulatory pathways. The collaboration affords us with unique and proprietary insight into these diseases and allows us to better select therapeutic approaches.

- *Work with experienced third parties in the field of diagnostics.* Because we often target genetic alterations that are detectable, companion diagnostics can be developed to identify these alterations. Once we have identified a target, we will initially use existing diagnostic tools to identify patient subsets that we believe will derive increased benefit from our product candidates. As we advance our targets clinically and determine the most important screening criteria, we will develop companion diagnostics as appropriate, with the help of technology partners, to identify patients and support registration and marketing of our product candidates.

- *Opportunistically in-license and acquire novel therapies for the treatment of rare and orphan disease.* We plan to leverage our clinical drug development expertise and our relationships in the rare and orphan diseases community to identify and in-license or acquire additional product candidates that we believe have the potential to become novel treatments for diseases with significant unmet medical needs.

- *Potentially seek strategic collaborative relationships while maintaining flexibility in commercializing and maximizing the value of our development programs.* We plan to develop and seek regulatory approval for multiple product candidates in our development pipeline. While we may develop these products independently, we still may enter into strategic relationships with biotechnology or pharmaceutical companies to realize the full value of these products.

Intellectual Property

Our goals are to obtain, maintain, and enforce patent and trademark protection for our products, processes, methods, and other proprietary technologies, including the platform collaboration with CHOP and to preserve our trade secrets both in the United States and elsewhere in the world. Our policy is to actively seek to obtain, where appropriate, the broadest intellectual property protection possible for our products, processes and methods that arise from our genomics platform collaboration with CHOP through a combination of contractual arrangements, trade secrets, patents, and trademarks both in the United States and abroad.

Our ability to compete depends on our ability to maintain and enforce our intellectual property rights and operating without infringing the intellectual property of others and our ability to enforce our licenses. Our business could be materially harmed, and we could be subject to liabilities, because of lawsuits brought by others against us or our licensors and licensees. We will be able to protect our technology from unauthorized use by third parties only to the extent it is covered by valid and enforceable patents or is effectively maintained as trade secrets. Patents and other proprietary rights are an essential and material element of our business. Applications for patents and other intellectual property rights capable of being registered have been, and will be, filed in certain key jurisdictions. As we identify additional rare and orphan disease targets, we will seek protection for the related intellectual property rights in the United States and other relevant jurisdictions. There can be no assurance that the pending applications will result in patents ultimately being issued.

Our patent portfolio for AEVI-001 and AEVI-002 consists of licensed patents and patent applications. The applicable licenses are discussed below.

We also depend upon the skills, knowledge and experience of our scientific and technical personnel, as well as that of our advisors, consultants and other contractors, none of which is patentable. To help protect our proprietary knowledge and experience that is not patentable, and for inventions for which patents may be difficult to enforce, we rely on trade secret protection and confidentiality agreements with our employees, consultants, vendors, collaborators, advisors, customers and other third parties to protect our interests. To this end, we require all employees, consultants, advisors and other contractors to enter into confidentiality agreements that prohibit the disclosure of confidential information and, where applicable, require disclosure and assignment to us of the ideas, developments, discoveries and inventions important to our business. We also require confidentiality or material transfer agreements from third parties that receive our confidential data or materials. We intend to continue to take all appropriate steps to protect our intellectual property, including maintaining an active program for patent protection for novel elements in the development of our products and technology.

Licenses

neuroFix License

Immediately prior to and in connection with our acquisition of neuroFix in September 2015, neuroFix entered into a license agreement with CHOP, pursuant to which CHOP licensed to neuroFix certain technology owned and controlled by CHOP related to ADHD and certain other neurological and neuropsychological indications. Pursuant to this license agreement, CHOP licensed to neuroFix (coupled with a right to sublicense) certain patent rights and compound know-how on an exclusive, worldwide, royalty-bearing right and license basis, and certain CHOP know-how (other than compound know-how) on a non-exclusive, worldwide, royalty-bearing right and license basis. CHOP also granted to neuroFix an exclusive option during the term of the license agreement to negotiate an exclusive license to certain future CHOP intellectual property.

Pursuant to this license agreement, CHOP retained rights to the licensed patent rights and know-how to conduct teaching, educational, research and patient care activities itself and to conduct collaborations with certain not-for-profit, governmental, educational or non-commercial third parties and for purposes outside of the field of the license. Under the license agreement, neuroFix granted to CHOP a non-exclusive, worldwide, fully paid-up, royalty-free license under all intellectual property rights controlled by neuroFix to make and use certain products for education and non-commercial research purposes.

In addition to neuroFix having issued equity to CHOP in partial consideration for the rights granted under the license agreement (which equity was issued immediately prior to, and subsequently purchased by us in, to the acquisition), CHOP is eligible for certain milestone and royalty payments under the license agreement as further described below:

- up to $1.5 million in regulatory and sales milestone payments in connection with each FDA-approved indication obtained by neuroFix utilizing intellectual property licensed under the license agreement;
- royalty payments equal to a percentage of certain product sales by neuroFix using a fluctuating rate in the low single digits (adjusted downward to the extent third party royalty payments exceed a certain percentage in a given calendar quarter);
- annual maintenance fees of equal to or less than $100,000 depending on the year; and
- a certain percentage (ranging from mid-single digits to the mid-teens depending on if other rights of neuroFix are also licensed to the sublicensee at the same time) of all sublicensee income (except any amounts attributable to sublicensed sales by a certain party in Japan).

The license agreement will terminate, with respect to each product and each territory covered by the license agreement, upon the later of (i) the expiration of certain CHOP patent rights and (ii) January 1, 2025, at which time the license rights granted to neuroFix become perpetual, irrevocable, fully paid-up and royalty-free. The license agreement could also be subject to termination by CHOP if neuroFix is not achieving certain specified development plans and diligence events and is not undertaking commercially reasonable efforts to achieve such events.

CHOP License Agreement and Sponsored Research Agreement

In November 2014, we entered into a license agreement, or the License Agreement, and a sponsored research agreement, or the Research Agreement, each with CHOP. Under the terms of the License Agreement, CHOP granted us (i) an exclusive, sublicensable license to use certain patent rights covering potential diagnostic and therapeutic targets, (ii) an exclusive, non-sublicensable license to use certain biospecimen and phenotypic data collected from patients with rare and orphan diseases and their family members, or the Biobank, (iii) a non-exclusive, sublicensable license to use certain know-how related to such patent rights, biospecimen and phenotypic data, (iv) a non-exclusive and non-sublicensable license to use certain biospecimen and phenotypic data collected from patients with non-rare and orphan diseases, and (v) an exclusive option to negotiate licenses to commercialize certain inventions that may be created in the future that target rare and orphan diseases. In consideration of the licenses and option granted under the License Agreement, we agreed to pay to CHOP a license issuance fee of $500,000, certain maintenance fees, certain milestone payments, low single-digit royalties on net sales of all licensed products and a percentage of amounts received from sublicensing activities. In February 2017, we amended the License Agreement. The amendment allows us to extend the period of our exclusive commercial access to the Biobank for rolling two year periods. The cost of each extension is $125,000 per year.

Under the terms of the Research Agreement, we agreed to sponsor research at CHOP with respect to the recruitment and genetic analysis of patients with rare and/or orphan diseases to accelerate discovery of diagnostic and therapeutic targets. In February 2017, we amended the License Agreement. The amendment allows us to extend the period of our exclusive commercial access to the Biobank for rolling two year periods. The cost of each extension is $125,000 per year. In June 2017, we entered into an amendment to the Research Agreement, which extended the Research Agreement through June 30, 2019, for which payments totaling $5.94 million will be due in 2018 and $2.38 million will be due in 2019.

The License Agreement would terminate upon the expiration date of the last-to-expire royalty term under the License Agreement, however (i) CHOP may terminate the License Agreement upon an uncured default by us or the failure by us to meet certain development and/or commercialization milestones under the License Agreement or if we become insolvent or enter into bankruptcy proceedings, and (ii) we may terminate the License at any time with six months prior written notice to CHOP.

Development and Option Agreement, with Kyowa Hakko Kirin Co., Ltd. (KHK)

In June 2016, we entered into the Development and Option Agreement with KHK pursuant to which we acquired certain rights with respect to the development and potential commercialization of the Antibody. If we exercise our option under the Development and Option Agreement, KHK has 60 days to select one of two development and commercialization structures as follows:

PLAN A: Co-Development/Co-Commercialization Arrangement

If KHK selects the co-development/co-commercialization arrangement (Plan A), we will have the exclusive right to develop, manufacture and commercialize the Antibody Licensed Products in the treatment, prevention, and diagnosis of specified pediatric onset rare and orphan inflammatory diseases (including severe pediatric onset inflammatory bowel diseases such as Crohn's disease and ulcerative colitis, or IBD) and other specified pediatric onset rare and orphan auto-immune diseases, or collectively, the Field, in the United States and Canada. We will also be responsible for development and regulatory approval of the first Antibody Licensed Product in the European Union and then transferring such regulatory approval to KHK or its designee. We will be responsible for the manufacture of the Antibody Licensed Products for use by the parties in clinical trials as well as for commercialization in their respective fields and/or territories, with KHK purchasing the Antibody Licensed Products from us.

We will be required to pay KHK an initial license fee in the low single-digit millions of dollars upon the co-development/co-commercialization arrangement becoming effective. We may pay KHK up to an additional $18 million upon the achievement of certain regulatory milestones related to the Antibody Licensed Products. The parties will share the anticipated costs of development of the first Antibody Licensed Product in the Field in the United States, Canada and the European Union with us being responsible for any costs in excess of an agreed cap. The parties will split profits from our sales of Antibody Licensed Products in the United States and Canada equally. KHK will pay us low double-digit royalties for sales of Antibody Licensed Products outside the United States and Canada and outside the Field in the United States and Canada.

PLAN B: Licensing Arrangement

If KHK selects the licensing arrangement (Plan B), we will have the exclusive right to develop, manufacture and commercialize the Antibody Licensed Products in the Field in the United States, Canada and the European Union. We will be responsible for the manufacture of the Antibody Licensed Products for use by the parties in clinical trials as well as for commercialization in their respective fields and/or territories.

We will be required to pay KHK an initial license fee in the low single-digit millions of dollars upon the licensing arrangement becoming effective. We may pay KHK up to an additional $28 million upon the achievement of certain regulatory milestones related to the Antibody Licensed Products. The parties will split profits from our sales of Antibody Licensed Products in the United States, Canada and the European Union with us being entitled to approximately 74% of such profits and KHK being entitled to approximately 26% of such profits. KHK will pay us low double-digit royalties for sales of Antibody Licensed Products outside the United States, Canada and the European Union and outside the Field in the United States, Canada and the European Union. We will be responsible for costs of development of Licensed Products in the United States, Canada and the European Union. KHK will have the right to purchase the Antibody Licensed Products from us.

Trademarks

Certain names utilized for our products and tools are trademarked, and certain names utilized for our products and tools are the subject of trademark registrations and applications in certain jurisdictions. The final choice of names for products and tools has not yet been made and will be subject to marketing considerations and other factors.

There can be no assurance that a third party will not oppose any registration, that the respective Trademark Offices will issue a registration certificate or that we will otherwise be successful in perfecting trademark rights for the marks in the United States or in foreign countries, the results of any of which would likely have a material adverse effect on our company.

Government Regulation

General

The production, distribution, and marketing of products employing our technology, and our development activities, are subject to extensive governmental regulation in the United States and in other countries. In the United States, our products are subject to the Federal Food, Drug, and Cosmetic Act, as amended, or FDCA, and the regulations of the FDA, as well as to other federal, state, and local statutes and regulations. These laws, and similar laws outside the United States, govern the clinical and preclinical testing, manufacture, safety, effectiveness, approval, labeling, distribution, sale, import, export, storage, record-keeping, reporting, advertising, and promotion of our products. Product development and approval within this regulatory framework, if successful, will take many years and involve the expenditure of substantial resources. Violations of regulatory requirements at any stage may result in various adverse consequences, including the FDA's and other regulatory health agencies' delay in approving or refusal to approve a product. Violations of regulatory requirements also may result in enforcement actions.

The following paragraphs provide further information on certain legal and regulatory issues with a particular potential to affect our operations or future marketing of products employing our technology.

FDA Approval Process

To obtain approval of a new product from the FDA, we must, among other requirements, submit data demonstrating the product's safety and efficacy as well as detailed information on the manufacture and composition of the product candidate. In most cases, this entails extensive laboratory tests and preclinical and clinical trials. This testing and the preparation of necessary applications and processing of those applications by the FDA are expensive and typically take many years to complete. The FDA may deny our applications or may not act quickly or favorably in reviewing these applications, and we may encounter significant difficulties or costs in our efforts to obtain FDA approvals that could delay or preclude us from marketing any products we may develop. The FDA also may require post-marketing testing and surveillance to monitor the effects of approved products or place conditions on any approvals that could restrict the commercial applications of these products. Regulatory authorities may withdraw product approvals if we fail to comply with regulatory standards or if we encounter problems following initial marketing. With respect to patented products or technologies, delays imposed by the governmental approval process may materially reduce the period during which we may have the exclusive right to exploit the products or technologies.

Currently all of our product candidates as well as other therapies we are exploring, regardless of therapeutic modality, will be considered to be a drug or biologic from a regulatory standpoint. The process required by the FDA before a new drug or biologic may be marketed in the United States generally involves the following:

- completion of pre-clinical laboratory tests or studies and formulation studies;
- submission to the FDA of an IND for a new drug or biologic, which must become effective before human clinical trials may begin;
- performance of adequate and well-controlled human clinical trials to establish the safety and efficacy of the proposed drug or biologic for its intended use;
- detailed information on product characterization and manufacturing process; and
- submission and approval of a New Drug Application, or NDA, for a drug, or a BLA for a biologic.

Pre-clinical tests include laboratory evaluation of product chemistry formulation and stability, as well as animal and other studies to evaluate toxicity. Under FDA regulations, the results of any pre-clinical testing, together with manufacturing information and analytical data, are submitted to the FDA as part of an IND. Additionally, for certain pediatric products, the sponsor may be required to submit an initial Pediatric Study Plan (discussed below) as a pre-IND submission. The FDA requires a 30-day waiting period after the filing of each IND before clinical trials may begin, in order to ensure that human research patients will not be exposed to unreasonable health risks. At any time during this 30-day period or at any time thereafter, the FDA may halt proposed or ongoing clinical trials, may authorize trials only on specified terms, or may require additional trials. The IND process may become extremely costly and substantially delay development of our products. Moreover, positive results of pre-clinical tests will not necessarily indicate positive results in clinical trials.

The sponsor typically conducts human clinical trials in three sequential phases, which may overlap. These phases generally include the following:

- Phase 1: The product candidate is usually first introduced into healthy humans or, on occasion, into patients, and is tested for safety, dosage tolerance, absorption, distribution, excretion and metabolism;
- Phase 2: The product candidate is introduced into a limited patient population to:
    - assess its efficacy in specific, targeted indications;
    - assess dosage tolerance and optimal dosage; and
    - identify possible adverse effects and safety risks.
- Phase 3: These are commonly referred to as pivotal studies. If a product candidate is found to have an acceptable safety profile and to be potentially effective in Phase 2 clinical trials, clinical trials in Phase 3 will be initiated to further demonstrate clinical efficacy, optimal dosage and safety within an expanded and diverse patient population at geographically dispersed clinical trial sites; and
- If the FDA does ultimately approve the product candidate, it may require post-marketing testing, including potentially expensive Phase 4 studies, to confirm or further evaluate its safety and effectiveness. Continued ability to commercialize the product may be based on the successful completion of these additional studies.

Before proceeding with a trial, the sponsor may seek a written agreement from the FDA regarding the design, size, and conduct of a clinical trial. This is known as a Special Protocol Assessment, or SPA. Among other things, SPAs can cover clinical trials for pivotal studies whose data will form the primary basis to establish a product's efficacy. SPAs thus help establish up-front concurrence with the FDA about the adequacy of a clinical trial design to support a regulatory approval, but the agreement is not binding if new circumstances arise. Even if the FDA agrees to a SPA, the agreement may be changed by the sponsor or the FDA on written agreement by either parties, or if a senior FDA official determines that a substantial scientific issue essential to determining the safety or effectiveness of the product was identified after the testing began. There is no guarantee that a study will ultimately be adequate to support an approval, even if the study is subject to a SPA. The FDA retains significant latitude and discretion in interpreting the terms of the SPA and the data and results from any study that is the subject of the SPA.

Pediatric product development is subject to additional FDA regulations, including the Best Pharmaceuticals for Children Act and the Pediatric Research Equity Act, as amended by the FDA Reauthorization Act of 2017, which may impact whether FDA grants orphan designation for pediatric subpopulations of common diseases (discussed below) and could require pediatric studies. Sponsors may be required to submit an initial Pediatric Study Plan (iPSP) before the initiation of any phase 3 studies unless certain exemptions apply. Where a sponsor is required to submit an iPSP, the sponsor must reach an agreement with FDA before submitting a marketing application or supplement. FDA agreement on a iPSP does not guarantee that the study will ultimately be adequate to support an approval.

Clinical trials must meet requirements for Institutional Review Board, or IRB, oversight, patient informed consent and the FDA's Good Clinical Practice, or GCP. Prior to commencement of each clinical trial, the sponsor must submit to the FDA a clinical plan, or protocol, accompanied by the approval of the committee responsible for overseeing clinical trials at the clinical trial sites. The FDA or the IRB at each institution at which a clinical trial is being performed may order the temporary or permanent discontinuation of a clinical trial at any time if it believes that the clinical trial is not being conducted in accordance with FDA requirements or presents an unacceptable risk to the clinical trial patients. Data safety monitoring committees, which monitor certain studies to protect the welfare of study patients, may also require that a clinical trial be discontinued or modified.

The sponsor must submit to the FDA the results of the pre-clinical and clinical trials, together with, among other things, detailed information on the manufacturing and composition of the product, and proposed labeling, in the form of an NDA, or, in the case of a biologic, a BLA. The applicant must also submit with the NDA or BLA a substantial user fee payment, unless a waiver or reduction applies. In some cases, a sponsor may be able to expand the indications in an approved NDA or BLA through a submission of a Prior Approval Supplement. Each NDA or BLA submitted for FDA approval is usually reviewed for administrative completeness and reviewability within 60 days following submission of the application. If deemed complete, the FDA will "file" the NDA or BLA, thereby triggering substantive review of the application. The FDA can refuse to file any NDA or BLA that it deems incomplete or not properly reviewable. Once the submission has been accepted for filing, the FDA will review the application and will usually respond to the applicant in accordance with performance goals the FDA has established for the review of NDAs and BLAs - six months from the receipt of the application for priority applications and ten to twelve months for regular applications. The review process is often significantly extended by FDA requests for additional information, pre-clinical studies or clinical trials, clarification, or a risk evaluation and mitigation strategy, or REMS, or by changes to the application submitted by the applicant in the form of amendments. The FDA may refer applications for novel product candidates which present difficult questions of safety or efficacy to an advisory committee, typically a panel that includes clinicians and other experts, for review, evaluation, and a recommendation as to whether the application should be approved and under what conditions. The FDA is not bound by the recommendations of an advisory committee, but it considers such recommendations carefully when making decisions.

Before approving an NDA or BLA, the FDA will often inspect the facilities at which the product is manufactured and will not approve the product unless the manufacturing facilities are in compliance with current Good Manufacturing Practice, or cGMP, requirements which govern the manufacture, holding and distribution of a product.

It is possible that our product candidates will not successfully proceed through this approval process or that the FDA will not approve them in any specific period of time, or at all. The FDA may deny or delay approval of applications that do not meet applicable regulatory criteria, or if the FDA determines that the clinical data does not adequately establish the safety and efficacy of the product. Satisfaction of FDA pre-market approval requirements for a new product candidate is a process that may take a number of years and the actual time required may vary substantially based upon the type, complexity and novelty of the product or disease. The FDA reviews these applications and, when and if it decides that adequate data is available to show that the product is both safe and effective and that other applicable requirements have been met, approves the product candidate for marketing. Government regulation may delay or prevent marketing of potential products for a considerable period of time and imposes costly procedures upon our activities. Success in early stage clinical trials does not assure success in later stage clinical trials. Data obtained from clinical activities is not always conclusive and may be susceptible to varying interpretations that could delay, limit or prevent regulatory approval. Upon approval, a product candidate may be marketed only for those indications approved in the NDA or BLA and will be subject to labeling and promotional requirements or limitations, including warnings, precautions, contraindications and use limitations, which could materially impact profitability. Once approved, the FDA may withdraw the product approval if compliance with pre- and post-market regulatory standards and requirements are not maintained or if safety, efficacy or other problems occur after the product reaches the marketplace.

The FDA may, during its review of an NDA or BLA, ask for additional study data. If the FDA does ultimately approve the product, approval may be subject to limitations based on the FDA's interpretation of the existing pre-clinical and clinical data and the FDA may require post-marketing testing, including potentially expensive Phase 4 studies, to confirm or otherwise further evaluate the safety and effectiveness of the product. The FDA also may require, as a condition to approval or continued marketing of a drug, a REMS to ensure that the benefits of a drug or biologic product outweigh its risks. REMS can include additional educational materials for healthcare professionals and patients such as Medication Guides and Patient Package Inserts, a plan for communicating information to healthcare professionals, and restricted distribution of the product. In addition, the FDA may, in some circumstances, impose restrictions on the use of the product, which may be difficult and expensive to administer and may require prior approval of promotional materials. Following approval, the FDA may require labeling changes or impose new post-approval study, risk management, or distribution restriction requirements.

The FDA has developed four distinct approaches intended to make drugs that address unmet medical needs for serious or life threatening conditions available as rapidly as possible, especially when the drugs are the first available treatment or have advantages over existing treatments: accelerated approval, fast track, breakthrough therapy, and priority review. The FDA requires a manufacturer who receives certain designations to make publicly available its policy for responding to requests for individual patient expanded access.

- Accelerated Approval. The FDA may grant "accelerated approval" status to drugs or biologics that treat serious or life-threatening illnesses and that provide meaningful therapeutic benefits to patients over existing treatments. Under this pathway, the FDA may approve a product based on surrogate endpoints, or clinical endpoints other than survival or irreversible morbidity. When approval is based on surrogate endpoints or clinical endpoints other than survival or morbidity, the sponsor will be required to conduct additional post-approval clinical trials to verify and describe clinical benefit. Under the agency's accelerated approval regulations, if the FDA concludes that a product that has been shown to be effective can be safely used only if distribution or use is restricted, it may require certain post-marketing restrictions as necessary to assure safe use. In addition, for products approved under accelerated approval, sponsors will be required to submit all copies of their promotional materials, including advertisements, to the FDA at least thirty days prior to initial dissemination unless otherwise informed by the FDA. After a hearing, the FDA may withdraw a previously granted accelerated approval if, for instance, post-marketing studies fail to verify any clinical benefit, it becomes clear that restrictions on the distribution of the product are inadequate to ensure its safe use, or if a sponsor fails to comply with the conditions of the accelerated approval.

- Breakthrough Therapy. The FDA may grant "breakthrough therapy" status to drugs or biologics designed to treat, alone or in combination with another drug(s) or biologic(s), a serious or life-threatening disease or condition and for which preliminary evidence suggests a substantial improvement on clinically-meaningful endpoints over existing therapies. Such products need not address an unmet need, but are nevertheless eligible for expedited review if they offer the potential for an improvement over existing therapies. Breakthrough therapy status entitles the sponsor to earlier and more frequent meetings with the FDA regarding the development of nonclinical and clinical data and permits the FDA to offer product development or regulatory advice for the purpose of shortening the potential time to product approval. Breakthrough therapy status does not guarantee that a product will be developed or reviewed more quickly and does not ensure FDA approval.

- Fast Track. The FDA may grant "fast track" status to drugs or biologics that treat serious diseases or illness and fill an unmet medical need. Fast track is a process designed to expedite the review of such products by providing, among other things, more frequent meetings with the FDA to discuss the product's development plan, more frequent written correspondence from the FDA about trial design, eligibility for accelerated approval if certain criteria are met, and rolling review, which allows submission of individually completed sections of a NDA or BLA for the FDA's review before the entire filing is completed. Fast track status does not ensure that a product will be developed more quickly or receive FDA approval more quickly, if at all.

- Priority Review. The FDA may grant "priority review" status to products that, if approved, would be significant improvements in safety or effectiveness of the treatment, diagnosis or prevention of serious conditions. Priority review is intended to reduce the time it takes for the FDA to review a NDA or BLA.

Additionally, there are various designations available to drugs and biologics which provide a sponsor with incentives to support approval of the product candidate, including, but is not limited to, orphan drug designation and rare pediatric disease designation.

Orphan Drug Designation

Under the U.S. Orphan Drug Act, as amended by the FDA Reauthorization Act of 2017, the FDA may grant orphan drug designation to drugs or biologics intended to treat a "rare disease or condition," which is defined as having a prevalence of less than 200,000 individuals in the United States. FDA is currently implementing a modernization plan which may include new requirements or procedures that could impact the success of an orphan drug designation request. In certain circumstances, a sponsor may need to demonstrate that the product is clinically superior to a previously-approved drug in order to obtain orphan drug status, and FDA may issue regulations to implement this requirement. These regulations will also affect Rare Pediatric Disease Designation Requests, which were previously exempted from the clinical trial requirements of the Pediatric Research Equity Act; FDA may now require clinical studies in pediatric populations for these requests to obtain orphan drug designation. Orphan drug designation must be requested before submitting a NDA or BLA for the product. The FDA aims to respond to all orphan drug designation requests within 90 days of submission. Orphan drug designation does not shorten the regulatory review and approval process, nor does it provide any advantage in the regulatory review and approval process. However, if an orphan drug later receives approval for the indication for which it has designation, the relevant regulatory authority may not approve any other applications to market the same drug for the same indication, except in very limited circumstances, for seven years in the United States. Although obtaining approval to market a product with orphan drug exclusivity may be advantageous, we cannot be certain:

- that we will be the first to obtain approval for any drug for which we obtain orphan drug designation;

- that orphan drug designation will result in any commercial advantage or reduce competition; or

- that the limited exceptions to this exclusivity will not be invoked by the relevant regulatory authority.

Additionally, orphan drug exclusive marketing rights may be lost under certain conditions, such as if the request for designation was materially defective or if the manufacturer is unable to assure sufficient quantity of the drug.

Ongoing FDA Requirements and Post-Marketing Obligations

The Food and Drug Administration Amendments Act of 2007 expanded FDA authority over drug products after approval. All approved drug products are subject to continuing regulation by the FDA, including record-keeping requirements, reporting of adverse experiences with the product, sampling and distribution requirements, notifying the FDA and gaining its approval of certain manufacturing or labeling changes, complying with certain electronic records and signature requirements, submitting periodic reports to the FDA, maintaining and providing updated safety and efficacy information to the FDA, and complying with FDA promotion and advertising requirements. Failure to comply with the statutory and regulatory requirements can subject a manufacturer to possible legal or regulatory action, such as warning letters, suspension of manufacturing, seizure of product, injunctive action, criminal prosecution, or civil penalties.

The FDA may require post-marketing studies or clinical trials to develop additional information regarding the safety of a product. These studies or trials may involve continued testing of a product and development of data, including clinical data, about the product's effects in various populations and any side effects associated with long-term use. The FDA may require post-marketing studies or trials to investigate possible or known serious risks or signals of serious risks, or to identify unexpected serious risks, and may require periodic status reports if new safety information develops. Failure to conduct these studies in a timely manner may result in substantial civil fines, or withdrawal of product approval.

Also, newly discovered or developed safety or efficacy data may require changes to a product's approved labeling, including the addition of new warnings and contraindications, additional pre-clinical studies or clinical trials, or even in some instances, withdrawal of the approval. Violations of regulatory requirements at any stage, including after approval, may result in various adverse consequences, including the FDA's withdrawal of an approved product from the market, other voluntary or FDA-initiated action that could delay or restrict further marketing, and the imposition of civil fines and criminal penalties against the manufacturer and NDA or BLA holder. In addition, later discovery of previously unknown problems may result in restrictions on the product, manufacturer or NDA or BLA holder, including withdrawal of the product from the market.

The labeling, advertising, promotion, marketing and distribution of a drug or biologic product also must be in compliance with FDA requirements which include, among others, promotional activities, standards and regulations for direct-to-consumer advertising, promotional activities involving the internet, and industry sponsored scientific and educational activities. In general, all product promotion must be consistent with the labeling approved by the FDA for such product, contain a balanced presentation of information on the product's uses, benefits, risks, and important safety information and limitations on use, and otherwise not be false or misleading. The FDA has very broad enforcement authority, and failure to abide by these regulations can result in penalties, including the issuance of a warning letter directing a company to correct deviations from regulatory standards and enforcement actions that can include seizures, injunctions and criminal prosecution. Failure to comply with applicable FDA requirements and restrictions also may subject a company to adverse publicity and enforcement action by the FDA, the U.S. Department of Justice, or DOJ, or the Office of the Inspector General of the U.S. Department of Health and Human Services, or HHS, as well as state authorities. This could subject the company to a range of penalties that could have a significant commercial impact, including civil and criminal fines and agreements that materially restrict the manner in which a company promotes or distributes its products.

Drug and biologic manufacturers and their subcontractors are required to register their establishments with the FDA and certain state agencies, and to list their products with the FDA. The FDA periodically inspects manufacturing facilities in the United States and abroad in order to assure compliance with the applicable cGMP regulations and other requirements. Facilities also are subject to inspections by other federal, foreign, state or local agencies. In complying with the cGMP regulations, manufacturers must continue to assure that the product meets applicable specifications, regulations and other post-marketing requirements. Failure to comply with these requirements subjects the manufacturer to possible legal or regulatory action, such as suspension of manufacturing or recall or seizure of product.

Sponsors and their third-party contractors are also subject to various laws and regulations governing laboratory practices, the experimental use of animals and the use and disposal of hazardous or potentially hazardous substances in connection with their research. In each of the above areas, the FDA has broad regulatory and enforcement powers, including the ability to levy fines and civil penalties, suspend or delay issuance of approvals, seize or recall products and deny or withdraw approvals.

Furthermore, new government requirements may be established that could delay or prevent regulatory approval of our products under development, or affect the conditions under which approved products are marketed.

Potential Competition with "Biosimilar" Products

The Biologics Price Competition and Innovation Act, or BPCIA, was enacted as part of the Patient Protection and Affordable Care Act of 2010, or the ACA, Pub. L. No. 111-148 (2010). The BPCIA authorizes the FDA to approve "abbreviated" BLAs for products whose sponsors demonstrate they are "biosimilar" to reference products previously approved under BLAs. The FDA may also separately determine whether "biosimilar" products are "interchangeable" with their reference products. However, the FDA may not approve an "abbreviated" BLA for a biosimilar product until at least twelve years after the date on which the BLA for the reference product was approved. FDA approval could be further delayed if the reference products are subject to unexpired and otherwise valid patents.

Prior to the enactment of the BPCIA, information in approved BLAs could not be relied upon by other manufacturers to establish the safety and efficacy of their products for which they were seeking FDA approval. (In contrast, since at least 1984, pharmaceutical manufacturers have been able to submit Abbreviated New Drug Applications for "generic drugs" that are materially identical to reference drugs approved under NDAs.) Accordingly, if our products are approved under a BLA, other manufacturers potentially could develop and seek FDA approval of "biosimilar" products at some point in the future.

In Vitro Companion Diagnostics

FDA defines an In Vitro, or IVD, companion diagnostic device is as an in vitro diagnostic device that provides information that is essential for the safe and effective use of a corresponding therapeutic product. The use of an IVD companion diagnostic device with a therapeutic product is stipulated in the instructions for use in the labeling of both the diagnostic device and the corresponding therapeutic product, including the label. Such tests include genetic diagnostic tests. Approval of such of treatment with the therapeutic product may be dependent on the approval of an IVD to:

- Monitor response to treatment with the therapeutic product for the purpose of adjusting treatment (e.g., schedule, dose, discontinuation) to achieve improved safety or effectiveness; and/or

- Identify patients in the population for whom the therapeutic product has been adequately studied and found safe and effective, i.e., there is insufficient information about the safety and effectiveness of the therapeutic product in any other population.

Applications for an IVD companion diagnostic device and its corresponding therapeutic product will be reviewed and approved according to applicable regulatory requirements. The IVD companion diagnostic device application will be reviewed and approved or cleared under the device authorities of the Federal Food, Drug, and Cosmetic Act (FD&C Act) and relevant medical device regulations; the therapeutic product application will be reviewed and approved under section 505 of the FD&C Act (i.e., drug products) or section 351 of the Public Health Service Act (i.e., biological products) and relevant drug and biological product regulations. FDA intends to review each IVD companion diagnostic device submission within the context of, or in conjunction with, its corresponding therapeutic product, and FDA review of the IVD companion diagnostic device and the therapeutic product will be carried out collaboratively among relevant FDA offices.

Ideally, a therapeutic product and its corresponding IVD companion diagnostic device should be developed contemporaneously, with the clinical performance and clinical significance of the IVD companion diagnostic device established using data from the clinical development program of the corresponding therapeutic product. Many of our current and future product development candidates, including AEVI-001 and AEVI-002, will depend upon co-development of accurate genetic and potentially other IVDs. Thus, we will likely need to comply with both FDA drug and medical device regulations. This adds additional cost and complexity to our development programs. The availability of IVD companion diagnostics can allow more efficient development programs and more appropriate use of products in the marketplace with more predictable outcomes for patients and higher value medicines.

Ultimately FDA approval of the IVD will be required to allow approval of many of our products. However, technical difficulties or other issues could delay or disrupt the development of our products.

HIPAA Requirements

Other federal legislation may affect our ability to obtain certain health information in conjunction with our research activities. We may be subject to data privacy and security regulation by both the federal government and the states in which we conduct our business. The Health Insurance Portability and Accountability Act of 1996, or HIPAA, as amended by the Health Information Technology for Economic and Clinical Health Act of 2009, or HITECH, and its implementing regulations, imposes requirements relating to the privacy, security and transmission of individually identifiable health information. Among other things, HITECH makes HIPAA's privacy and security standards directly applicable to "business associates"— independent contractors or agents of covered entities that receive or obtain protected health information in connection with providing a service on behalf of a covered entity. HITECH also increased the civil and criminal penalties that may be imposed against covered entities, business associates and possibly other persons, and gave state attorneys general new authority to file civil actions for damages or injunctions in federal courts to enforce the federal HIPAA laws and seek attorney's fees and costs associated with pursuing federal civil actions. The 21st Century Cares Act, Pub. L. 114-255, signed into law on December 13, 2016, among other changes, directs HHS to issue new HIPAA guidance which might differ from current regulations. In addition, state laws govern the privacy and security of health information in specified circumstances, many of which differ from each other in significant ways and may not have the same effect, thus complicating compliance efforts.

Other U.S. Regulatory Requirements

In the United States, the research, manufacturing, distribution, sale, and promotion of drug and biologic products are potentially subject to regulation by various federal, state and local authorities in addition to the FDA, including the Centers for Medicare and Medicaid Services (formerly the Health Care Financing Administration), other divisions of the HHS (e.g., the Office of Inspector General), the DOJ and individual U.S. Attorney offices within the DOJ, and state and local governments. For example, sales, marketing and scientific/educational grant programs must comply with the anti-fraud and abuse provisions of the Social Security Act, the False Claims Act, and similar state laws, each as amended.

If a drug or biologic product is reimbursed by Medicare or Medicaid, pricing and rebate programs must comply with, as applicable, the Medicare Modernization Act as well as the Medicaid rebate requirements of the Omnibus Budget Reconciliation Act of 1990, or OBRA, and the Veterans Health Care Act of 1992, or VHCA, each as amended. Among other things, the OBRA imposes certain reporting requirements on pharmaceutical manufacturers and requires pharmaceutical manufacturers to pay rebates on prescription products to state Medicaid programs and empowers states to negotiate rebates on pharmaceutical prices, which may result in prices for our future products that will likely be lower than the prices we might otherwise obtain. If products are made available to authorized users of the Federal Supply Schedule of the General Services Administration, additional laws and requirements apply. Under the VHCA, drug companies are required to offer some products at a reduced price to a number of federal agencies including the U.S. Department of Veterans Affairs and the U.S. Department of Defense, the Public Health Service and some private Public Health Service designated entities in order to participate in other federal funding programs including Medicaid. Participation under the VHCA requires submission of pricing data and calculation of discounts and rebates pursuant to complex statutory formulas, as well as the entry into government procurement contracts governed by the Federal Acquisition Regulation. All of these activities are also potentially subject to federal and state consumer protection, unfair competition, and other laws.

In March 2010, President Obama signed the Affordable Care Act of 2010, or the ACA. The ACA substantially changes the way healthcare will be financed by both governmental and private insurers, and significantly impacts the pharmaceutical industry. The ACA was a sweeping law intended to broaden access to health insurance, reduce or constrain the growth of healthcare spending, enhance remedies against fraud and abuse, add new transparency requirements for health care and health insurance industries, impose new taxes and fees on the health industry and impose additional health policy reforms. The ACA has resulted in downward pressure on coverage and the price of products covered by Medicare and other government programs. Any reduction in reimbursement from Medicare and other government programs may result in a similar reduction in payments and coverage from private payors. The implementation of cost containment measures or other healthcare reforms may prevent us from being able to generate revenue, attain profitability, or commercialize our products. In addition, it is possible that there will be further legislation or regulation that could harm our business, financial condition and results of operations.

The federal Anti-Kickback Statute prohibits, among other things, knowingly and willfully offering, paying, soliciting or receiving remuneration to induce or in return for purchasing, leasing, ordering or arranging for the purchase, lease or order of any healthcare item or service reimbursable under Medicare, Medicaid or other federally financed healthcare programs. This statute has been interpreted to apply to arrangements between pharmaceutical manufacturers on one hand and prescribers, purchasers, and formulary managers on the other. Although there are a number of statutory exemptions and regulatory safe harbors protecting some business arrangements from prosecution, the exemptions and safe harbors are drawn narrowly and practices that involve remuneration intended to induce prescribing, purchasing or recommending may be subject to scrutiny if they do not qualify for an exemption or safe harbor. Our practices may not in all cases meet all of the criteria for safe harbor protection from federal Anti-Kickback Statute liability. The reach of the Anti-Kickback Statute was broadened by the ACA, which, among other things, amends the intent requirement of the federal Anti-Kickback Statute.

Pursuant to the statutory amendment, a person or entity no longer needs to have actual knowledge of this statute or specific intent to violate it in order to have committed a violation. In addition, the ACA provides that the government may assert that a claim including items or services resulting from a violation of the federal Anti-Kickback Statute constitutes a false or fraudulent claim for purposes of the civil False Claims Act (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.

The federal False Claims Act prohibits any person from knowingly presenting, or causing to be presented, a false claim for payment to the federal government or knowingly making, using, or causing to be made or used a false record or statement material to a false or fraudulent claim to the federal government. As a result of a modification made by the Fraud Enforcement and Recovery Act of 2009, a claim includes “any request or demand” for money or property presented to the U.S. government. Recently, several pharmaceutical and other healthcare 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 product. Other companies have been prosecuted for causing false claims to be submitted because of the companies’ marketing of the product for unapproved, and thus non-reimbursable, uses. HIPAA created new federal criminal statutes that prohibit knowingly and willfully executing a scheme to defraud any healthcare benefit program, including private third-party payors and 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 healthcare benefits, items or services. Also, many states have similar fraud and abuse statutes or regulations that apply to items and services reimbursed under Medicaid and other state programs, or, in several states, apply regardless of the payor.

Foreign Regulatory Requirements

We may be subject to widely varying foreign regulations, which may be quite different from those of the FDA, governing clinical trials, manufacturing, product registration and approval, and pharmaceutical sales.

Whether or not FDA approval has been obtained, we must obtain a separate approval for a product by the comparable regulatory authorities of foreign countries prior to the commencement of product marketing in these countries. In certain countries, regulatory authorities also establish pricing and reimbursement criteria. The approval process varies from country to country, and the time may be longer or shorter than that required for FDA approval.

In addition, pharmaceutical products may not be imported into, or manufactured or marketed in, the State of Israel absent drug registration or the appropriate license/approval to import/manufacture for clinical trials use.

Reimbursement and Pricing Controls

Third-party payers (Medicare, Medicaid, private health insurance companies and other organizations) may affect the pricing or relative attractiveness of our product candidates by regulating the level of reimbursement provided to the physicians and clinic utilizing our product candidates or by refusing reimbursement. If reimbursement under these programs, or if the amount of time to secure reimbursement is too long, our ability to market our technology and product candidates may be adversely and materially affected. In international markets, reimbursement by private third-party medical insurance providers, including government insurers and independent providers, varies from country to country. In certain countries, our ability to achieve significant market penetration may depend upon the availability of third-party government reimbursement.

In many of the markets where we or our collaborative partners would commercialize a product following regulatory approval, the prices of pharmaceutical products are subject, by law, to direct price controls and to drug reimbursement programs with varying price control mechanisms. Public and private health care payers control costs and influence drug pricing through a variety of mechanisms, including the setting of reimbursement amounts for drugs and biological products covered by Medicare Part B based on their Average Sales Prices calculated by manufacturers in accordance with the Medicare Prescription Drug, Improvement, and Modernization Act of 2010, Pub. L. No. 108-173 (2003), as amended, through negotiating discounts with the manufacturers, and through the use of tiered formularies and other mechanisms that provide preferential access to certain drugs over others within a therapeutic class. Drug manufacturers also may be subject to drug rebate agreements with public or private health care payers in exchange for the manufacturers' products being included on plan formularies.

Payers also set other criteria to govern the uses of a drug that will be deemed medically appropriate and therefore reimbursed or otherwise covered. If a payer concludes that a drug is experimental or investigational, in many cases it will deny coverage on that basis alone. Further, many public and private health care payers limit reimbursement and coverage to the uses of a drug that are either approved by the FDA or that are supported by other appropriate evidence (for example, published medical literature) and appear in a recognized drug compendium. Drug compendia are publications that summarize the available medical evidence for particular drug products and identify which uses of a drug are supported or not supported by the available evidence, whether or not such uses have been approved by the FDA. For example, in the case of Medicare coverage for physician-administered oncology drugs, the Omnibus Budget Reconciliation Act of 1993, with certain exceptions, prohibits Medicare carriers from refusing to cover unapproved uses of an FDA-approved drug if the unapproved use is supported by one or more citations in the American Hospital Formulary Service Drug Information the American Medical Association Drug Evaluations, or the United States Pharmacopoeia Drug Information. Another commonly cited compendium, for example under Medicaid, is the DRUGDEX Information System.

Employees

We currently employ 17 full-time employees. None of our employees are represented by a labor union and we have not experienced any strikes or work stoppages. We generally provide our employees with benefits and working conditions beyond the required minimums. We believe our relations with our employees are good.

Additional Information

Aevi Genomic Medicine, Inc., a Delaware corporation was organized on January 27, 2000. Our principal executive offices are located at 435 Devon Park Drive, Suite 715, Wayne, Pennsylvania 19087. Our telephone number is (610) 254-4201.

Our website address is *www.aevigenomics.com*. The information on or accessible through our website is not part of this Annual Report on Form 10-K. Copies of our Annual Reports on Form 10-K, Quarterly Reports on Form 10-Q, Current Reports on Form 8-K and amendments to such reports are available without charge on our website or upon request to us. In addition, our Code of Business Conduct and Ethics, Audit Committee Charter, Compensation Committee Charter and Nominating and Corporate Governance Committee Charter are all available without charge on our website or upon request to us. All such requests should be sent to Aevi Genomic Medicine, Inc., Corporate Secretary, 435 Devon Park Drive, Suite 715, Wayne, Pennsylvania 19087, or by email request from our website at *www.aevigenomics.com*. Amendments to, or waivers from, our Code of Business Conduct and Ethics that apply to our executive officers will be posted to our website. We also post or otherwise make available on our website from time to time other information that may be of interest to our investors.

ITEM 1A - Risk Factors.

Business-Related Risks

Our recurring losses from operations raise substantial doubt regarding our ability to continue as a going concern.

Our recurring losses from operations raise substantial doubt about our ability to continue as a going concern. There is no assurance that sufficient financing will be available when needed to allow us to continue as a going concern. The perception that we may not be able to continue as a going concern may cause others to choose not to deal with us due to concerns about our ability to meet our contractual obligations.

We are a clinical stage biopharmaceutical company and have a history of significant and continued operating losses and a substantial accumulated earnings deficit and we may continue to incur significant losses and may never achieve or maintain profitability.

We are a clinical stage biopharmaceutical company and since our inception have been focused on research and development and have not generated any substantial revenues. We have incurred net losses of approximately $34.71 million, $41.90 million and $37.99 million for the years ended December 31, 2017, 2016 and 2015, respectively. As of December 31, 2017, we had stockholders' equity of approximately $30.62 million. We expect to incur significant expenses and increasing operating losses, as well as negative cash flow from operations, for the foreseeable future, as we continue to expand our research and development and commence commercialization of our potential product candidates. The net losses we incur may fluctuate significantly from quarter to quarter and year to year. It could be several years, if ever, before we have a commercialized product. Our ability to generate revenues from sales of our potential products will depend on:

- successful completion of necessary clinical trials;
- regulatory approval;
- commercialization (through partnership or licensing deals or through internal development) and market acceptance of new technologies and product candidates under development;
- medical community awareness; and
- changes in regulation or regulatory policy.

We will need substantial additional capital for the continued development of our product candidates and for our long-term operations.

As of December 31, 2017, our cash and cash equivalents were approximately $33.73 million. We believe our existing cash and cash equivalents should be sufficient to meet our operating and capital requirements into the first quarter of 2019. However, changes in our business, whether or not initiated by us, may affect the rate at which we deplete our cash and cash equivalents. Our present and future capital requirements depend on many factors, including:

- the rate and level of patient recruitment into our clinical trials, particularly those in Phase 2 and Phase 3 stages of development, including those trials for which we are currently recruiting; for example, the identification and recruitment of patients into the AEVI-002 proof-of-concept study has been challenging. The ability to produce initial data by year-end 2018 is highly dependent on timely recruiting; thus, continued difficulties in recruitment could cause a delay in the delivery of data for the program, and potentially result in increased costs to complete the study;
- the level of research and development investment required to develop our product candidates;

- changes in product development plans needed to address any difficulties that may arise in manufacturing, pre-clinical activities, clinical trials or commercialization;
- our ability and willingness to enter into new agreements with strategic partners, and the terms of these agreements;
- our success rate in pre-clinical and clinical efforts;
- the costs of future commercialization activities, including product sales, marketing, manufacturing and distribution, for any of our product candidates for which we receive marketing approval;
- revenue, if any, received from commercial sales of our product candidates, should any of our product candidates receive marketing approval;
- costs of recruiting and retaining qualified personnel;
- the timing and amount of milestone payments we are required to make under our license agreements;
- time and costs involved in obtaining regulatory approvals; and
- costs of filing, prosecuting, defending, and enforcing patent claims and other intellectual property rights.

We will require significant amounts of additional capital in the future, and such capital may not be available when we need it on terms that we find favorable, if at all. Identifying potential product candidates and conducting preclinical testing and clinical trials is a time-consuming, expensive and uncertain process that takes years to complete. We may never progress to the point where we have commercially successful product sales which generate sufficient commercial revenue or such revenue may not be achieved for many years. Accordingly, we may seek to raise these funds through public or private equity offerings, debt financings, credit facilities, or partnering or other corporate collaborations and licensing arrangements. If adequate funds are not available or are not available on acceptable terms, our ability to fund our operations, take advantage of opportunities, develop products and technologies, and otherwise respond to competitive pressures could be significantly delayed or limited, and we may need to downsize or halt our operations.

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

Until such time, if ever, as we can generate substantial product revenues, we expect to finance our cash needs through a combination of equity offerings and debt financings. We do not have any committed external source of funds. We may seek additional capital through a combination of private and public equity offerings, debt financings, collaborations and strategic and licensing arrangements. To the extent that we raise additional capital through the sale of common stock or securities convertible or exchangeable into common stock, the ownership interest of our stockholders in our company will be diluted. In addition, the terms of any such securities may include liquidation or other preferences that materially adversely affect the rights of our stockholders. Debt financing, if available, would increase our fixed payment obligations and 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.

We cannot be certain that additional funding will be available on acceptable terms, or at all. If we raise additional funds through collaboration, strategic partnerships and licensing arrangements with third parties, we may have to relinquish valuable rights to our product candidates, our intellectual property, future revenue streams or grant licenses on terms that are not favorable to us.

We are still in the process of clinical trials and do not have a commercialized product and may never be able to commercialize our product candidates.

Only a small number of research and development programs ultimately result in commercially successful drugs and drug delivery systems. Potential products that appear to be promising at early stages of development may not reach the market for a number of reasons, including:

- failure to obtain regulatory approvals for AEVI-001, AEVI-002 or any of our product candidates or companion products;
- lack of familiarity of health care providers and patients;
- low market acceptance as a result of lower demonstrated clinical safety or efficacy compared to other products or other potential disadvantages relative to alternative treatment methods;
- inability to obtain favorable coverage determinations from health plans and third-party payers;
- insufficient or unfavorable levels of reimbursement from government or third-party payers;
- infringement on proprietary rights of others for which we (or our licensees, if any) have not received licenses;
- incompatibility with other therapeutic products;
- potential advantages of alternative treatment methods;
- ineffective marketing and distribution support;
- lack of cost-effectiveness; or
- timing of market introduction of competitive products.

If any of these potential problems occur, we may never successfully commercialize our product candidates, including AEVI-001 and AEVI-002. If we are unable to develop commercially viable products, our business, results of operations and financial condition will be materially and adversely affected.

We have limited history as an organization in conducting clinical trials.

We have limited history as an organization in conducting advanced clinical trials and may not possess the necessary resources and expertise to complete such trials, and we may need to seek additional partnerships or collaborations with third parties to advance these trials. Our most advanced clinical program is an ongoing Phase 2 trial in mGluR+ Genetic Subset ADHD to confirm genetic responders to AEVI-001. For potential marketing application approval, additional clinical testing will be required, which involves significantly greater resources, commitments and expertise and so it is likely that we would need to enter into a collaborative relationship with a pharmaceutical company that could assume responsibility for late-stage development and commercialization.

Our product candidates are still being developed and have not been tested on a large patient population, and, therefore, we do not know all of the possible adverse events and may not be able to commercialize our product candidates as planned.

Our product candidates have not been tested on a large number of patients, and are still in an early stage of development. While we have attained acceptable adverse event profile (or safety results) in our early stages of development and early clinical trials for AEVI-001, our product candidates are not yet fully developed or proven, and disappointing results and problems could delay or prevent the completion of our development programs and commercialization of our product candidates.

Our previous safety tests and results obtained in previous clinical trials of our product candidates may not be representative of either a larger multi-centric test or the commercial version of the technology in the general population. Specifically, the Phase 1b clinical trial for AEVI-001 completed prior to our acquisition of neuroFix was conducted on a single-blinded basis and may have been subject to bias and such results may not be replicated in a double-blinded clinical trial. In addition, the full impact of our product candidates, and their many possible variations, on the body is, as yet, unknown.

Treatment-related adverse events or complications in clinical trials, or post-approval, could result in limitations on the use of our product candidates and may also result in financial claims and losses against us, damage our reputation, and increase our expenses and reduce our assets. In addition, our product candidates may not gain commercial acceptance or ever be commercialized.

We are currently dependent upon the successful development of our lead product candidates, AEVI-001 and AEVI-002. If we or our strategic partners, licensees and sublicensees fail to successfully complete their development and commercialization, we will not generate operating revenues.

A substantial portion of our efforts and expenses are currently focused on the development of AEVI-001 and AEVI-002. Our ability to generate product revenues, which we do not expect will occur for many years, if ever, will depend heavily on the successful development and eventual commercialization of AEVI-001 and AEVI-002. There is no guarantee that we will succeed in developing AEVI-001 or AEVI-002. If the development of both AEVI-001 and AEVI-002 fails, we may be unable to generate any revenues. There is no certainty as to our success, whether within a given time frame or at all. Any delays in our schedule for clinical trials, regulatory approvals or other stages in the development of our technology are likely to cause us additional expense and may even prevent the successful commercialization of any or all of our product candidates. Delays in the timing for development of our technology may also have a material adverse effect on our business, financial condition and results of operations due to the possible absence of financing sources for our operations during such additional periods of time. Although we may pursue other technologies (either developed in-house or acquired), there is no assurance that any other technology will be successfully identified or exploited.

Clinical trials involve lengthy and expensive processes with uncertain outcomes, and results of earlier studies and trials may not be predictive of future trial results.

The risk of failure of our product candidates is high. We cannot predict whether we will encounter problems with any of our completed, ongoing, planned or future clinical trials, which would cause us or regulatory authorities to delay or suspend clinical trials, or delay the analysis of data from completed or ongoing clinical trials. The FDA Reauthorization Act, signed into law in August 2017, authorizes FDA to impose additional clinical trial requirements on manufacturers seeking orphan drug designation and/or pediatric indications. The impact of these future regulations is uncertain and could result in the need for additional clinical trials. We estimate that clinical trials involving AEVI-001 and AEVI-002 will continue for several years; however, such trials may also take significantly longer to complete and may cost more money than we expect. Failure can occur at any stage of testing, and we may experience numerous unforeseen events during, or as a result of, the clinical trial process that could delay or prevent commercialization of the current, or a future, more advanced, version of our product candidates, including but not limited to:

- delays in obtaining regulatory approvals to commence a clinical trial;
- failure or inability to recruit qualified investigators;
- difficulty finding qualified patients for clinical studies, including slower than anticipated patient recruitment and enrollment;
- negative or inconclusive results from clinical trials;

- inability, delay, or failure in identifying and maintaining a sufficient number of trial sites, many of which may already be engaged in other clinical programs;
- lack of adequate funding to continue the clinical trial, including the incurrence of unforeseen costs due to enrollment delays, requirements to conduct additional clinical studies and increased expenses associated with the services of our clinical research organizations, or CROs, and other third parties;
- clinical trials of our product candidates may produce negative or inconclusive results, and we may decide, or regulators may require us, to conduct additional clinical trials or abandon product development programs;
- our third-party contractors may fail to comply with regulatory requirements or meet their contractual obligations to us in a timely manner, or at all;
- there may be changes in governmental regulations or administrative actions;
- unforeseen safety issues;
- an inability to monitor patients adequately during or after treatment; and
- problems with investigator or patient compliance with the trial protocols.

A number of companies in the biopharmaceutical and pharmaceutical industries including those with greater resources and experience than us have suffered significant setbacks in advanced clinical trials, even after seeing promising results in earlier clinical trials. We do not know whether any clinical trials we or any future clinical partners may conduct will demonstrate adequate efficacy and safety to result in regulatory approval to market AEVI-001, AEVI-002 or any other product. If subsequent clinical trials involving AEVI-001 or AEVI-002 do not produce favorable results, we may be required to perform additional clinical trials or our ability to obtain regulatory approval may be adversely impacted, either of which would have an adverse material effect on our business, financial condition and the results of our operations.

Potential difficulty with, and delays in, recruiting patients for human clinical trials may adversely affect the timing of our clinical trials and our working capital requirements.

Our research and development is highly dependent on timely recruitment of the requisite number and type of patients for our clinical trials. We have previously found it very difficult to recruit such patients, and the increased volume and ethnic backgrounds required for future testing may render such testing even more difficult. Such larger studies will likely be based on the use of multicenter, multinational design, which can prove difficult to manage and could result in delays in patient recruitment. In addition, as we pursue development of our product candidates in orphan and rare disease applications, including for pediatric populations, we may find it difficult to find sufficient treatment-naïve patients needed for initial trials, especially within commercially-reasonable geographical regions. Delays in the recruitment of such patients could delay our trials and negatively impact our working capital requirements and ability to raise capital.

We may not successfully establish and maintain relationships with third-party service providers and collaborators, which could adversely affect our ability to develop, manufacture and commercialize our product candidates.

Our ability to develop and commercialize our product candidates is dependent on our ability to reach strategic licensing and other development agreements with appropriate partners, including biopharmaceutical and pharmaceutical companies and CROs. If we are unable to successfully negotiate such agreements, we may not be able to continue to develop our product candidates, including AEVI-001 and AEVI-002, without raising significant additional capital for development and commercialization.

Our core business strategy is to develop our product candidates for use in specific indications and disease markets that we would internally develop and launch. However, we do plan to explore collaborative relationships or strategic partnerships and/or license our product candidates. We may not be able to identify such collaborators and partners on a timely basis, and we may not be able to enter into relationships with any future collaborator(s) or partner(s) on terms that are commercially beneficial to us or at all. In addition, such relationships and partnerships may not come to fruition or may not be successful. Our agreements with these third parties may also contain provisions that restrict our ability to develop and test our product candidates or that give third parties rights to control aspects of our product development and clinical programs.

The third-party contractors may not assign as great of a priority to our clinical development programs or pursue them as diligently as we would if we were undertaking such programs directly and, accordingly, may not complete activities on schedule, or may not conduct the studies or our clinical trials in accordance with regulatory requirements or with our trial design. If these third parties do not successfully carry out their contractual duties or meet expected deadlines, or if their performance is substandard, we may be required to replace them.

In addition, conflicts may arise with our collaborators (e.g. those concerning the interpretation of clinical data), the achievement of milestones, the interpretation of financial provisions or the ownership of intellectual property developed during the collaboration. If any conflicts arise with our existing or future collaborators, they may act in their self-interest, which may be adverse to our best interests. The third-party contractors may also have relationships with other commercial entities, some of whom may compete with us. If the third-party contractors work with our competitors, our competitive position may be harmed.

In addition, although we attempt to audit and control the quality of third-party data, we cannot guarantee the authenticity or accuracy of such data, nor can we be certain that such data has not been fraudulently generated. The failure of third parties to carry out their obligations towards us would materially adversely affect our ability to develop and market product candidates.

We have no marketing experience, sales force or distribution capabilities. If our product candidates are approved, and we are unable to recruit key personnel to perform these functions, we may not be able to successfully commercialize the products.

Although we do not currently have any marketable products, our ability to produce revenues ultimately depends on our ability to commercialize our product candidates if and when they are approved by the FDA and/or other regulatory health agencies. We currently do not have a marketing and sales staff or distribution capabilities. Developing a marketing and sales force is also time-consuming and expensive and these costs may be incurred in advance of any approval of our product candidates. Failure to develop these capabilities could delay the launch of new products or expansion of existing product sales. In addition, we will compete with many companies that currently have extensive and well-funded marketing, sales and distribution operations. If we fail to establish successful marketing, sales and distribution capabilities or fail to enter into successful marketing sales or distribution arrangements with third parties, our ability to generate revenues will suffer.

Furthermore, even if we enter into marketing, sales and distributing arrangements with third parties, these third parties may not be successful or effective in marketing, selling or distributing our product candidates. If we fail to create successful and effective marketing, sales and distribution channels, our ability to generate revenue and achieve our anticipated growth could be adversely affected. If these distributors experience financial or other difficulties, sales of our products could be reduced, and our business, financial condition and results of operations could be harmed.

We are subject to intense government regulation and we may not be able to successfully complete the necessary clinical trials.

Approval for clinical trials depends, among other things, on data obtained from our pre-clinical and clinical activities, including completion of pre-clinical animal and *in vitro* studies in a timely manner. These pre-clinical and clinical activities must meet stringent quality assurance and compliance requirements. Data obtained from such activities are susceptible to varying interpretations, which could delay, limit or prevent regulatory approvals.

We currently have limited experience in and resources for conducting the large-scale clinical trials which may hamper our ability to obtain or comply with regulatory approval. The failure to comply with applicable regulatory requirements may result in criminal prosecution, civil penalties, product recalls, withdrawal of product approval, mandatory restrictions and other actions, which could impair our ability to conduct business.

Use of third parties to manufacture our product candidates or diagnostics may increase the risk that we will not have sufficient quantities of our product candidates or such quantities at an acceptable cost or that development of the diagnostics will be delayed. Clinical development and commercialization of our product candidates could be delayed, prevented or impaired.

We do not own or operate manufacturing facilities for production of our product candidates or diagnostics. We lack the resources and the capabilities to manufacture any of our product candidates or diagnostics on a clinical or commercial scale. We currently outsource the manufacturing and packaging of our pre-clinical and clinical product candidates to third parties and if we pursue a diagnostic product, we anticipate that we would outsource manufacturing to a third party. The manufacture of pharmaceutical products requires significant expertise and capital investment, including the development of advanced manufacturing techniques and process controls. Manufacturers of pharmaceutical products often encounter difficulties in production, particularly in scaling up initial production. These problems include difficulties with production costs and yields and quality control, including stability of the product candidate. The occurrence of any of these problems could significantly delay our clinical trials or the commercial availability of our products.

We do not currently have any agreements with third party manufacturers for the long-term commercial supply of any of our product candidates or agreements with any third party for development of diagnostics. We may be unable to enter into agreements for development and commercial supply with third party manufacturers or with a third party for development of diagnostics, or may be unable to do so on acceptable terms. Even if we enter into these agreements, the manufacturers of each product candidate and developer of diagnostics will likely be single source suppliers to us for a significant period of time.

Reliance on third party manufacturers entails risks, to which we would not be subject if we manufactured product candidates or products ourselves, including:

- reliance on the third party for regulatory compliance and quality assurance;
- limitations on supply availability resulting from capacity and scheduling constraints of the third parties;
- impact on our reputation in the marketplace if manufacturers of our products, once commercialized, fail to meet the demands of our customers;
- the quality or stability of the product candidates falling below acceptable standards;
- the inability to produce sufficient quantities of our product candidates;
- the timely development of the required diagnostics;
- exceeding budgeted costs due to difficulties in accurately predicting such costs or other factors impacting the cost of manufacturing our product candidates or developing diagnostics;
- the possible breach of the manufacturing agreement by the third party because of factors beyond our control; and
- the possible termination or non-renewal of the agreement by the third party, based on its own business priorities, at a time that is costly or inconvenient for us.

The failure of any of our contract manufacturers to maintain high manufacturing standards could result in injury or death of clinical trial participants or patients using products. Such failure could also result in product liability claims, product recalls, product seizures or withdrawals, delays or failures in testing or delivery, cost overruns or other problems that could seriously harm our business or profitability.

Our contract manufacturers are required to adhere to FDA regulations setting forth cGMP. These regulations cover all aspects of the manufacturing, testing, quality control and recordkeeping relating to our product candidates and any products that we may commercialize. Our manufacturers may not be able to comply with cGMP regulations or similar regulatory requirements outside the United States. Our failure or the failure of our third party manufacturers, to comply with applicable regulations could significantly and adversely affect regulatory approval and supplies of our product candidates.

Our product candidates and any products that we may develop or acquire may compete with other product candidates and products for access to manufacturing facilities. There are a limited number of manufacturers that operate under cGMP regulations and that are both capable of manufacturing for us and willing to do so. If the third parties that we engage to manufacture products for our pre-clinical tests and clinical trials should cease to continue to do so for any reason, we likely would experience delays in advancing these trials while we identify and qualify replacement suppliers and we may be unable to obtain replacement supplies on terms that are favorable to us. Later relocation to another manufacturer will also require notification, review and other regulatory approvals from the FDA and other regulators and will subject our production to further cost and instability in the availability of our product candidates. In addition, if we are not able to obtain adequate supplies of our product candidates or the drug substances used to manufacture them, it will be more difficult for us to develop our product candidates and compete effectively.

Our current and anticipated future dependence upon others for the manufacture of our product candidates may adversely affect our future profit margins and our ability to develop product candidates and commercialize any products that obtain regulatory approval on a timely and competitive basis.

Materials necessary to manufacture our product candidates may not be available on commercially reasonable terms, or at all, which may delay the development and commercialization of our product candidates.

We rely on the manufacturers of our product candidates to purchase from third party suppliers the materials necessary to produce the compounds for our preclinical and clinical studies and will rely on these other manufacturers for commercial distribution if we obtain marketing approval for any of our product candidates. Suppliers may not sell these materials to our manufacturers at the time we need them or on commercially reasonable terms and all such prices are susceptible to fluctuations in price and availability due to transportation costs, government regulations, price controls and changes in economic climate or other foreseen circumstances. We do not have any control over the process or timing of the acquisition of these materials by our manufacturers. Moreover, we currently do not have any agreements for the commercial production of these materials. If our manufacturers are unable to obtain these materials for our preclinical and clinical studies, product testing and potential regulatory approval of our product candidates would be delayed, significantly impacting our ability to develop our product candidates. If our manufacturers or we are unable to purchase these materials after regulatory approval has been obtained for our product candidates, the commercial launch of our product candidates would be delayed or there would be a shortage in supply, which would materially affect our ability to generate revenues from the sale of our product candidates.

We may not be successful in our efforts to in-license or acquire additional product candidates.

A significant element of our strategy is to build and expand our pipeline of product candidates through in-licensing or acquiring additional product candidates. Currently, we do not have the internal expertise, nor do we intend to develop the internal expertise, necessary to discover new chemical entities for therapeutic purposes. As a result, if we are not able to identify and acquire additional product candidates, we will not be able to expand our pipeline. Even if we are successful in continuing to build our pipeline through in-licensing or acquisitions, the potential product candidates that we in-license or acquire may not be suitable for clinical development, including as a result of being shown to have harmful side effects or other characteristics that indicate that they are unlikely to be drugs that will receive marketing approval and achieve market acceptance.

Our business activities involve the use of hazardous materials, which require compliance with environmental and occupational safety laws regulating the use of such materials. If we violate these laws, we could be subject to significant fines, liabilities or other adverse consequences.

Our research and development programs involve the controlled use of hazardous materials, including microbial agents and other hazardous compounds in addition to certain biological hazardous waste. Ultimately, the activities of our third party product manufacturers when a product candidate reaches commercialization will also require the use of hazardous materials. Accordingly, we are subject to federal, state and local laws governing the use, handling and disposal of these materials. Although we believe that our safety procedures for handling and disposing of these materials comply in all material respects with the standards prescribed by local, state and federal regulations, we cannot completely eliminate the risk of accidental contamination or injury from these materials. In addition, our collaborators may not comply with these laws. In the event of an accident or failure to comply with environmental laws, we could be held liable for damages that result, and any such liability could exceed our assets and resources or we could be subject to limitations or stoppages related to our use of these materials which may lead to an interruption of our business operations or those of our third-party contractors. While we believe that our existing insurance coverage is generally adequate for our normal handling of these hazardous materials, it may not be sufficient to cover pollution conditions or other extraordinary or unanticipated events. Furthermore, an accident could damage or force us to shut down our operations. Changes in environmental laws may impose costly compliance requirements on us or otherwise subject us to future liabilities and additional laws relating to the management, handling, generation, manufacture, transportation, storage, use and disposal of materials used in or generated by the manufacture of our products or related to our clinical trials. In addition, we cannot predict the effect that these potential requirements may have on us, our suppliers and contractors or our customers.

The FDA and other regulatory health agencies will regulate our product candidates and we may never receive regulatory approval to market and sell our product candidates.

Our product candidates will require regulatory approvals prior to sale. In particular, our product candidates are subject to stringent approval processes, prior to commercial marketing, by the FDA and other regulatory health agencies in all countries where we operate and desire to introduce our product candidates, whether sold via a strategic partner or directly by us. These requirements range from efficacy and safety assessments in multiple clinical trials to long-term follow-up assessments on treated patients in clinical trials for product approval for sale. The process of obtaining FDA and corresponding foreign approvals is costly and time-consuming, and we cannot assure that such approvals will be granted. Also, the regulations we are subject to change frequently and such changes could cause delays in the development of our product candidates.

It typically takes a company several years or longer to satisfy the substantial requirements imposed by the FDA and other regulatory health agencies in other countries for the introduction of therapeutic pharmaceutical and biological products. Pharmaceutical or biological products must be registered in accordance with applicable law before they can be manufactured, marketed and distributed. This registration must include medical data proving the product's safety, efficacy and clinical testing. Also included in product registration should be references to medical publications and information about the production methods and quality control.

To obtain regulatory approvals in the United States or other jurisdictions, we or a collaborator must ultimately demonstrate to the satisfaction of the FDA and other health regulatory agencies that our product candidates are sufficiently safe and effective for their proposed administration to humans. Many factors, both known and unknown, can adversely impact the development of our product candidates and our ability to obtain regulatory approval for our product candidates, including:

- the FDA or other health regulatory authorities or instructional review boards decision(s) not to approve a clinical trial protocol or place a clinical trial on hold;

- suitable patients not enrolling in a clinical trial in sufficient numbers or at the expected rate, for reasons such as the size of the prospective patient population, the proximity of patients to clinical sites, the eligibility criteria for the trial, the perceptions of investigators and patients regarding safety, and the availability of other treatment options;

- clinical trial data being adversely affected by trial conduct or patient withdrawal prior to completion of the trial;
- competition with ongoing clinical trials and scheduling conflicts with participating clinicians;
- patients that experience adverse events, including treatment-related adverse events of our product candidates, for a variety of reasons that may or may not be related to our product candidates, including the advanced stage of their disease and other medical problems;
- patients in the placebo or untreated control group exhibiting greater than expected improvements or fewer than expected adverse events;
- third-party clinical investigators not performing the clinical trials on the anticipated schedule or consistently with the clinical trial protocol and GCP, or other third-party organizations not performing data collection and analysis in a timely or accurate manner;
- service providers, collaborators or co-sponsors not adequately performing their obligations in relation to the clinical trial or cause the trial to be delayed or terminated;
- being unable to obtain a sufficient supply of manufactured clinical trial materials;
- regulatory inspections of manufacturing facilities requiring us or a co-sponsor to undertake corrective action or suspend the clinical trials;
- interim results of the clinical trial being inconclusive or negative;
- clinical trials, although approved and completed, generating data that are not considered by the FDA or other health regulatory agencies to be sufficient to demonstrate safety and efficacy;
- clinical trials, although approved and completed outside the United States, not considered by the FDA or others outside the jurisdiction hosting such clinical trials to be sufficient to demonstrate safety and efficacy; and
- changes in governmental regulations or administrative actions affecting the conduct of the clinical trial or the interpretation of its results.

There can be no assurance that our clinical trials will in fact demonstrate, to the satisfaction of the FDA and others, that our product candidates are sufficiently safe or effective. The FDA or we may also restrict or suspend our clinical trials at any time if either believes that we are exposing the subjects participating in the trials to unacceptable health risks.

Delays in obtaining such clearances and/or changes in existing requirements could have a material adverse effect on our company by making it difficult to advance product candidates or by reducing or eliminating their potential or perceived value and, therefore, our ability to conduct our business as currently planned could materially suffer. Failure to obtain required regulatory approvals could require us to delay, curtail or cease our operations. Even if we invest the necessary time, money and resources required to advance through the FDA approval process, there is no guarantee that we will receive FDA approval of our product candidates.

Our failure to comply with applicable regulatory requirements could result in enforcement action by the FDA or other regulatory health agencies, which may include any of the following sanctions:

- warning letters, fines, injunctions, consent decrees and civil penalties;
- repairs, replacements, refunds, recalls, or seizures of our products;

- operating restrictions, partial suspension, or total shutdown of production;
- refusing our requests for regulatory clearance or premarket approval of new products, new intended uses, or modifications to existing products;
- withdrawing regulatory clearance or premarket approvals that have already been granted; and
- criminal prosecution.

If any of these events were to occur, it could adversely affect our business, financial condition and results of operations.

Even if we obtain regulatory approvals, our products will be subject to ongoing regulatory review and if we fail to comply with continuing regulations, we could lose those approvals and our business, financial condition and results of operations would be seriously harmed.

Even if our product candidates receive initial regulatory approval or clearance for specific therapeutic applications, we will still be subject to ongoing reporting obligations, and such product and the related manufacturing operations will be subject to continuing regulatory review, including FDA and other health regulatory inspections. This ongoing review may result in the withdrawal of our product from the market, the interruption of manufacturing operations and/or the imposition of labeling and/or marketing limitations related to specific applications of our product. Since many more patients will be exposed to our product candidates following their marketing approval, serious but infrequent adverse events that were not observed in clinical trials may be observed during the commercial marketing of such product. In addition, the manufacturer(s) and the manufacturing facilities that we will use to produce our product candidates will be subject to periodic review and inspection by the FDA and other health regulatory agencies. Late discovery of previously unknown problems with any product, manufacturer or manufacturing process, or failure to comply with regulatory requirements, may result in actions, such as:

- restrictions on such product, manufacturer or manufacturing process;
- warning letters from the FDA or other regulatory authorities;
- withdrawal of the product from the market;
- suspension or withdrawal of regulatory approvals;
- refusal by such regulator to approve pending applications or supplements to approved applications that we or our licensees (if any) submit;
- voluntary or mandatory recall;
- fines;
- refusal to permit the import or export of our product;
- product seizures or detentions;
- injunctions or the imposition of civil or criminal penalties; and
- adverse publicity.

In addition, from time to time, legislation is drafted and introduced in the United States that could significantly change the statutory provisions governing any regulatory clearance or approval that we receive from the U.S. regulatory authorities. FDA regulations and guidance are often revised or reinterpreted by the FDA in ways that may significantly affect our business and our product. We cannot predict what these changes will be, how or when they will occur or what effect they will have on the regulation of our product. If we, or our licensees, suppliers, collaborative research partners or clinical investigators are slow to adapt, or are unable to adapt, to changes in existing regulatory requirements or the adoption of new regulatory requirements or policies, we may lose marketing approval for any of the therapeutic applications of our product (to the extent that such applications are initially approved), resulting in decreased or lost revenue from milestones, product rental or usage fees, or royalties.

Off-label use is common in the indications for which our product candidates are under development, which may result in enforcement actions by the FDA and other regulatory health agencies for violations of the laws and regulations prohibiting the promotion of off-label uses.

Although physicians, in the practice of medicine, may prescribe approved drugs for unapproved indications, pharmaceutical companies are prohibited from marketing or promoting their drug products for uses outside the approved label, a practice known as off-label promotion. Certain of our product candidates, including AEVI -001 and AEVI -002, are under development for indications for which off-label use is common. To the extent the price of our product candidates, if approved, is significantly higher than the prices of commercially available products that are frequently prescribed off-label, physicians may recommend and prescribe these commercial alternatives instead of writing prescriptions for our products. Either of these outcomes may adversely impact our results of operations by limiting how we price our product and increasing our competition.

In addition, if any of our product candidates are approved, our product labeling, advertising and promotional materials would be subject to regulatory requirements and continuing review by the FDA, Department of Justice, Department of Health and Human Services' Office of Inspector General, state attorneys general, members of Congress and the public. If we are found to have improperly promoted off-label uses of our product candidates, if approved, we may become subject to significant liability. Such enforcement has become more common in the industry. If we are found to have promoted our products for any such off-label uses, the federal government could levy civil, criminal or administrative penalties, and seek fines against us. The FDA or other regulatory authorities could also request that we enter into a consent decree or a corporate integrity agreement, or seek a permanent injunction against us under which specified promotional conduct is monitored, changed or curtailed. If we cannot successfully manage the promotion of our product candidates, if approved, we could become subject to significant liability, which would materially adversely affect our business and financial condition.

In the United States, engaging in the impermissible promotion of our products, following approval, for off-label uses can also subject us to false claims litigation under federal and state statutes, which can lead to civil and criminal penalties and fines, agreements with governmental authorities that materially restrict the manner in which we promote or distribute drug products through, for example, corporate integrity agreements, and debarment, suspension or exclusion from participation in federal and state healthcare programs. These false claims statutes include, among others, federal civil False Claims Act, which allows any individual to bring a lawsuit against a pharmaceutical company on behalf of the federal government alleging submission of false or fraudulent claims, or causing others to present such false or fraudulent claims, for payment by a federal program such as Medicare or Medicaid. If the government decides to intervene and prevails in the lawsuit, the individual will share in the proceeds from any fines or settlement funds. If the government declines to intervene, the individual may pursue the case alone. These false claims lawsuits against pharmaceutical companies have increased significantly in volume and breadth, leading to several substantial civil and criminal settlements regarding certain sales practices promoting off-label drug uses. This growth in litigation has increased the risk that a pharmaceutical company will have to defend a false claim action, pay settlement fines or restitution, as well as criminal and civil penalties, agree to comply with burdensome reporting and compliance obligations, and be excluded from Medicare, Medicaid and other federal and state healthcare programs. If we do not lawfully promote our approved products, if any, we may become subject to such litigation and, if we do not successfully defend against such actions, those actions may have an adverse effect on our business, financial condition, results of operations and prospects.

Even if any of our product candidates receives marketing approval, it may fail to achieve the degree of market acceptance by physicians, patients, third-party payors and others in the medical community necessary for commercial success.

Even if the FDA or any other regulatory health agency approves the marketing of any product candidates that we develop, physicians, patients, third-party payors or the medical community may not accept or use them. Efforts to educate the medical community and third-party payors on the benefits of our product candidates may require significant resources and may not be successful. If AEVI-001, AEVI -002 or any future product candidate that we develop does not achieve an adequate level of acceptance, we may not generate significant product revenue or any profits from operations. The degree of market acceptance of AEVI-001, AEVI -002 or any of our future product candidates that are approved for commercial sale will depend on a variety of factors, including:

- the efficacy and potential advantages compared to alternative treatments;
- effectiveness of sales and marketing efforts;
- the cost of treatment in relation to alternative treatments, including any similar generic treatments;
- our ability to offer our products, if approved, for sale at competitive prices;
- the convenience and ease of administration compared to alternative treatments;
- the willingness of the target patient population to try new therapies and of physicians to prescribe these therapies;
- the strength of marketing and distribution support;
- the availability of third-party coverage and adequate reimbursement, and patients' willingness to pay out-of-pocket in the absence of third-party coverage or adequate reimbursement;
- the prevalence and severity of any side effects;
- any restrictions on the use of our products, if approved, together with other medications; and
- other potential advantages over alternative treatment methods.

Our efforts to educate physicians, patients, third-party payors and others in the medical community on the benefits of our products, if approved, may require significant resources and may never be successful. Such efforts may require more resources than are typically required due to the complexity and uniqueness of our product candidates. Because we expect sales of our product candidates, if approved, to generate substantially all of our product revenue for the foreseeable future, the failure of our product candidates to find market acceptance would harm our business and could require us to seek additional financing.

Our efforts to comply with federal and state fraud and abuse laws could be costly, and, if we are unable to fully comply with such laws, we could face substantial penalties.

We are subject to extensive federal and state healthcare fraud and abuse laws and regulations, including, but not limited to, the following:

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

- federal False Claims Act, which prohibits, among other things, individuals or entities from 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;

- federal Health Insurance Portability and Accountability Act of 1996 (HIPAA), which creates federal criminal laws that prohibit executing a scheme to defraud any healthcare benefit program and which also imposes certain obligations on entities with respect to the privacy, security and transmission of individually identifiable health information;

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

- federal Foreign Corrupt Practices Act (FCPA), which prohibits, among other things, making payments to foreign officials of any country outside of the United States for the purpose of obtaining or retaining business; and

- state laws analogous to each of the above federal laws, such as state anti-kickback and false claims laws (some of which may apply to healthcare items or services reimbursed by any third-party payer, including commercial insurers), as well as certain state laws that require pharmaceutical and medical device companies to comply with industry voluntary compliance guidelines and the relevant compliance guidance promulgated by the federal government.

If our past or present operations are found to be in violation of any of these laws or any other governmental regulations that may apply to us, we may be subject to significant civil, criminal and administrative penalties, damages, fines, exclusion from third-party payer programs such as Medicare and Medicaid and/or the curtailment or restructuring of our operations. If any of the physicians or other providers or entities with whom we may do business are found to be non-compliant with applicable laws, they may be subject to criminal, civil or administrative sanctions including exclusions from government-funded health care programs, which could also negatively impact our operations. Our ongoing efforts to comply with these laws may be costly, and our failure to comply with these laws could have a material adverse effect on our business, financial condition and results of operations. The risk of our being found in violation of these laws is increased by the fact that many of them have not been definitively interpreted by the regulatory authorities or the courts, and their provisions are open to a variety of subjective interpretations. In addition, these laws and their interpretations are subject to change. Any action against us for violation of these laws, even if we successfully defend against it, could cause us to incur significant legal expenses, divert our management's attention from the operation of our business and damage our reputation.

Governments outside the United States tend to impose strict price controls, which may adversely affect our revenues, if any.

In some countries, particularly the countries of the European Union, the pricing of prescription pharmaceuticals is subject to governmental control. In these countries, pricing negotiations with governmental authorities can take considerable time after the receipt of marketing approval for a product. To obtain reimbursement or pricing approval in some countries, we may be required to conduct a clinical trial that compares the cost-effectiveness of our product candidate to other available therapies. If reimbursement of our products is unavailable or limited in scope or amount, or if pricing is set at unsatisfactory levels, our business could be harmed, possibly materially.

We expect to rely on third-party contractors and organizations to conduct our clinical trials, and those third parties may not perform satisfactorily, including failing to meet deadlines for the completion of such trials.

We rely and expect to continue to rely on third-party third-party contractors, clinical data management organizations, independent contractors, medical institutions and clinical investigators to conduct our clinical trials of AEVI-001 and AEVI-002, and for our other programs. These agreements may terminate for a variety of reasons, including a failure to perform by the third parties. If we needed to enter into alternative arrangements, our product development activities could be delayed.

We compete with many other companies, some of which may be our competitors, for the resources of these third parties. Large pharmaceutical companies often have significantly more extensive agreements and relationships with such third-party providers, and such third-party providers may prioritize the requirements of such large pharmaceutical companies over ours. The third parties on whom we rely may terminate their engagements with us at any time, which may cause delay in the development and commercialization of our product candidates. If any such third party terminates its engagement with us or fails to perform as agreed, we may be required to enter into alternative arrangements, which would result in significant cost and delay to our product development program. Moreover, our agreements with such third parties generally do not provide assurances regarding employee turnover and availability, which may cause interruptions in the research on our product candidates by such third parties.

Our reliance on these third parties to conduct our clinical trials will reduce our control over these activities but will not relieve us of our responsibilities. For example, we will remain responsible for ensuring that each of our clinical trials is conducted in accordance with the general investigational plan and protocols for the trial. Moreover, the FDA and other regulatory authorities require us to comply with standards, commonly referred to as good clinical practices, or GCPs, for conducting, recording and reporting the results of clinical trials to assure that data and reported results are credible and accurate and that the rights, integrity and confidentiality of trial participants are protected. We are also required to register ongoing clinical trials and post the results of completed clinical trials on a government-sponsored database, ClinicalTrials.gov, within specified timeframes. Failure to do so can result in fines, adverse publicity and civil and criminal sanctions.

If these third parties do not successfully carry out their contractual duties, meet expected deadlines or conduct our clinical trials in accordance with regulatory requirements or our stated protocols, we will not be able to obtain, or may be delayed in obtaining, marketing approvals for our product candidates and will not be able to, or may be delayed in our efforts to, successfully commercialize our product candidates.

If any of our key employees discontinue his or her services with us, our efforts to develop our business may be delayed.

Our success will depend on the retention of our directors and other current and future members of our management and technical team, including Michael F. Cola, our President and Chief Executive Officer, Brian D. Piper, our Chief Financial Officer, and Garry A. Neil, our Chief Scientific Officer, and on our ability to continue to attract and retain highly skilled and qualified personnel. There can be no assurance that we will retain the services of any of our directors, officers or employees, or attract or retain additional senior managers or skilled employees. Furthermore, we do not carry key man insurance with respect to any of such individuals.

Our lead product candidates, including AEVI-001 and AEVI-002, are still in development and are dependent on further development and testing. We currently employ a small number of key personnel including top managers, scientists, engineers and clinical experts who are important to developing AEVI-001 and AEVI-002 and have a high level of accumulated knowledge which would be lost if they left our Company. If these employees leave our Company or otherwise are unable to provide services, there could be significant implications on the timing and cost of future development of the technology. Because competition for qualified personnel in our industry is intense, we may be unable to timely find suitable replacements with the necessary scientific expertise. We cannot assure you that our efforts to attract or retain such personnel will be successful.

If we are not able to obtain and maintain adequate patent protection for our product candidates, we may be unable to prevent our competitors from using our technology.

Our ability to commercialize AEVI-001, AEVI-002 or our other product candidates, will depend, in part, on our ability, both in the United States and in other countries, to obtain patents, enforce those patents, preserve trade secrets and operate without infringing the proprietary rights of third parties. We have licensed certain intellectual property in connection with AEVI-001 and AEVI-002. Applications for patents and other intellectual property rights capable of being registered have been, and will be, filed in certain key jurisdictions. We may not successfully obtain patents in the countries in which patent applications have been or will be filed, and we may not develop other patentable products or processes. In addition, any future patents may not prevent other persons or companies from developing similar or medically equivalent products, and other persons or companies may be issued patents that may prevent the sale of our products or that will require us to license or pay significant fees or royalties. Furthermore, our own issued and in-licensed patents may not be valid or enforceable or be able to provide our company with meaningful protection. Patent litigation is costly and time-consuming, and there can be no assurance that we will have, or will be able to devote, sufficient resources to pursue such litigation. In addition, potentially unfavorable outcomes in such proceedings could limit our intellectual property rights and activities and have an adverse effect on our business.

We cannot be certain that any of our patent applications, or those of our licensors, will result in issued patents. In addition, because the patent positions of biopharmaceutical companies are highly uncertain and involve complex legal and factual questions, the patents we own and license, or any further patents we may own or license, may not prevent other companies from developing similar or therapeutically equivalent products. Patents also will not protect our product candidates if competitors devise ways of making or using these product candidates without legally infringing our patents. In recent years, several companies have been extremely aggressive in challenging patents covering pharmaceutical products, and the challenges have often been successful. We cannot be assured that our patents will not be challenged by third parties or that we will be successful in any defense we undertake. Failure to successfully defend a patent challenge could materially and adversely affect our business.

In addition, changes in either patent laws or in interpretations of patent laws in the United States and other countries may materially diminish the value of our intellectual property or narrow the scope of our patent protection. On September 16, 2011, the Leahy-Smith America Invents Act, or the Leahy-Smith Act, was signed into law. The Leahy-Smith Act includes a number of significant changes to United States patent law. These include provisions that affect the way patent applications will be prosecuted in the United States and may also affect patent defense and enforcement in the United States.

We also rely on trade secrets, technical know-how and continuing innovation to develop and maintain our competitive position. We seek to protect our proprietary information by requiring our employees, consultants, contractors, outside scientific collaborators and other advisors to execute non-disclosure and confidentiality agreements and our employees to execute assignment of invention agreements to us on commencement of their employment. Agreements with our employees aim to prevent employees from bringing any proprietary rights of third parties to us. We also require confidentiality or material transfer agreements from third parties that receive our confidential data or materials. However, if our employees, consultants, contractors, outside scientific collaborators or other advisors breach their confidentiality or other obligations to us, we may not be able to successfully or effectively prevent such breach and we could be adversely impacted if the protection of our trade secrets or other intellectual property is compromised.

Even if our product candidates and the methods for treating patients for prescribed indications using these product candidates are covered by valid and enforceable patents and have claims with sufficient scope, disclosure and support in the specification, the patents will provide protection only for a limited amount of time. Our and our licensors' ability to obtain patents can be highly uncertain and involve complex and in some cases unsettled legal issues and factual questions.

Furthermore, different countries have different procedures for obtaining patents, and patents issued in different countries provide different degrees of protection against the use of a patented invention by others. Therefore, if the issuance to us or our licensors, in a given country, of a patent covering an invention is not followed by the issuance, in other countries, of patents covering the same invention, or if any judicial interpretation of the validity, enforceability, or scope of the claims in, or the written description or enablement in, a patent issued in one country is not similar to the interpretation given to the corresponding patent issued in another country, our ability to protect our intellectual property in those countries may be limited.

Unauthorized parties may try to copy aspects of our product candidates and technologies or obtain and use information we consider proprietary. Policing the unauthorized use of our proprietary rights is difficult. We cannot guarantee that no harm or threat will be made to our or our collaborators' intellectual property. In addition, changes in, or different interpretations of, patent laws in the United States and other countries may also adversely affect the scope of our patent protection and our competitive situation. Further, we may not have sufficient rights under our license agreements with collaborators to enforce the intellectual property licensed to us against third-party infringers.

As we develop our product candidates, we may need to obtain licenses to protect our rights to make and use our technology. These licenses may not be available on acceptable terms or at all. Even if we are able to obtain a license, the license would likely obligate us to pay license fees or royalties or both, and the rights granted to us might be non-exclusive, which could result in our competitors gaining access to the same intellectual property. Ultimately, we could be prevented from commercializing a product or be forced to cease some aspect of our business operations, if, as a result of actual or threatened patent infringement claims, we are unable to enter into licenses on acceptable terms. All of the issues described above could also impact our collaborators, which would also impact the success of the collaboration and therefore us.

Under certain of our in-licensed patents, the licensor is responsible for maintaining, controlling or enforcing the licensed intellectual property portfolio. Thus, we cannot ensure that the patent rights licensed to us will be adequately maintained, controlled or enforced by our licensor. In addition, even when we have the right to control patent prosecution of licensed patents and patent applications, enforcement of licensed patents, or defense of claims asserting the invalidity of those patents, we may still be adversely affected or prejudiced by actions or inactions of our licensors and their counsel that took place prior to or after our assuming control.

There is certain subject matter that is patent eligible in the United States but not generally patent eligible outside of the United States and vice versa. Differences in what constitutes patent eligible subject matter in various countries may limit the protection we can obtain in the United States and outside of the United States.

Third parties may bring patent infringement or other intellectual property claims against us, which would cause us to incur substantial expenses and, if successful against us, could cause us to pay substantial damages. Further, if a patent infringement suit were brought against us, we could be forced to stop or delay research, development, manufacturing or sales of the product candidate that is the subject of the suit. Additionally, if it is determined that our product candidates infringe third-party patents or other intellectual property rights, there can be no assurance that we can successfully develop non-infringing alternatives on a timely basis or license non-infringing alternatives, if any exist, on commercially reasonable terms. A significant intellectual property impediment to our ability to develop and commercialize our product candidates could materially adversely affect our business prospects.

Even if patents are issued to us or our licensors covering embodiments of our product candidates, devices, or methods of using them, those patents can be challenged by our competitors or other third parties who can argue such patents are invalid or unenforceable, dispute the ownership of the patents, or that the claims of the issued patents should be limited or narrowly construed, which may place our company in a position without meaningful patent rights. Patents also will not protect our product candidates if competitors devise ways of making or using these product candidates without legally infringing our patent claims.

If we fail to comply with our obligations under our existing and any future intellectual property licenses with third parties, we could lose license rights that are important to our business.

We are party to several license agreements under which we in-license patent rights and other intellectual property related to our business, including a license agreement with CHOP, under which we license certain technology owned and controlled by CHOP related to ADHD and certain other neurological and neuropsychological indications. Pursuant to this license agreement, CHOP licensed to neuroFix (coupled with a right to sublicense) certain patent rights and compound know-how on an exclusive, worldwide, royalty-bearing right and license basis, and certain CHOP know-how (other than compound know-how) on a non-exclusive, worldwide, royalty-bearing right and license basis. We may enter into additional license agreements in the future. Our license agreements impose, and we expect that future license agreements will impose, various diligence, milestone payment, royalty, insurance and other obligations on us. Any uncured, material breach under these license agreements could result in our loss of rights to practice the patent rights and other intellectual property licensed to us under these agreements, and could compromise our development and commercialization efforts for AEVI-001 and AEVI-002, or any future product candidates. See the section entitled “Business” for a more detailed description of our current license agreements.

We may be required to make significant payments in connection with our license and development agreements.

We are party to license agreements with CHOP and a Development and Option Agreement with KHK pursuant to which we exclusively license certain technology related to the development of AEVI-001 and AEVI-002. Under our license agreements with CHOP, we may be required to make significant payments in connection with the achievement of certain milestones and royalties on the sale of resulting products. If we exercise our option under the terms of KHK Development and Option Agreement, we will be obligated to cover significant development costs for AEVI-002 and make significant payments in connection with certain milestones and the sale of resulting products. If these obligations become due under the terms of the CHOP license agreements or the Development and Option Agreement, we may not have sufficient funds available to meet our obligations and our development efforts may be negatively impacted.

Our intellectual property in-licenses with third parties may be subject to disagreements over contract interpretations, which could narrow the scope of our rights to the relevant intellectual property or technology or increase our financial or other obligations to our licensors.

The agreements under which we currently in-license intellectual property or technology from third parties are complex, and certain provisions in such agreements may be susceptible to multiple interpretations. The resolution of any contract interpretation disagreement that may arise could narrow what we believe to be the scope of our rights to the relevant intellectual property or technology, or increase what we believe to be our financial or other obligations under the relevant agreement, either of which could harm our business, financial condition, results of operations and prospects. If any of our current or future licenses or material relationships or any in-licenses upon which our current or future licenses are based are terminated or breached, we may:

- lose our rights to develop and market AEVI-001, AEVI-002 or any future product candidates;
- lose patent protection for AEVI-001, AEVI-002 or any future product candidates;
- experience significant delays in the development or commercialization of AEVI-001, AEVI-002 or any future product candidates;
- not be able to obtain any other licenses on acceptable terms, if at all; or
- incur liability for damages.

If we experience any of the foregoing, it could harm our business, financial condition and results of operations.

Our business is dependent on proprietary rights that may be difficult to protect and such dependence could affect our ability to effectively compete.

In addition to our patents, we also rely on trade secrets, know-how, continuing technological innovations and licensing opportunities to develop and maintain our competitive position especially where we do not believe that patent protection is appropriate or obtainable. However, others, including our competitors, may independently develop substantially equivalent proprietary information and techniques or otherwise gain access to our trade secrets or disclose our technology. We take precautionary measures to protect our proprietary rights and information, including the use of confidentiality agreements with employees and consultants, and those with whom we have academic and commercial relationships. However, we may not have such agreements in place with all such parties and, in spite of the measures, there can still be no guarantee that agreements will not be violated or that there will be an adequate remedy available for a violation of an agreement. Any of these events could prevent us from developing or commercializing our product candidates. Trade secrets are by nature difficult to protect. Our employees, consultants, contractors, outside scientific collaborators and other advisors may unintentionally or willfully disclose our confidential information to competitors, and confidentiality agreements may not provide an adequate remedy in the event of unauthorized disclosure of confidential information. Enforcing a claim that a third party illegally obtained and is using our trade secrets is expensive and time-consuming, and the outcome is unpredictable. Moreover, our competitors may independently develop equivalent knowledge, methods and/or know-how. Failure to obtain or maintain trade secret protection could adversely affect our competitive business position.

In addition, third parties may have trademarks or pending applications on our contemplated marks, similar marks, or in confusingly similar fields of use (or may be using our contemplated marks or similar marks). We may have to change our use of certain marks which could have an adverse impact on our business and may require us to spend additional funds to develop new marks. We anticipate that we will spend both time and management resources to develop and file trademark applications in the future.

We are subject to intense competition from companies with greater resources and more mature products, which may result in our competitors developing or commercializing products before or more successfully than us.

While we believe our product candidates have significant advantages, there are a number of well-established and sizeable companies engaged in the development, production, marketing, sale and distribution of products and product candidates that may potentially be competitive with our product candidates. Many of these companies are more experienced than our company and represent significant competition. It is also possible that other parties have in development product candidates substantially similar to or with properties that are more efficacious, less invasive and more cost effectively delivered than our product candidates. The success of our competitors in developing, bringing to market, selling and distributing their products could negatively affect our result of operations and/or general acceptance of our product candidates.

We face risks related to the general economic conditions that may adversely affect our business.

In general, our operating results can be significantly and adversely affected by negative economic conditions, high labor, material and commodity costs, and unforeseen changes in demand for our potential products. These conditions have resulted and could continue to result in slower adoption of new technologies and cost containment efforts by governments and other payers for healthcare research and development, products and services.

Health care policy changes may have a material adverse effect on us.

Health care reform is often a subject of attention in governments that are trying to control health care expenditures. Health care reform proposals have been the subject of much debate in the U.S. Congress and some state legislatures, as well as in other countries. There is no assurance that legislation or underlying rules and guidelines resulting in adverse effects on our company or our product candidates will not be adopted in a country in which we intend to operate and/or upon the distribution of our product candidates in the United States.

In March 2010, President Obama signed into law the ACA and the Health Care and Education Reconciliation Act of 2010. The legislation imposes significant new taxes on medical device makers in the form of a 2.3% excise tax on all U.S. medical device sales that began January 1, 2013. The FDA classifies IVD companion diagnostics as medical devices. Under the law, the total cost to the medical device industry from the tax is expected to be approximately $29 billion over ten years. This significant increase in the tax burden on our industry could have a material, negative impact on our results of operations and our cash flows, especially if any of our product candidates were determined to be a medical device. Other elements of this legislation, such as comparative effectiveness research, an independent payment advisory board, payment system reforms, including shared savings pilots, and other provisions, could meaningfully change the way health care is developed and delivered, and may materially impact numerous aspects of our business. Finally, there are ongoing efforts to modify or eliminate the ACA. It is unknown what form any such modifications or any law proposed to replace the ACA would take, and how or whether it may affect our business in the future.

In August 2017, President Trump signed into law the Food & Drug Administration Reauthorization Act (FDARA). This legislation imposes significant new requirements for clinical trial sponsors which will affect, among other things, obtaining orphan drug designation, and the development of drugs and biological products for pediatric use. This legislation will result in new regulations which might materially impact our business.

Reimbursement policies of third-party payers may negatively affect the acceptance of our product candidates by subjecting the product candidates to sales and pharmaceutical pricing controls.

Third-party payers (Medicare, Medicaid, private health insurance companies and other organizations) may affect the pricing or relative attractiveness of our product candidates by regulating the level of reimbursement provided to the physicians and clinics utilizing our product candidates or by refusing reimbursement. If reimbursement under these programs, or if the amount of time to secure reimbursement is too long, our ability to market our technology and product candidates may be adversely and materially affected. In international markets, reimbursement by private third-party medical insurance providers, including government insurers and independent providers, varies from country to country. In certain countries, our ability to achieve significant market penetration may depend upon the availability of third-party government reimbursement. Pharmaceutical pricing is also subject to regulation in other countries within which we may wish to distribute our product candidates.

The ACA reduces Medicare and Medicaid payments to hospitals, clinical laboratories and pharmaceutical companies, and could otherwise reduce the volume of medical procedures. Further, the Budget Control Act enacted in August 2011 committed the U.S. federal government to significantly reduce the federal deficit over ten years. In addition to placing caps on discretionary spending through 2021, the Budget Control Act also established a budget sequestration that calls for automatic spending cuts over a nine-year period. Across-the-board spending cuts went into effect on March 1, 2013, and Medicare spending cuts that reduce Part A and Part B payments by 2% went into effect on April 1, 2013. Further, the Bipartisan Budget Act of 2013, passed in December 2013, extends the sequestration automatic Medicare spending cuts to 2023 from 2021. Although we cannot predict the full effect on our business of the implementation of existing legislation such as the ACA and the Budget Control Act, or the enactment of additional legislation, we believe that legislation or regulation that reduces reimbursement for our products could adversely affect how much or under what circumstances health care providers will prescribe or administer our products. This could materially and adversely impact our business by reducing our ability to generate revenue, raise capital, obtain additional collaborators and market our products. In addition, we believe the increasing emphasis on managed care in the United States has and will continue to put pressure on the price and usage of pharmaceutical products, which may adversely impact product sales.

The pricing of pharmaceutical products, in general, and specialty drugs, in particular, has also been a topic of concern in the U.S. government. There can be no assurance as to how this scrutiny on pricing of pharmaceutical products will impact future pricing of our products or orphan drugs or pharmaceutical products generally.

We may experience product liability claims, which could adversely affect our business and financial condition.

We may become subject to product liability claims. We have not experienced any product liability claims to date; however, the production at commercial scale, distribution, sale and support of our product candidates may entail the risk of such claims, which is likely to be substantial in light of the use of our product candidates in the treatment of medical conditions. We carry product liability insurance coverage in connection with the clinical trials of our product candidates. If we are unable to obtain a renewal or if we suffer a successful product liability claim in excess of our insurance coverage, such claim could result in significant monetary liability and could have a material adverse impact on our business, operations, financial position and/or reputation.

Failure to maintain effective internal controls could have a material adverse effect on our business and operating results. In addition, current and potential stockholders could lose confidence in our financial reporting, which could have a material adverse effect on the price of our common stock.

Effective internal controls are necessary for us to provide reliable financial reports and effectively prevent fraud. If we cannot provide reliable financial reports or prevent fraud, our results of operation could be harmed.

Section 404 of the Sarbanes-Oxley Act of 2002 requires annual management assessments of the effectiveness of our internal controls over financial reporting and a report by our independent registered public accounting firm addressing these assessments. We continuously monitor our existing internal controls over financial reporting systems to confirm that they are effective, and we may identify deficiencies that we may not be able to remediate in time to meet the deadlines imposed by the Sarbanes-Oxley Act. This process may divert internal resources and will take a significant amount of time and effort to complete.

If at any time it is determined that our internal controls are not effective, we may be required to implement new internal control procedures and reevaluate our financial reporting. We may experience higher than anticipated operating expenses as well as increased independent auditor fees during the implementation of these changes and thereafter. Further, we may need to hire additional qualified personnel. If we fail to maintain the adequacy of our internal controls, as such standards are modified, supplemented or amended from time to time, we may not be able to conclude on an ongoing basis that we have effective internal controls over financial reporting in accordance with Section 404 of the Sarbanes-Oxley Act, which could result in our being unable to obtain an unqualified report on internal controls from our independent auditors. Failure to maintain an effective internal control environment could also cause investors to lose confidence in our reported financial information, which could have a material adverse effect on the price of our common stock.

Compliance with changing regulation of corporate governance and public disclosure may result in additional expenses, divert management's attention from operating our business which could have a material adverse effect on our business.

There have been changing laws, regulations and standards relating to corporate governance and public disclosure, as well as new regulations promulgated by the SEC and rules promulgated by the national securities exchanges, including the Nasdaq Global Market. These new or changed laws, regulations and standards are subject to varying interpretations in many cases due to their lack of specificity, and as a result, their application in practice may evolve over time as new guidance is provided by regulatory and governing bodies, which could result in continuing uncertainty regarding compliance matters and higher costs necessitated by ongoing revisions to disclosure and governance practices. As a result, our efforts to comply with evolving laws, regulations and standards are likely to continue to result in increased general and administrative expenses and a diversion of management time and attention from revenue-generating activities to compliance activities. Our board members, principal executive officer and principal financial officer could face an increased risk of personal liability in connection with the performance of their duties. As a result, we may have difficulty attracting and retaining qualified board members and executive officers, which could have a material adverse effect on our business. If our efforts to comply with new or changed laws, regulations and standards differ from the activities intended by regulatory or governing bodies, we may incur additional expenses to comply with standards set by regulatory authorities or governing bodies which would have a material adverse effect on our business, financial condition and results of operations.

Security breaches and other disruptions to our information technology infrastructure could interfere with our operations or clinical trials, compromise information belonging to us and our suppliers and expose us to liability, which could adversely impact our business and reputation.

In the ordinary course of business, we rely on information technology networks and systems, some of which are managed by third parties, to process, transmit and store electronic information, and to manage or support a variety of business processes and activities, including the conduct of our clinical trials. Additionally, we collect and store sensitive data, including proprietary business information and confidential patient health information. Despite security measures and business continuity plans, our information technology networks and infrastructure may be vulnerable to damage, disruptions or shutdowns due to attack by hackers or breaches, employee error or malfeasance, power outages, computer viruses, telecommunication or utility failures, systems failures, natural disasters or other catastrophic events. Any such event could result in legal claims or proceedings, liability or significant penalties under privacy laws, disruption in operations and damage to our reputation, which could adversely affect our business.

The recently passed comprehensive tax reform bill could adversely affect our business and financial condition.

On December 22, 2017, President Trump signed into law the final version of the tax reform bill commonly known as the "Tax Cuts and Jobs Act," or the TCJA, that significantly reforms the Internal Revenue Code of 1986, as amended, with many of its provisions effective for tax years beginning on or after January 1, 2018. The TCJA, among other things, contains significant changes to corporate taxation, including a permanent reduction of the corporate income tax rate, a partial limitation on the deductibility of business interest expense, a limitation of the deduction for net operating loss carryforwards to 80% of current year taxable income, an indefinite net operating loss carryforward and the elimination of the two-year net operating loss carryback, temporary, immediate expensing for certain new investments , and the modification or repeal of many business deductions and credits. We continue to examine the impact this tax reform legislation may have on our business. Notwithstanding the reduction in the corporate income tax rate, the overall impact of the TCJA is uncertain and our business and financial condition could be adversely affected. The impact of this reform on our stockholders is uncertain. Stockholders should consult with their tax advisors regarding the effect of the TCJA and other potential changes to the U.S. Federal tax laws on them.

Risk Related to our Securities

Our securities are thinly traded, resulting in relative illiquidity and price volatility, and there may not ever be an active market for our securities.

Although our common stock has been traded on the Nasdaq Global Market since October 21, 2016 and, prior to that on the NYSE MKT since April 8, 2011, the volumes and trading in our securities have been extremely sporadic. As a result, the ability of holders to purchase or sell our securities is limited, with low-volume trading creating wide shifts in price. For our securities to continue to be listed on the Nasdaq Global Market, we must meet the current listing requirements of that exchange. If we were unable to meet these requirements, our securities could be delisted from the Nasdaq Global Market. Any such delisting of our securities could have an adverse effect on the market price of, and the efficiency of the trading market for, our securities, not only in terms of the number of shares that can be bought and sold at a given price, but also through delays in the timing of transactions and less coverage of us by securities analysts, if any. Also, if in the future we were to determine that we need to seek additional equity capital, it could have an adverse effect on our ability to raise capital in the public or private equity markets.

Further, the share prices of public companies, particularly those operating in high growth sectors, are often subject to significant fluctuations. The market price of our common stock on the Nasdaq Global Market has been volatile, ranging from $0.98 per share to $6.18 per share during the 52-week trading period ending March 9, 2018. We expect that the market price of our common stock will continue to fluctuate significantly due to factors including, but not limited to, the following:

- results of our clinical trials, such as the Phase 2 trial in mGluR+ Genetic Subset ADHD to confirm genetic responders to AEVI-001, for which data is expected by mid-2018 and the 8-week signal finding study at CHOP enrolling up to 12 patients with the DcR3 mutation and a Pediatric Onset Crohn's disease diagnosis, for which initial data is expected by year-end 2018;
- announcements of developments by us or our competitors;
- announcements by us or our competitors of significant acquisitions, strategic partnerships, joint ventures or capital commitments;
- adoption of new accounting standards affecting our industry;
- introduction of new products by us or our competitors;

- changes in market valuations of companies in our industry;
- actual or anticipated variations in our operating results;
- future issuances of our common stock or other securities;
- other events or factors, including those beyond our control; and
- general market or economic conditions.

Securities analysts may not initiate coverage or continue to cover our common stock, and this may have a negative impact on its market price.

The trading market for our securities could depend in part on the research and reports that securities analysts publish about our business and us. We do not have any control over these analysts. There is no guarantee that securities analysts will cover our securities. If securities analysts do not cover our securities, the lack of research coverage may adversely affect their market prices. If we are covered by securities analysts, and our securities are the subject of an unfavorable report, the prices for our securities would likely decline. If one or more of these analysts ceases to cover us or fails to publish regular reports on us, we could lose visibility in the financial markets, which could cause our stock price and/or trading volume to decline.

The exercise of options and other issuances of shares of common stock or securities convertible into or exercisable for shares of common stock will dilute the ownership interests of our current stockholders and may adversely affect the future market price of our common stock.

Sales of our common stock in the public market, either by us or by our current stockholders, or the perception that these sales could occur, could cause a decline in the market price of our securities. Nearly all of the shares of our common stock held by those of our current stockholders who are not affiliates may be immediately eligible for resale in the open market either in compliance with an exemption under Rule 144 promulgated under the Securities Act of 1933, as amended, or the Securities Act, or pursuant to an effective resale registration statement that we have previously filed with the SEC. Such sales, along with any other market transactions, could adversely affect the market price of our common stock.

In addition, as of December 31, 2017, there were outstanding options and warrants to purchase an aggregate of 11,004,152 and 7,203,223 shares, respectively. Of the 11,004,152 outstanding options, ranging in exercise price from $1.07 per share to $8.80 per share, 6,778,945 shares were exercisable as of December 31, 2017. The exercise of options at prices below the market price of our common stock could adversely affect the price of shares of our common stock. Additional dilution may result from the issuance of shares of our common stock in connection with collaborations or manufacturing arrangements or in connection with other financing efforts.

Any issuance of our common stock that is not made solely to then-existing stockholders proportionate to their interests, such as in the case of a stock dividend or stock split, will result in dilution to each stockholder by reducing his, her or its percentage ownership of the total outstanding shares. Moreover, if we issue options or warrants to purchase our common stock in the future and those options or warrants are exercised, stockholders may experience further dilution. Delaware law and our corporate governance documents do not prohibit the number of options or other securities that are convertible into, exchangeable for or represent the right to receive common stock that we may issue in the future, except to the extent we are limited by the number of our authorized shares of common stock which is currently 200,000,000 shares. Holders of shares of our common stock have no preemptive rights that entitle them to purchase their pro rata share of any offering of shares of any class or series.

We have a significant stockholder, which will limit your ability to influence corporate matters and may give rise to conflicts of interest.

The Children's Hospital of Philadelphia Foundation (the "CHOP Foundation") is our largest stockholder. As of March 9, 2018, the CHOP Foundation beneficially owned 18,697,233 shares of our common stock. The shares of common stock beneficially owned by the CHOP Foundation represent approximately 30.1% of our outstanding shares of common stock. Accordingly, the CHOP Foundation exerts significant influence over us and any action requiring the approval of the holders of our common stock, including the election of directors and approval of significant corporate transactions. This concentration of voting power makes it less likely that any other holder of common stock or directors of our business will be able to affect the way we are managed and could delay or prevent an acquisition of us on terms that other stockholders may desire. In addition, if the CHOP Foundation obtains a majority of our common stock, the CHOP Foundation would be able to control all matters submitted to our stockholders for approval, as well as our management and affairs. For example, the CHOP Foundation would be able to control the election of directors, amendments to our organizational documents and approval of any merger, consolidation, sale of all or substantially all of our assets or other business combination or reorganization. In addition, if the CHOP Foundation obtains a majority of our common stock, we would be deemed a "controlled company" for purposes of NASDAQ listing requirements. Under NASDAQ rules, a "controlled company" may elect not to comply with certain NASDAQ corporate governance requirements, including (i) the requirement that a majority of our board of directors consist of independent directors, (ii) the requirement that the compensation of our officers be determined or recommended to the board by a majority of independent directors or a compensation committee that is composed entirely of independent directors and (iii) the requirement that director nominees be selected or recommended to the board by a majority of independent directors or a nominating committee that is composed of entirely independent directors.

Furthermore, the interests of the CHOP Foundation may not always coincide with your interests or the interests of other stockholders and the CHOP Foundation may act in a manner that advances its best interests and not necessarily those of other stockholders, including seeking a premium value for its common stock, and might affect the prevailing market price for our common stock. Our board of directors, which currently consists of eight directors, including one designated by the CHOP Foundation, has the power to set the number of directors on our board from time to time. Matthew D. Bayley, who currently serves as the Senior Vice President and Chief Strategy Officer at the CHOP Foundation, is a member of our board of directors and some of its committees.

We have never declared or paid dividends on our capital stock and we do not anticipate paying any cash dividends in the foreseeable future.

We have never declared or paid dividends on our capital stock and we do not anticipate paying any cash dividends in the foreseeable future. We currently intend to retain future earnings, if any, to fund the development and growth of our business. Any future determination to pay dividends will be at the discretion of our Board of Directors and will be dependent upon our financial condition, operating results, capital requirements, applicable contractual restrictions and other such factors as our Board of Directors may deem relevant.

Provisions of Delaware law may delay or prevent efforts to acquire a controlling interest in us, even if such acquisition were in the best interests of our stockholders.

We are subject to the anti-takeover provisions of Section 203 of the Delaware General Corporation Law, which regulates corporate acquisitions. These provisions could discourage potential acquisition proposals and could delay or prevent a change in control transaction. They could also have the effect of discouraging others from making tender offers for our common stock. These provisions may also prevent changes in our management.

ITEM 1B - Unresolved Staff Comments.

None.

ITEM 2 - Properties.

Our principal executive offices are located at 435 Devon Park Drive, Suite 715, Wayne, Pennsylvania 19087. We believe that this facility is adequate to meet our current needs. We believe that if additional or alternative space is needed in the future, such space will be available on commercially reasonable terms as necessary.

ITEM 3 - Legal Proceedings.

We are not currently a party, as plaintiff or defendant, to any legal proceedings which, individually or in the aggregate, are expected by us to have a material effect on our business, financial condition or results of operation if determined adversely to us.

ITEM 4 -Mine Safety Disclosures.

Not applicable.

PART II

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

Market Information

Our common stock issued in connection with our U.S. initial public offering in April 2011 was previously traded on the NYSE MKT under the symbol "MDGN".

On October 10, 2016, we provided written notice to the NYSE MKT of our intention to voluntarily delist our common stock from the NYSE MKT and to list our common stock on the Nasdaq Global Market. The listing and trading of our common stock on the NYSE MKT ceased at market close on October 20, 2016, and trading of our common stock on the Nasdaq Global Market commenced on October 21, 2016. The common stock was approved for listing on the Nasdaq Global Market and continuing to trade under the symbol "MDGN" until December 15, 2016. Effective December 16, 2016, in connection with our corporate name change to Aevi Genomic Medicine, Inc., our common stock ceased trading under the ticker symbol "MDGN" and commenced trading under the new ticker symbol "GNMX".

The following table sets forth, for the periods indicated, the high and low sale prices for our common stock as reported by the NYSE MKT or the Nasdaq Global Market, as applicable:

2016	**High**	**Low**
First Quarter (January 1, 2016 to March 31, 2016)	$ 6.18	$ 3.09
Second Quarter (April 1, 2016 to June 30, 2016)	6.60	4.34
Third Quarter (July 1, 2016 to September 30, 2016)	6.32	4.74
Fourth Quarter (October 1, 2016 to December 31, 2016)	6.89	4.00

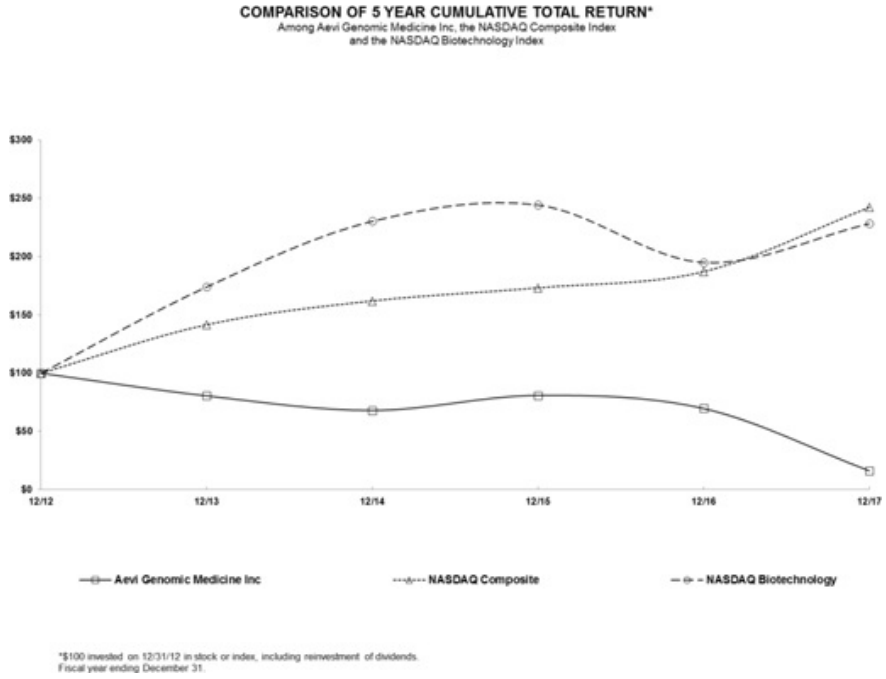
2017	**High**	**Low**
First Quarter (January 1, 2017 to March 31, 2017)	$ 6.18	$ 1.50
Second Quarter (April 1, 2017 to June 30, 2017)	1.92	0.98
Third Quarter (July 1, 2017 to September 30, 2017)	1.47	1.12
Fourth Quarter (October 1, 2017 to December 31, 2017)	1.91	1.01

Holders of Record

As of March 9, 2018, there were 253 holders of record of our common stock. We believe there are a substantially greater number of beneficial holders.

Stock Performance Graph

The following graph compares the cumulative total stockholder return data for our common stock from January 1, 2012 through December 31, 2017 to the cumulative return over such time period of (i) the NASDAQ Composite Index and (ii) the NASDAQ Biotechnology Index. The graph assumes an investment of $100 on January 1, 2012 in (i) our common stock, (ii) the securities comprising the NASDAQ Composite Index and (iii) the securities comprising the NASDAQ Biotechnology Index, including dividend reinvestment, if any. The comparisons in the graph are not intended to forecast or be indicative of possible future performance of our common stock.



Dividends

We have never declared dividends on our equity securities, and currently do not plan to declare dividends on shares of our common stock in the foreseeable future. We expect to retain our future earnings, if any, for use in the operation and expansion of our business. Subject to the foregoing, the payment of cash dividends in the future, if any, will be at the discretion of our Board of Directors and will depend upon such factors as earnings levels, capital requirements, our overall financial condition and any other factors deemed relevant by our Board of Directors.

Recent Sales of Unregistered Securities

None.

Purchases of Equity Securities by the Issuer and Affiliated Purchasers

None.

Equity Compensation Plan Information

The following table provides information as of December 31, 2017 regarding the common stock that may be issued as stock grants or upon exercise of options, warrants and rights under all of our equity compensation plans, including individual compensation arrangements.

Plan Category	Number of Shares to Be Issued Upon Exercise of Outstanding Options and Warrants[1] (a)	Weighted Average Exercise Price of Outstanding Options and Warrants (b)	Number of Shares Remaining Available for Future Issuance Under Equity Compensation Plans (Excluding Securities Reflected in Column (a)) (c)
Equity compensation plans approved by security holders	7,254,152[2]	$ 4.35	901,270
Equity compensation plans not approved by security holders	3,875,000[3]	$ 4.35	—
Total	11,129,152	$ 4.35	901,270

(1) The number of shares is subject to adjustment in the event of stock splits and other similar events.

(2) Consists of options awarded under the Stock Incentive Plan.

(3) Consists of:

(i) Inducement awards granted in September 2013 outside of the Stock Incentive Plan to Mr. Cola (1,500,000 options), Dr. Leaman (800,000 options) and Dr. Neil (900,000 options). Dr. Leaman's options expired on February 9, 2018;

(ii) Inducement awards of an aggregate of 275,000 options granted outside of the Stock Incentive Plan to two new employees in December 2015 having an exercise price of $7.37 per share and expiring on December 10, 2025. 125,000 of which expired as of December 31, 2017;

(iii) An inducement award of 100,000 options granted outside of the Stock Incentive Plan to a new employee in February 2016 having an exercise price of $3.64 per share and expiring on February 2, 2026;

(iv) An inducement award of 200,000 options granted outside of the Stock Incentive Plan to a new employee in March 2016 having an exercise price of $4.42 per share and expiring on March 7, 2026;

(v) An inducement award of 100,000 options granted outside of the Stock Incentive Plan to a new employee in April 2016 having an exercise price of $5.07 per share and expiring on April 19, 2026; and

(vi) Warrants to purchase an aggregate of 125,000 shares of common stock issued as compensation to consultants, as further described in Note 7 to the condensed consolidated financial statements included herein.

ITEM 6 - Selected Financial Data.

The selected data presented below under the captions "Statement of Operations Data," "Statement of Cash Flows Data" and "Balance Sheet Data" for, and as of the end of, each of the fiscal years in the five-year period ended December 31, 2017, are derived from, and should be read in conjunction with, our audited consolidated financial statements.

The information contained in this table should also be read in conjunction with "Management's Discussion and Analysis of Financial Condition and Results of Operations" and the financial statements and related notes thereto included elsewhere in this report (in thousands of dollars except share and per share data):

	Year Ended December 31,				
	2017	**2016**	**2015**	**2014**	**2013**
Statement of Operations Data:					
Operating expenses:					
Research and development expenses, net	$ 25,176	$ 28,356	$ 15,444	$ 8,253	$ 7,297
Non-recurring research and development expenses resulting from acquisition	-	-	8,170	-	-
General and administrative expenses	9,524	13,523	12,954	10,686	10,521
Operating loss	(34,700)	(41,879)	(36,568)	(18,939)	(17,818)
Financial expenses	(41)	(24)	(1,408)	(68)	(20)
Financial income	27	15	1	586	726
Loss before taxes on income	(34,714)	(41,888)	(37,975)	(18,421)	(17,112)
Taxes on income	-	16	17	12	17
Net loss	(34,714)	(41,904)	(37,992)	(18,433)	(17,129)
Basic loss per share	$ (0.83)	$ (1.19)	$ (1.42)	$ (0.96)	$ (0.97)
Diluted loss per share	$ (0.83)	$ (1.19)	$ (1.45)	$ (1.00)	$ (1.06)
Weighted average number of shares used in computing basic loss per share	41,675,814	35,161,823	26,783,623	19,246,611	17,629,436
Weighted average number of shares used in computing diluted loss per share	41,675,814	35,161,823	26,846,270	19,294,259	17,683,510
Statement of Cash Flows Data:					
Net cash used in operating activities	$ (33,246)	$ (32,749)	$ (24,347)	$ (12,195)	$ (12,732)
Net cash provided by (used in) investing activities	148	(221)	(187)	(363)	(183)
Net cash provided by financing activities	26,989	19,744	44,310	23,456	28,874
(Decrease) increase in cash and cash equivalents	(6,109)	(13,226)	19,776	10,898	15,959
Balance Sheet Data:					
Cash and cash equivalents	$ 33,729	$ 39,838	$ 53,064	$ 33,288	$ 22,390
Current assets	34,622	40,173	53,811	33,603	22,592
Long-term assets	139	388	447	677	495
Total assets	34,761	40,561	54,258	34,280	23,087
Current liabilities	4,140	5,583	3,908	3,638	3,014
Long-term liabilities	-	-	-	980	1,650

ITEM 7 - Management's Discussion and Analysis of Financial Condition and Results of Operations.

The following discussion and analysis of our financial condition and results of operations should be read together with our financial statements and related notes appearing elsewhere in this Annual Report on Form 10-K.

Overview

We are a clinical stage biopharmaceutical company with an emphasis on identifying the genetic drivers of disease and applying this understanding to the pursuit of differentiated novel therapies primarily for pediatric onset, life-altering diseases, including rare and orphan diseases. We look to find treatments for genetically defined diseases for which there are limited therapeutic options currently available, with a primary focus on pediatric patients. This strategy begins with identifying and genetically validating a therapeutic target and using genomics to guide product development. The strategy also involves identifying and acquiring otherwise abandoned or overlooked drug candidates and matching targets and mechanisms of action to novel genetic discoveries.

We have partnered with the Center for Applied Genomics, or CAG, at The Children's Hospital of Philadelphia, or CHOP, to implement a genomic medicine driven approach to drug development. CAG's assets include a fully automated biorepository containing specimens from more than 75,000 pediatric patients and 150,000 relatives of those patients. The sample is highly enriched for rare and orphan diseases and the large majority of patients have been genotyped. Their phenotypes are recorded in a modern electronic health record that is linked to the genomics database and biorepository. The patients in the database have consented to anonymized use of their data for research and follow up contact if needed.

CAG continues to discover important and novel genetic biomarkers by both genome-wide association studies and exome sequencing and analysis of affected individuals and their family members. Such markers not only identify patients with the disease but frequently point to the cause of the disease and suggest targets and feasible intervention strategies that include protein or peptide therapy, monoclonal antibodies, drugs or gene therapy. By working initially in pediatric populations of specific diseases, we can minimize the confounding environmental factors seen in older patients. In addition, the availability of robust genetic biomarkers allows us to design trials that focus on a highly-enriched patient population that we believe is more likely to respond to targeted therapies and further enhance the likelihood of clinical and regulatory success. We believe this will allow us to implement more efficient and shorter clinical development programs, that will lead to higher value medicines that can address critical needs in patients suffering from rare and orphan diseases.

We have generated significant losses to date, and we expect to continue to generate losses as we progress towards the commercialization of our product candidates. We have incurred net losses of approximately $34.71 million, $41.90 million and $37.99 million for the years ended December 31, 2017, 2016 and 2015, respectively. As of December 31, 2017, we had cash and cash equivalents of $33.73 million, which we believe will provide funding for us into the first quarter 2019. We are unable to predict the extent of any future losses or when we will become profitable, if at all.

AEVI-001 (*mGluR+ Genetic Subset ADHD)*

The lead program from our genomic research collaboration with CHOP is the development candidate AEVI-001, an oral, non-stimulant glutamatergic neuromodulator. Through our acquisition of neuroFix, LLC, or neuroFix, in September 2015, we acquired the rights to develop AEVI-001 (then known as NFC-1), as well as the rights to certain data derived from a clinical trial and other studies of AEVI-001.

The selection of AEVI-001 for development in the mGluR+ ADHD patients was the result of a rationale search process conducted to specifically identify therapeutic candidates with a demonstrated ability to modulate glutamate signaling via mGluR network. The role of glutamate in ADHD and other central nervous system (CNS) disorders is supported by recent neuroimaging studies that suggest glutamate levels are abnormal in children with ADHD. These abnormalities appear to be concentrated in the anterior singular cortex region of the brain, as evidenced by volumetric and functional magnetic resonance imagery studies, as well as targeted studies of magnetic resonance spectroscopy. Additional supportive evidence for targeting glutamate modulation is provided by genetic studies that have identified mutations in glutamatergic genes that are enriched in children with ADHD.

AEVI-002 ***(Anti-LIGHT Monoclonal Antibody)***

The second program arising out of our genomic research collaboration with CHOP is the development candidate AEVI-002, a first-in-class anti-LIGHT monoclonal antibody, or the Antibody, being developed for use in Pediatric Onset Crohn's disease. Pediatric Onset Crohn's disease has a more aggressive phenotype at younger ages. The genomic rationale for the use of anti-LIGHT antibody in Crohn's disease was validated by CAG research showing the association to a loss of function mutation in decoy receptor 3 (DcR3). The estimated prevalence of the mutation is 10-15% of Pediatric Onset Crohn's cases.

In June 2016, we entered into a Clinical Development and Option Agreement, or the Development and Option Agreement, with Kyowa Hakko Kirin Co., Ltd., or KHK, pursuant to which we acquired certain rights with respect to the development and potential commercialization of the Antibody. Under the Development and Option Agreement, we received an exclusive option for exclusive rights to develop products containing the Antibody, or an Antibody Licensed Product, exclusive rights to commercialize Antibody Licensed Product in various countries and to conduct various development activities with respect to the Antibody Licensed Product, including the conduct of a signal finding study testing the Antibody in Severe Pediatric Onset Inflammatory Bowel Disease, or the Study. The terms of the Development and Option Agreement with KHK are more fully described under the section entitled "Licenses."

An 8-week Phase Ib proof-of-concept study has been initiated at CHOP, with the goal of enrolling up to 12 patients with a Pediatric Onset Crohn's disease diagnosis that are refractory to treatment with TNF-α inhibitors, with or without a DcR3 mutation. Active recruitment for the trial is underway and we have enrolled zero patients. The endpoints of the trial will include endoscopic evaluation, Crohn's Disease Activity Index ratings and safety. Initial data from the proof-of-concept study is expected second half of 2018, at which point we will make a determination on our option to license exclusive rights to the Antibody for further development. The identification and recruitment of patients into the proof-of-concept study continues to be challenging. The ability to produce initial data by year-end 2018 is highly dependent on timely recruiting; thus, continued difficulties in recruitment could cause a delay in the delivery of initial data for the program. In an effort to address the recruitment challenges, we are currently initiating three additional trial sites for the program.

Financial Operations Overview

Research and Development Expense

Research and development expense consists of: (i) internal costs associated with our development activities; (ii) payments we make to third party CROs, contract manufacturers, clinical trial sites and consultants; (iii) technology and intellectual property license costs; (iv) manufacturing development costs; (v) personnel related expenses, including salaries, and other related costs, including stock-based compensation expense, for the personnel involved in product development; (vi) activities related to regulatory filings and the advancement of our product candidates through preclinical studies and clinical trials; and (vii) facilities and other allocated expenses, which include direct and allocated expenses for rent, facility maintenance, as well as laboratory and other supplies. All research and development costs are expensed as incurred.

Conducting a significant amount of development is central to our business model. Product candidates in later-stage clinical development generally have higher development costs than those in earlier stages of development, primarily due to the significantly increased size and duration of the clinical trials.

The process of conducting pre-clinical studies and clinical trials necessary to obtain regulatory approval is costly and time consuming. The probability of success for each product candidate and clinical trial may be affected by a variety of factors, including, among others, the quality of the product candidate's early clinical data, investment in the program, competition, manufacturing capabilities and commercial viability. As a result of these uncertainties, together with the uncertainty associated with clinical trial enrollments and the risks inherent in the development process, we are unable to determine the duration and completion costs of current or future clinical stages of our product candidates or when, or to what extent, we will generate revenues from the commercialization and sale of any of our product candidates. Development timelines, probability of success and development costs vary widely. We are concurrently focusing on pursuing clinical and pre-clinical research and development in targeted orphan and rare disease.

Research and development expenses are shown net of participation by OCS in previous years.

General and Administrative Expense

General and administrative expense consists primarily of salaries and other related costs, including stock-based compensation expense, for persons serving as our directors and in our executive, finance and accounting functions. Other general and administrative expense includes facility-related costs not otherwise included in research and development expense, costs associated with industry and trade shows, and professional fees for legal services and accounting services. We expect that our general and administrative expenses will increase as we add personnel.

Financial Income and Expense

Financial income and expense consist primarily of warrant valuations and foreign currency exchange differences.

Results of Operations for the Year Ended December 31, 2017 and 2016

Research and Development Expenses

Net research and development expenses for year ended December 31, 2017 decreased to $25.18 million from $28.36 million in 2016. This decrease was primarily driven by the closure of our operations in Israel of $3.40 million.

General and Administrative Expenses

General and administrative expenses for the year ended December 31, 2017 were $9.52 million, decreasing from $13.52 million in 2016 primarily due to severance benefits recorded in 2016 related to termination of an officer of $0.97 million, decreased stock compensation expense related to options which have fully vested of $2.00 million, and closure of our operations in Israel of $0.84 million.

Financial Income and Expenses

Financial income and expenses for the year ended December 31, 2017 and 2016 was de minimis.

Results of Operations for the Year Ended December 31, 2016 and 2015

Research and Development Expenses

Gross research and development expenses for year ended December 31, 2016 increased to $28.55 million from $18.36 million in 2015. This increase was primarily driven by increased spending on third party related costs used to advance our clinical activities related to the AEVI-001 program. Net research and development expenses for the year ended December 31, 2016 increased to $28.36 million from $15.44 million in 2015 due to the increase in gross research and development expenses as detailed above in addition to reduced OCS funding in 2016.

During 2016 there were no non-recurring research and development costs compared to $8.17 million for the year ended December 31, 2015 which pertained to the neuroFix acquisition.

General and Administrative Expenses

General and administrative expenses for the year ended December 31, 2016 were $13.52 million, increasing from $12.95 million in 2015 primarily due to an increase in professional fees and US-based headcount offset in part by a decrease in stock-based compensation expense related to options granted to directors.

Financial Income and Expenses

Financial expenses for the year ended December 31, 2016 were $0.02 million, decreasing from $1.41 million in 2015. This decrease was mainly due to the non-cash change in valuation of the warrant liability in 2015.

Financial income for the year ended December 31, 2016 and 2015 was de minimis.

Liquidity and Capital Resources

Sources of Liquidity

We have financed our operations primarily through equity issuances and grants from the OCS and other third parties.

In the year ended December 31, 2017 and 2016, options and warrants were exercised in consideration of $0.02 million and $0.18 million, respectively, and 6,200 shares and 407,865 shares of common stock were issued upon such exercises, respectively.

On October 17, 2017, we sold an aggregate of 22,222,222 shares of our common stock, and warrants exercisable for up to an aggregate of 3,953,904 shares of common stock at a purchase price of $1.26 per share of common stock and accompanying warrants pursuant to that certain securities purchase agreement dated as of August 9, 2017, or the 2017 Funding. The aggregate gross proceeds from the offering to us were approximately $28.00 million and net proceeds after deducting estimated offering expenses were approximately $26.97 million.

On June 24, 2016, we completed a registered public offering of 3,835,261 shares of common stock, which included 195,261 shares sold pursuant to the partial exercise of the underwriters' over-allotment option, at a price to the public of $5.50 per share, or the 2016 Funding. The net proceeds from the 2016 Funding to us were approximately $19.56 million, after deducting underwriting discounts and commissions and offering expenses of $1.55 million.

On October 6, 2015, we completed a registered public offering of 7,078,250 shares of common stock, including 923,250 shares sold pursuant to the full exercise of the underwriters' over-allotment option, at a price to the public of $6.50 per share, or the 2015 Funding. The net proceeds from the 2015 Funding to us were approximately $42.88 million, after deducting underwriting discounts and commissions and offering expenses payable by us.

Cash Flows

We had cash and cash equivalents of $33.73 million at December 31, 2017 and $39.84 million at December 31, 2016. The decrease in our cash balance during 2017 was primarily related to advancement of our AEVI-001 program offset with 2017 Funding.

Net cash used in operating activities of $33.25 million, $32.75 million, and $24.35 million for the year ended December 31, 2017, 2016 and 2015, respectively, primarily reflected our net cash expenses for our operations.

Net cash provided by investing activities relates mainly to $0.15 million of proceeds received from the sale of property and equipment as we closed our operations in Israel.

Net cash provided by financing activities was $26.99 million, $19.74 million and $44.31 million for the years ended December 31, 2017, 2016 and 2015, respectively resulting primarily from the 2017 Funding, the 2016 Funding and the 2015 Funding.

Funding Requirements

Our future capital requirements will depend on a number of factors, including our success in targeting rare and orphan disease candidates, design of our clinical trials and regulatory guidance and feedback, the timing and outcome of clinical trials and regulatory approvals, the costs involved in preparing, filing, prosecuting, maintaining, defending, and enforcing patent claims and other intellectual property rights, the acquisition of licenses to new products or compounds, the status of competitive products, the availability of financing, and our success in developing markets for our product candidates.

We believe that cash on hand will be sufficient to enable us to fund our operating expenses and capital expenditure requirements into the first quarter 2019. We have based this estimate on assumptions that may prove to be wrong and we could use our available resources sooner than we currently expect. Because of the numerous risks and uncertainties associated with the development and commercialization of our product candidates, we are unable to estimate the amounts of increased capital outlays and operating expenditures associated with our current and anticipated clinical trials.

We do not anticipate that we will generate revenue from the sale of products for several years or more given the uncertainty of drug development. Absent significant corporate collaboration and licensing arrangements, we will need to finance our future cash needs through public or private equity offerings or debt financings in 2018. We do not currently have any commitments for future external funding. We may need to raise additional funds more quickly if one or more of our assumptions prove to be incorrect or if we choose to expand our product development efforts more rapidly than we presently anticipate. We may seek to encourage holders of our warrants to exercise, sell additional equity or debt securities or obtain a bank credit facility. The sale of additional equity or debt securities, if convertible, could result in dilution to our stockholders. The incurrence of indebtedness would result in increased fixed obligations and could also result in covenants that would restrict our operations.

Contractual Obligations

The following table sets forth our contractual payment obligations as of December 31, 2017 for the periods indicated below:

Contractual Obligations	Total	Less than 1 Year	1 – 3 Years	3 – 5 Years	More than 5 Years and Thereafter
Operating lease obligations	$ 176,000	$ 132,000	$ 44,000	$ -	$ -
Purchase obligations	$ 8,311,795	$ 5,936,795	$ 2,375,000	$ -	$ -
Total	$ 8,487,795	$ 6,068,795	$ 2,419,000	$ -	$ -

We are a party to license and research and development agreements with universities and other third parties, as well as patent assignment agreements, under which we have obtained rights to patents, patent applications and know-how. We enter into contracts in the normal course of business with CROs for clinical trials, clinical and commercial supply manufacturing, with vendors for preclinical research studies and for other services and products for operating purposes. Our agreements generally provide for termination within 30-60 days of notice. Such agreements are cancelable contracts and not included in the table of contractual obligations and commitments. We have included as purchase obligations our commitments under agreements to the extent they are quantifiable and are not cancelable.

Critical Accounting Policies

Our management's discussion and analysis of our financial condition and results of operations is based on our financial statements, which have been prepared in accordance with accounting principles generally accepted in the United States. The preparation of these financial statements requires us to make estimates and judgments that affect the reported amounts of assets, liabilities and expenses. On an ongoing basis, we evaluate these estimates and judgments, including those described below. We base our estimates on our historical experience and on various other assumptions that we believe to be reasonable under the circumstances. These estimates and assumptions form the basis for making judgments about the carrying values of assets and liabilities that are not readily apparent from other sources. Actual results and experiences may differ materially from these estimates.

While our significant accounting are more fully described in Note 2 to our financial statements included elsewhere in this Annual Report on Form 10-K, we believe that the following accounting policies are the most critical to aid you in fully understanding and evaluating our reported financial results and affect the more significant judgments and estimates that we use in the preparation of our financial statements.

Stock-Based Compensation

We account for stock options according to the Accounting Standards Codification No. 718 (ASC 718) "Compensation - Stock Compensation." Under ASC 718, stock-based compensation cost is measured at grant date, based on the estimated fair value of the award, and is recognized as an expense over the employee's requisite service period on a straight-line basis.

We account for stock options granted to non-employees on a fair value basis using an option pricing method in accordance with ASC 718. The initial non-cash charge to operations for non-employee options with vesting are revalued at the end of each reporting period based upon the change in the fair value of the options and amortized to consulting expense over the related vesting period.

For the purpose of valuing options and warrants granted to our employees, non-employees and directors and officers during the year ended December 31, 2017, 2016 and 2015, we used the Binomial options pricing model. To determine the risk-free interest rate, we utilized the U.S. Treasury yield curve in effect at the time of grant with a term consistent with the expected term of our awards. We estimated the expected life of the options granted based on anticipated exercises in the future periods assuming the success of our business model as currently forecast. The expected dividend yield reflects our current and expected future policy for dividends on our common stock. The expected stock price volatility for our stock options was calculated by examining historical volatilities for publicly traded industry peers as well as considering the Company's historical volatility. We will continue to analyze the expected stock price volatility and expected term assumptions as more historical data for our common stock becomes available. We currently estimate that we will experience a range from 6% - 8% forfeitures for those options currently outstanding.

Off-Balance Sheet Arrangements

CHOP License Agreement and Research Agreement

In November 2014, we entered into a license agreement, or the License Agreement, and a sponsored research agreement, or the Research Agreement, each with CHOP. Under the terms of the License Agreement, CHOP granted us (i) an exclusive, sublicensable license to use certain patent rights covering potential diagnostic and therapeutic targets, (ii) an exclusive, non-sublicensable license to use certain biospecimen and phenotypic data collected from patients with rare and orphan diseases and their family members, or the Biobank. In February 2017, we amended the License Agreement. The amendment allows us to extend the period of our exclusive commercial access to the Biobank for rolling two year periods. The cost of each extension is $125,000 per year. In June 2017, we entered into an amendment to the Research Agreement, which extended the Research Agreement through June 30, 2019, for which payments totaling $5.94 million will be due in 2018 and $2.38 million will be due in 2019. The amendment also allows us to extend the Research Agreement for rolling two year periods in connection with us extending our exclusive commercial access to the Biobank under the License Agreement.

Development and Option Agreement, with Kyowa Hakko Kirin Co., Ltd. (KHK)

In June 2016, we entered into the Development and Option Agreement with KHK pursuant to which we acquired certain rights with respect to the development and potential commercialization of AEVI-002, the Antibody. If we exercise our option under the Development and Option Agreement, KHK has 60 days to select one of two development and commercialization structures as follows:

PLAN A: Co-Development/Co-Commercialization Arrangement

If KHK selects the co-development/co-commercialization arrangement (Plan A), we will have the exclusive right to develop, manufacture and commercialize the Antibody Licensed Products in the Field in the United States and Canada. We will also be responsible for development and regulatory approval of the first Antibody Licensed Product in the European Union and then transferring such regulatory approval to KHK or its designee. We will be responsible for the manufacture of the Antibody Licensed Products for use by the parties in clinical trials as well as for commercialization in their respective fields and/or territories, with KHK purchasing the Antibody Licensed Products from us.

We will be required to pay KHK an initial license fee in the low single-digit millions of dollars upon the co-development/co-commercialization arrangement becoming effective. We may pay KHK up to an additional $18 million upon the achievement of certain regulatory milestones related to the Antibody Licensed Products. The parties will share the anticipated costs of development of the first Antibody Licensed Product in the Field in the United States, Canada and the European Union with us being responsible for any costs in excess of an agreed cap. The parties will split profits from our sales of Antibody Licensed Products in the United States and Canada equally. KHK will pay us low double-digit royalties for sales of Antibody Licensed Products outside the United States and Canada and outside the Field in the United States and Canada.

PLAN B: Licensing Arrangement

If KHK selects the licensing arrangement (Plan B), we will have the exclusive right to develop, manufacture and commercialize the Antibody Licensed Products in the Field in the United States, Canada and the European Union. We will be responsible for the manufacture of the Antibody Licensed Products for use by the parties in clinical trials as well as for commercialization in their respective fields and/or territories.

We will be required to pay KHK an initial license fee in the low single-digit millions of dollars upon the licensing arrangement becoming effective. We may pay KHK up to an additional $28 million upon the achievement of certain regulatory milestones related to the Antibody Licensed Products. The parties will split profits from our sales of Antibody Licensed Products in the United States, Canada and the European Union with us being entitled to approximately 74% of such profits and KHK being entitled to approximately 26% of such profits. KHK will pay us low double-digit royalties for sales of Antibody Licensed Products outside the United States, Canada and the European Union and outside the Field in the United States, Canada and the European Union. We will be responsible for costs of development of Antibody Licensed Products in the United States, Canada and the European Union. KHK will have the right to purchase the Antibody Licensed Products from us.

OCS Agreements

Under agreements with the OCS in Israel regarding research and development projects, our Israeli subsidiary committed to pay royalties to the OCS at rates between 3.5% and 5% of the income resulting from this research and development, at an amount not to exceed the amount of the grants received by our subsidiary as participation in the research and development program, plus interest at LIBOR. The obligation to pay these royalties is contingent on actual income and in the absence of such income no payment is required. As of December 31, 2017, the principal amount of the aggregate contingent liability amounted to approximately $13.97 million.

ITEM 7A - Quantitative and Qualitative Disclosures About Market Risk.

Interest Rate Risk

We have no debt outstanding nor do we have any investments in debt instruments other than highly liquid short-term investments. We invest a major portion of our cash surplus in money market funds in the United States. Given the historic low levels of interest rates, we estimate that a further decline in the interest rate we are receiving will not result in a material adverse effect to our business. Accordingly, we consider our interest rate risk exposure to be insignificant at this time.

ITEM 8 - Financial Statements and Supplementary Data.

AEVI GENOMIC MEDICINE, INC. AND ITS SUBSIDIARY

CONSOLIDATED FINANCIAL STATEMENTS
AS OF DECEMBER 31, 2017

REPORT OF INDEPENDENT REGISTERED PUBLIC ACCOUNTING FIRM

To the Stockholders and Board of Directors of Aevi Genomic Medicine, Inc.

Opinion on the Financial Statements

We have audited the accompanying consolidated balance sheets of Aevi Genomic Medicine, Inc. and its subsidiary (the Company) as of December 31, 2017 and 2016, and the related consolidated statements of operations, stockholders' equity and cash flows for each of the two 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 consolidated financial position of the Company, at December 31, 2017 and 2016, and the consolidated results of their operations and their cash flows for each of the two years in the period ended December 31, 2017, in conformity with U.S. generally accepted accounting principles.

The Company's Ability to Continue as a Going Concern

The accompanying financial statements have been prepared assuming that the Company will continue as a going concern. As discussed in Note 3 to the consolidated financial statements, the Company has incurred operating losses and negative cash flows from operations and has stated that substantial doubt exists about the Company's ability to continue as a going concern. Management's evaluation of the events, conditions, and plans regarding these matters are also described in Note 3. The consolidated financial statements do not include any adjustments that might result from the outcome of this uncertainty.

Basis for Opinion

These financial statements are the responsibility of the Company's management. Our responsibility is to express an opinion on these 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.

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/ Ernst and Young LLP

We have served as the Company's auditor since 2015.

Philadelphia, Pennsylvania

March 13, 2018

REPORT OF INDEPENDENT REGISTERED PUBLIC ACCOUNTING FIRM

To the Stockholders and Board of Directors of

MEDGENICS, INC.

We have audited the accompanying consolidated balance sheet of Medgenics, Inc. ("the Company") and its subsidiary as of December 31, 2015, and the related consolidated statements of operations, stockholders' equity and cash flows for each of the two years in the period ended December 31, 2015. These financial statements are the responsibility of the Company's management. Our responsibility is to express an opinion on these financial statements based on our audits.

We conducted our audits in accordance with the standards of the Public Company Accounting Oversight Board (United States). Those standards require that we plan and perform the audit to obtain reasonable assurance about whether the financial statements are free of material misstatement. An audit includes examining, on a test basis, evidence supporting the amounts and disclosures in the financial statements. An audit also includes assessing the accounting principles used and significant estimates made by management, as well as evaluating the overall financial statement presentation. We believe that our audits provide a reasonable basis for our opinion.

In our opinion, the consolidated financial statements referred to above present fairly, in all material respects, the consolidated financial position of the Company and its subsidiary as of December 31, 20 15 and the consolidated results of their operations and their cash flows for each of the two years in the period ended December 31, 2015, in conformity with accounting principles generally accepted in the United States.

Haifa, Israel
February 26, 2016

/s/ KOST FORER GABBAY & KASIERER
A Member of EY Global

AEVI GENOMIC MEDICINE, INC. AND ITS SUBSIDIARY

CONDENSED CONSOLIDATED BALANCE SHEETS
U.S. dollars in thousands (except share and per share data)

	Note	December 31, 2017	December 31, 2016
ASSETS			
CURRENT ASSETS:			
Cash and cash equivalents	3	$ 33,729	$ 39,838
Prepaid expenses and other current assets		893	335
Total current assets		34,622	40,173
LONG-TERM ASSETS:			
Restricted lease deposits	6(e)	11	11
Property and equipment, net	4	85	377
Other long-term assets		43	-
Total long-term assets		139	388
Total assets		$ 34,761	$ 40,561
LIABILITIES AND STOCKHOLDERS' EQUITY			
CURRENT LIABILITIES:			
Trade payables		$ 943	$ 137
Other accounts payable and accrued expenses	5	3,197	5,446
Total current liabilities		4,140	5,583
Total liabilities		4,140	5,583
COMMITMENTS AND CONTINGENCIES	6		
STOCKHOLDERS' EQUITY:	7		
Common stock - $0.0001 par value; 200,000,000 shares authorized; 59,332,265 shares issued and outstanding at December 31, 2017; 100,000,000 shares authorized; 37,112,343 shares issued and 37,103,843 shares outstanding at December 31, 2016		6	4
Additional paid-in capital		245,593	215,008
Accumulated deficit		(214,978)	(180,034)
Total stockholders' equity		30,621	34,978
Total liabilities and stockholders' equity		$ 34,761	$ 40,561

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

AEVI GENOMIC MEDICINE, INC. AND ITS SUBSIDIARY

CONDENSED CONSOLIDATED STATEMENTS OF OPERATIONS
U.S. dollars in thousands (except share and per share data)

	Note	Year ended December 31, 2017	2016	2015
Research and development expenses		$ 25,176	$ 28,552	$ 18,356
Less:				
Participation by the Office of the Chief Scientist	6(d)	-	(196)	(2,912)
Research and development expenses, net		25,176	28,356	15,444
Non-recurring research and development expenses resulting from acquisition		-	-	8,170
General and administrative expenses		9,524	13,523	12,954
Operating loss		(34,700)	(41,879)	(36,568)
Financial expenses		(41)	(24)	(1,408)
Financial income		27	15	1
Loss before taxes on income		(34,714)	(41,888)	(37,975)
Taxes on income	8	-	16	17
Net loss		$ (34,714)	$ (41,904)	$ (37,992)
Basic loss per share	11	$ (0.83)	$ (1.19)	$ (1.42)
Diluted loss per share	11	$ (0.83)	$ (1.19)	$ (1.45)
Weighted average number of shares of common stock used in computing basic loss per share		41,675,814	35,161,823	26,783,623
Weighted average number of shares of common stock used in computing diluted loss per share		41,675,814	35,161,823	26,846,270

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

AEVI GENOMIC MEDICINE, INC. AND ITS SUBSIDIARY

CONDENSED CONSOLIDATED STATEMENTS OF CHANGES IN STOCKHOLDERS' EQUITY
U.S. dollars in thousands (except share and per share data)

	Common stock		Additional paid-in capital	Accumulated deficit	Total stockholders' equity
	Shares	Amount			
Balance as of December 31, 2014	24,818,075	$ 3	$ 129,797	$ (100,138)	29,662
Issuance of common stock at $6.50 per share, net	7,078,250	1	42,881	-	42,882
Stock-based consideration related to acquisition	459,770	(*)	3,200	-	3,200
Stock-based compensation related to the issuance and vesting of restricted common stock to directors	24,500	(*)	-	-	-
Stock-based compensation related to options and warrants granted to consultants, directors and employees	-	-	9,188	-	9,188
Exercise of warrants and options	480,122	(*)	3,410	-	3,410
Net loss	-	-	-	(37,992)	(37,992)
Balance as of December 31, 2015	32,860,717	$ 4	$ 188,476	$ (138,130)	50,350
Issuance of common stock at $5.50 per share, net	3,835,261	-	19,563	-	19,563
Stock-based compensation related to options and warrants granted to consultants, directors and employees	-	-	6,788	-	6,788
Exercise of warrants and options	407,865	(*)	181	-	181
Net loss	-	-	-	(41,904)	(41,904)
Balance as of December 31, 2016	37,103,843	$ 4	$ 215,008	$ (180,034)	34,978
Issuance of common stock at $1.26 per share, net	22,222,222	2	26,968	-	26,970
Stock-based compensation related to options and warrants granted to consultants, directors and employees	-	-	3,368	-	3,368
Exercise of warrants and options	6,200	(*)	19	-	19
Cumulative-effect adjustment from adoption of ASU 2016-09	-	-	230	(230)	-
Net loss	-	-	-	(34,714)	(34,714)
Balance as of December 31, 2017	59,332,265	$ 6	$ 245,593	$ (214,978)	30,621

(*) Represents an amount lower than $1.

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

CONDENSED CONSOLIDATED STATEMENTS OF CASH FLOWS
U.S. dollars in thousands

	Year ended December 31		
	2017	2016	2015
CASH FLOWS FROM OPERATING ACTIVITIES:			
Net loss	$ (34,714)	$ (41,904)	$ (37,992)
Adjustments to reconcile loss to net cash used in operating activities:			
Depreciation	112	268	240
Loss from disposal of property and equipment	32	-	18
Stock-based compensation related to options, warrants and restricted shares granted to employees, directors and consultants	3,368	6,788	9,188
Stock-based consideration related to acquisition	-	-	3,200
Changes in fair value of warrants classified as a liability	-	-	1,370
Accrued severance pay, net	-	-	(269)
Change in operating assets and liabilities:			
Prepaid and other current assets	(558)	412	(432)
Trade payables	806	(1,185)	246
Other accounts payable and accrued expenses	(2,249)	2,860	24
Restricted lease deposits	-	12	60
Other long-term assets	(43)	-	-
Net cash used in operating activities	$ (33,246)	$ (32,749)	$ (24,347)
CASH FLOWS FROM INVESTING ACTIVITIES:			
Purchase of property and equipment	$ (4)	$ (221)	$ (187)
Proceeds from disposal of property and equipment	152	-	-
Net cash provided by (used in) investing activities	$ 148	$ (221)	$ (187)

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

AEVI GENOMIC MEDICINE, INC. AND ITS SUBSIDIARY

CONDENSED CONSOLIDATED STATEMENTS OF CASH FLOWS
U.S. dollars in thousands

	Year ended December 31		
	2017	2016	2015
CASH FLOWS FROM FINANCING ACTIVITIES:			
Proceeds from issuance of shares and warrants, net	$ 26,970	$ 19,563	$ 42,882
Proceeds from exercise of options and warrants	19	181	1,428
Net cash provided by financing activities	$ 26,989	$ 19,744	$ 44,310
Increase (decrease) in cash and cash equivalents	(6,109)	(13,226)	19,776
Balance of cash and cash equivalents at the beginning of the period	39,838	53,064	33,288
Balance of cash and cash equivalents at the end of the period	$ 33,729	$ 39,838	$ 53,064
Supplemental disclosure of cash flow information:			
Cash paid during the period for taxes	$ -	$ 26	$ 19
Supplemental disclosure of non-cash flow information:			
Classification of liability in respect of warrants into equity due to the exercise of warrants	-	$ -	$ 1,982

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

NOTE 1: GENERAL

a. Aevi Genomic Medicine Inc., formerly Medgenics Inc., (the "Company") was incorporated in January 2000 in Delaware. The Company has a wholly-owned subsidiary, Medgenics Medical Israel Ltd. (the "Subsidiary"), which was incorporated in Israel in March 2000. The Company is a clinical stage biopharmaceutical company with an emphasis on genomic medicine.

The Company's common stock is traded on the NASDAQ. Prior to October 21, 2016 the Company's common stock was traded on the NYSE.

b. As reflected in the accompanying financial statements, the Company incurred a net loss for the twelve month period ended December 31, 2017 of $34,714 and had negative cash flow from operating activities of $33,246 during the twelve month period ended December 31, 2017. The accumulated deficit as of December 31, 2017 is $214,978. The Company and the Subsidiary have not yet generated revenues from product sales.

NOTE 2: SIGNIFICANT ACCOUNTING POLICIES

The consolidated financial statements are prepared in accordance with United States Generally Accepted Accounting Principles ("U.S. GAAP"), applied on a consistent basis, as follows:

a. Use of estimates:

The preparation of financial statements in conformity with U.S. GAAP requires management to make estimates and assumptions. The Company's management believes that the estimates and assumptions used are reasonable based upon information available at the time they are made. These estimates and assumptions can affect the amounts reported in the financial statements and accompanying notes. Actual results could differ from those estimates.

b. Financial statements in U.S. dollars:

The Company's management believes that the dollar is the primary currency of the economic environment in which the Company and its Subsidiary operate. Thus, the functional currency of the Company and the Subsidiary is the dollar.

Accordingly, transactions and balances denominated in dollars are presented at their original amounts. Non-dollar transactions and balances have been re-measured to dollars, in accordance with ASC 830, *"Foreign Currency Matters"* of the Financial Accounting Standards Board ("FASB"). All exchange gains and losses from re-measurement of monetary balance sheet items denominated in non-dollar currencies are reflected in the Statements of Operations as financial income or expenses, as appropriate.

c. New accounting pronouncements:

In 2016, the FASB issued ASU 2016-02, Leases (Topic 842), which will establish the principles that lessees and lessors shall apply to report useful information to users of financial statements about the amount, timing, and uncertainty of cash flows arising from a lease. The pronouncement is effective for fiscal years beginning after December 15, 2018. The Company is currently evaluating the effect this guidance will have on the Company's financial statements.

In 2016, the FASB issued ASU 2016-09, Compensation – Stock Compensation (Topic 718), Improvements to Employee Share-Based Payment Accounting, which is meant to reduce the complexity involving several aspects of the accounting for employee share-based payment transactions, including the income tax consequences, classifications of awards as either equity or liabilities, and classification on the statement of cash flows. ASU 2016-09 became effective for the Company in the first quarter 2017 and was applied using a modified retrospective transition approach. Under ASU 2016-09 the Company has elected to no longer estimate forfeiture rates in determining its stock compensation expense and will true up for forfeitures as they occur. As a result of the adoption, the Company recorded a cumulative adjustment to accumulated deficit as of December 31, 2016 for $230.

Other accounting standards that have been issued or proposed by the FASB or other standards-setting bodies that do not require adoption until a future date are not expected to have a material impact on the Company's consolidated financial statements upon adoption.

d. Principles of consolidation:

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

e. Cash equivalents:

The Company and the Subsidiary consider all highly liquid investments originally purchased with maturities of three months or less to be cash equivalents.

f. Property and equipment:

Property and equipment are stated at cost net of accumulated depreciation. Depreciation is computed using the straight-line method over the estimated useful lives of the assets.

The annual rates of depreciation are as follows:

	%
Computers and peripheral equipment	33
Leasehold improvements	The shorter of term of the lease or the useful life of the asset

g. Impairment of long-lived assets:

Long-lived assets are reviewed for impairment in accordance with ASC 360, "*Property, Plant, and Equipment*" ("ASC 360"), whenever events or changes in circumstances indicate that the carrying amount of an asset may not be recoverable. Recoverability of an asset to be held and used is measured by a comparison of the carrying amount of the asset to the future undiscounted cash flows expected to be generated by the asset. If such an asset is considered to be impaired, the impairment to be recognized is measured by the amount by which the carrying amount of the asset exceeds the fair value of the asset. No impairment charges have been recognized through December 31, 2017.

h. Income taxes:

The Company accounts for income taxes in accordance with ASC 740, "*Income Taxes*" ("ASC 740"). ASC 740 prescribes the use of the asset and liability method whereby deferred tax assets and liabilities 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 reverse. The Company provides a valuation allowance, if necessary, to reduce deferred tax assets to their estimated realizable value. As of December 31, 2017, a full valuation allowance was provided by the Company.

The Company also accounts for income taxes in accordance with ASC 740-10, "*Accounting for Uncertainty in Income Taxes*" ("ASC 740-10"). ASC 740-10 contains a two-step approach for recognizing and measuring uncertain tax positions accounted for in accordance with ASC 740-10. 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, 2016 and 2017, no liability has been recorded as a result of ASC 740-10.

On December 22, 2017, the U.S. government enacted comprehensive tax legislation commonly referred to as the Tax Cuts and Jobs Act (the "Tax Act"). The Tax Act makes broad and complex changes to the U.S. tax code, including, but not limited to, reducing the U.S. federal corporate tax rate from 35 percent to 21 percent; eliminating the corporate alternative minimum tax (AMT) and changing how existing AMT credits can be realized; creating a new limitation on deductible interest expense; changing rules related to uses and limitations of net operating loss carryforwards created in tax years beginning after December 31, 2017; limitations on the deductibility of certain executive compensation; and changes to the calculation of the orphan drug credit.

i. Accounting for stock based compensation:

The Company applies ASC 718, "*Compensation-Stock Compensation*" ("ASC 718") which requires the measurement and recognition of compensation expense based on estimated fair values for all share-based payment awards made to employees and directors.

The Company recognizes compensation expenses for awards granted based on the straight-line method over the requisite service period of each of the grants.

In 2015, 2016 and 2017, the Company estimated the fair value of stock options granted to employees and directors using the Binominal options pricing model with the following assumptions:

	2017	2016	2015
Dividend yield	0%	0%	0%
Expected volatility	72.0-78.6%	73.1-75.4%	75.7-78.3%
Risk-free interest rate	2.2-2.5%	1.5-1.9%	1.9-2.3%
Suboptimal exercise factor	1.5-2.5	1.5-2.5	1.5-2.5
Contractual life (years)	10	10	10
Exit rate	6-8%	8%	8%

The Company uses historical data to estimate post vesting exit rate within the valuation model; separate groups of employees that have similar historical exercise behavior are considered separately for valuation purposes.

The suboptimal exercise factor represents the value of the underlying stock as a multiple of the exercise price of the option which, if achieved, results in exercise of the option.

The risk-free interest rate assumption is based on observed interest rates appropriate for the term of the Company's stock options.

The Company has historically not paid dividends and has no foreseeable plans to pay dividends.

Prior to the fourth quarter of 2017, the expected stock price volatility of the Company's stock options had been calculated by examining historical volatilities for publicly traded industry peers as well as considering the Company's historical volatility. As of the fourth quarter of 2017, the company determined there was enough historical data to begin computing the expected price volatility based on the Company's historical data, alone. We currently estimate that we will experience a range from 6% - 8% forfeitures for those options currently outstanding.

The Company applies ASC 718 and ASC 505-50, "*Equity-Based Payments to Non-Employees*" ("ASC 505-50"), with respect to options issued to non-employees. ASC 718 requires the use of option valuation models to measure the fair value of the options. The fair value of these options was estimated at grant date and at the end of each reporting period, using the Binomial option pricing model with the following assumptions:

	2017	2016	2015
Dividend yield	0%	0%	0%
Expected volatility	78-79%	59-75%	69-75%
Risk-free interest rate	2.3-2.4%	0.9-1.7%	1.1-2.3%
Contractual life (years)	9.0-9.9	2.8-9.8	3.8-9.8

Prior to the fourth quarter of 2017, the expected stock price volatility of the Company's stock options had been calculated by examining historical volatilities for publicly traded industry peers as well as considering the Company's historical volatility. As of the fourth quarter of 2017, the company determined there was enough historical data to begin computing the expected price volatility based on the Company's historical data, alone. The Company expects to continue using this methodology going forward.

j. Loss per share:

Basic loss per share is computed based on the weighted average number of shares of common stock outstanding during each year. Diluted loss per share is computed based on the weighted average number of shares of common stock outstanding during each year, plus the dilutive effect of options, warrants and restricted shares considered to be outstanding during each year, in accordance with ASC 260, "*Earnings Per Share* " ("ASC 260").

k. Research and development expenses, net:

All research and development expenses are charged to the Consolidated Statements of Operations as incurred.

These costs include, but are not limited to, license fees related to the acquisition of in-licensed products; employee-related expenses, including salaries, benefits and travel; expenses incurred under agreements with clinical research organizations and investigative sites that conduct clinical trials and preclinical studies; the cost of acquiring, developing and manufacturing clinical trial materials; facilities, depreciation and other expenses, which include direct and allocated expenses for rent and maintenance of facilities, insurance and other supplies; and costs associated with preclinical activities and regulatory operations.

Costs for certain development activities, such as clinical trials, are recognized based on an evaluation of the progress to completion of specific tasks using data such as patient enrollment, clinical site activations, or information provided to the Company by its vendors with respect to their actual costs incurred. Payments for these activities are based on the terms of the individual arrangements, which may differ from the pattern of costs incurred, and are reflected in the consolidated financial statements as prepaid or accrued research and development expense, as the case may be.

Grants from the Office of the Chief Scientist in Israel ("OCS") related to such research and development expenses are offset against the expense at the later of when receipt is assured or the expenses are incurred.

l. Grants:

Royalty-bearing grants from the OCS for funding approved research and development projects are recognized at the time the Subsidiary is entitled to such grants, on the basis of the costs incurred, and are presented as a deduction from research and development expenses.

m. Concentrations of credit risks:

Financial instruments that potentially subject the Company and the Subsidiary to concentrations of credit risk consist principally of cash and cash equivalents.

Cash and cash equivalents are invested in major banks and financial institutions in the United States. Such deposits in the United States may be in excess of insured limits and are not insured in other jurisdictions. Management believes that the financial institutions that hold the Company's investments are institutions with high credit standing and accordingly, minimal credit risk exists with respect to these investments.

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

n. Fair value of financial instruments:

The carrying amount of cash and cash equivalents, accounts payable and accrued liabilities are generally considered to be representative of their respective fair values because of the short-term nature of those accounts.

NOTE 3: LIQUIDITY RISKS AND MANAGEMENT PLANS

The future success of the Company is dependent on its ability to develop its product candidates and ultimately upon its ability to attain profitable operations. The Company is subject to a number of risks similar to other early-stage life science companies, including, but not limited to, successful discovery and development of its product candidates, raising additional capital with favorable terms, and development by its competitors of new technological innovations, protection of proprietary technology and market acceptance of the Company's products. The successful discovery and development of product candidates requires substantial working capital which may not be available to the Company on favorable terms.

The Company has financed its operations primarily through issuance of equity and grants from other third parties. As of December 31, 2017, the Company had cash and cash equivalents of $33,729 and liabilities of $4,140. The Company has incurred recurring operating losses since inception. For the year ended December 31, 2017, the Company incurred a net loss of $34,714 and as of December 31, 2017 the Company has an accumulated deficit of $214,978. The Company anticipates operating losses to continue for the foreseeable future due to, among other things, costs related to research, development of its product candidates and its preclinical programs, and its administrative organization. The Company will require substantial additional financing to fund its operations and to continue to execute its strategy. These conditions raise substantial doubt about its ability to continue as a going concern within one year after the date that the financial statements are issued.

To alleviate the conditions that raise substantial doubt about the Company's ability to continue as a going concern, management is exploring various sources of funding such as strategic collaborations, license agreements, and issuance of equity and/or debt securities. If the Company raises additional funds through strategic collaborations and alliances or licensing agreements with third parties, which may include existing collaboration partners, the Company may have to relinquish valuable rights to its technologies or product candidates, including AEVI-001 and AEVI-002, or grant licenses on terms that are not favorable to the Company. To the extent that the Company raises additional capital through the sale of equity, the ownership interest of its existing shareholders will be diluted and other preferences may be necessary that adversely affect the rights of existing shareholders. If none of these alternatives is available, or if available, the Company is unable to raise sufficient capital through such transactions, it will not have sufficient cash resources and liquidity to fund its business operations for at least the next year following the date that the financial statements are issued. Accordingly, management has concluded that substantial doubt exists with respect to the Company's ability to continue as a going concern within one year after the date that the financial statements are issued.

NOTE 4: PROPERTY AND EQUIPMENT, NET

Composition of property and equipment is as follows:

	December 31,	
	2017	2016
Cost:		
Furniture and office equipment	$ -	$ 109
Computers and peripheral equipment	35	172
Laboratory equipment	-	781
Leasehold improvements	157	740
Total cost	192	1,802
Total accumulated depreciation	107	1,425
Depreciated cost	$ 85	$ 377

Depreciation expenses for the years ended December 31, 2017 and 2016 amounted to $112 and $268, respectively.

During the year ended December 31, 2017, the Company disposed of assets associated with the closure of the Israel site resulting in $152 of proceeds and the write down of assets and associated accumulated depreciation of $1,610 and $1,426, respectively.

NOTE 5: OTHER ACCOUNTS PAYABLE AND ACCRUED EXPENSES

	December 31,	
	2017	2016
Employees and payroll accruals	$ 1,297	$ 2,247
R&D accruals	1,539	2,403
Accrued expenses, other	361	796
Other accounts payable and accrued expenses	$ 3,197	$ 5,446

NOTE 6: COMMITMENTS AND CONTINGENCIES

a. In November 2014, the Company entered into a license agreement, or the License Agreement, and a sponsored research agreement, or the Research Agreement, each with CHOP. Under the terms of the License Agreement, CHOP granted the Company (i) an exclusive, sublicensable license to use certain patent rights covering potential diagnostic and therapeutic targets, (ii) an exclusive, non-sublicensable license to use certain biospecimen and phenotypic data collected from patients with rare and orphan diseases and their family members, or the Biobank. In February 2017, the Company amended the License Agreement. The amendment allows the Company to extend the period of its exclusive commercial access to the Biobank for rolling two year periods. The cost of each extension is $125 per year. In June 2017, the Company entered into an amendment to the Research Agreement, which extended the Research Agreement through June 30, 2019, for which payments totaling $5,937 will be due in 2018 and $2,375 will be due in 2019. The amendment also allows the Company to extend the Research Agreement for rolling two year periods in connection with it extending its exclusive commercial access to the Biobank under the License Agreement. As of December 31, 2017, the Company has total payables related to the CHOP sponsored research agreement of $1,048, allocated between accrued expenses and trade payables.

b. In 2015, the Company entered into an Equity Interest Purchase Agreement (the "Purchase Agreement") with neuroFix Therapeutics, Inc., a Delaware corporation ("Legacy Corp.), neuroFix, LLC, a Delaware limited liability company ("neuroFix"), CHOP, Philip Harper, an individual, and Hakon Hakonarson, an individual, pursuant to which the Company acquired all of the equity interests of neuroFix. Immediately prior to the execution of the Purchase Agreement, Legacy Corp. had contributed its assets to neuroFix.

Under the terms of the Purchase Agreement, Legacy Corp., neuroFix, CHOP, Harper and Hakonarson agreed to consummate the neuroFix Acquisition in consideration for certain upfront, milestone and earnout payments related to certain product sales by the Company. The payments made or to be made by the Company are as follows:

- an upfront payment of $2,000 in cash paid upon the consummation of the neuroFix Acquisition;
- a payment of $6,000 payable as $2,800 in cash and $3,200 in the Company's common stock, upon the earlier to occur of (i) the achievement of a corporate milestone and (ii) March 31, 2016. In October 2015, the cash payment was made and 459,770 shares of common shares were issued;

- additional payments of up to $450,000 upon the achievement of certain developmental, regulatory and sales milestones; and
- earnout payments equal to a percentage of certain product sales by the Company using tiered rates ranging from the mid-to-high single digits.

In addition to the foregoing, in the event a certain product is approved by the FDA for additional indications beyond the initial indication, additional payments of $25,000 for each such additional indication shall be paid by the Company to Legacy Corp. and CHOP.

As a result of a Legacy Corp lacking outputs necessary to be considered a business as that term is defined in ASC 805 – Business Combination, the acquisition was determined not to be a business since no significant processes were acquired. Therefore, the transaction was treated as an asset acquisition. The Company determined that the acquired assets can only be economically used for the specific and intended purpose and have no alternative future use after taking into consideration that further research and development, regulatory marketing approval efforts will be required to reach technological feasibility. Accordingly, the entire initial purchase consideration of $8,170 was immediately expensed to non-recurring research and development expense.

c. License agreements:

1. In 2014 the Subsidiary entered into a license agreement with the CHOP (License Agreement). According to the agreement, CHOP granted an exclusive license to the Subsidiary to use the rare and orphan disease samples at the Center for Applied Genomics biobank (Biobank) for the purpose of developing and commercializing therapeutic treatments and diagnostic targets for rare and orphan diseases.

   A License Issuance Fee of $500 was paid and expensed in 2014. Beginning in 2016 for a period of five years the Company will pay to CHOP an annual license maintenance fee of $100. For the remainder of the term of the agreement the Company shall pay an annual license fee of $200. The Company will pay to CHOP certain milestone payments, ranging from $250 to $500; low single-digit royalties on net sales of all licensed products and a percentage of amounts received from sublicensing activities.

   The License Agreement terminates upon the expiration date of the last-to-expire royalty term under the License Agreement. The Company may terminate the License Agreement at any time with six months' prior written notice to CHOP, and CHOP may terminate the License Agreement upon (i) an uncured default by the Company of the License Agreement, (ii) the failure by the Company to meet certain development and/or commercialization milestones under the License Agreement, or (iii) the Company becoming insolvent or entering into bankruptcy proceedings.

2. Immediately prior to and in connection with the Purchase Agreement, neuroFix entered into a License Agreement (the "License Agreement") with CHOP, pursuant to which CHOP would license to neuroFix certain technology owned and controlled by CHOP related to ADHD and certain other neurological and neuropsychological indications. Pursuant to the License Agreement, CHOP licensed to neuroFix (coupled with a right to sublicense) certain patent rights and compound know-how on an exclusive, worldwide, royalty-bearing right and license basis, and certain CHOP know-how (other than compound know-how) on a non-exclusive, worldwide, royalty-bearing right and license basis. CHOP also granted to neuroFix an exclusive option during the term of the License Agreement to negotiate an exclusive license to certain CHOP intellectual property.

Pursuant to the License Agreement, CHOP retained rights to the licensed patent rights and know-how to conduct teaching, educational, research and patient care activities itself and to conduct collaborations with certain not-for-profit, governmental, educational or non-commercial third parties and for purposes outside of the field of the license. Under the License Agreement, neuroFix granted to CHOP a non-exclusive, worldwide, fully paid-up, royalty-free license under all intellectual property rights controlled by neuroFix to make and use certain products for education and non-commercial research purposes.

In addition to neuroFix having issued equity to CHOP in partial consideration for the rights granted under the License Agreement (which equity was issued immediately prior to the Purchase Agreement described above), CHOP is eligible for certain milestone and royalty payments under the License Agreement as further described below:

- up to $1,500 in regulatory and sales milestone payments in connection with each FDA-approved indication obtained by neuroFix utilizing intellectual property licensed under the License Agreement;
- royalty payments equal to a percentage of certain product sales by neuroFix using a fluctuating rate in the low single digits (adjusted downward to the extent third party royalty payments exceed a certain percentage in a given calendar quarter);
- annual maintenance fees of equal to or less than $100 depending on the year; and
- a certain percentage (ranging from mid-single digits to the mid-teens depending on if other rights of neuroFix are also licensed to the sublicensee at the same time) of all sublicensee income (except any amounts attributable to sublicensed sales by a certain party in Japan).

The License Agreement will terminate, with respect to each product and each territory covered by the License Agreement, upon the later of (i) the expiration of the certain CHOP patent rights and (ii) January 1, 2025, at which time the license rights granted to neuroFix become perpetual, irrevocable, fully paid-up and royalty-free. The License Agreement could also be subject to termination by CHOP if neuroFix is not achieving certain specified development plans and diligence events and is not undertaking commercially reasonable efforts to achieve such events.

3. In June 2016, the Company entered into a Clinical Development and Option Agreement, or the Development and Option Agreement, with Kyowa Hakko Kirin Co., Ltd., or KHK, relating to the development and potential commercialization of KHK's first-in-class anti-LIGHT monoclonal antibody, or the Antibody (AEVI-002). Under the Development and Option Agreement, the Company received an exclusive option for exclusive rights to develop and commercialize products containing the Antibody, or the Licensed Products, and to conduct various development activities with respect to the Antibody, including the conduct of a signal finding study testing the Antibody in Severe Pediatric Onset Inflammatory Bowel Disease, or the Study.

For a certain period of time after the completion of the Study, or the Exercise Period, the Company will have the option, or the Option, to obtain exclusive rights for the development and commercialization of the Antibody. If the Company exercises the Option, KHK will have 60 days to select one of two potential development and commercialization structures: a co-development/co-commercialization arrangement or a licensing arrangement.

If, upon the Company's exercise of the Option, KHK chooses to continue the collaboration as a co-development/co-commercialization arrangement, the Company will have the exclusive right to develop, manufacture and commercialize the Licensed Products in the United States and Canada. The Company will be required to pay KHK an initial license fee in the low single-digit millions of dollars and may pay KHK up to an additional $18,000 upon the achievement of certain regulatory milestones related to the Licensed Products. The parties will share the anticipated costs of development of the first Licensed Product for the treatment, prevention, and diagnosis of specified pediatric onset rare and orphan inflammatory diseases (including severe pediatric onset inflammatory bowel diseases such as Crohn's disease and ulcerative colitis, or IBD) and other specified pediatric onset rare and orphan auto-immune diseases, or, collectively, the Field, in the United States, Canada and the European Union with the Company responsible for any costs in excess of an agreed cap.

If, upon the exercise of the Option, KHK chooses to continue the collaboration as a licensing arrangement, the Company will have the exclusive right to develop, manufacture and commercialize the Licensed Products in the Field in the United States, Canada and the European Union. The Company will be required to pay KHK an initial license fee in the low single-digit millions of dollars and may pay KHK up to an additional $28,000 upon the achievement of certain regulatory milestones related to the Licensed Products.

d. Office of the Chief Scientist (OCS):

Under agreements with the OCS in Israel regarding research and development projects, the Subsidiary is committed to pay royalties to the OCS at rates between 3.5% and 5% of the commercial revenues resulting from this research and development, at an amount not to exceed the amount of the grants received by the Subsidiary as participation in the research and development program, plus interest at LIBOR. The obligation to pay these royalties is contingent on actual income and in the absence of such income no payment is required. As of December 31, 2017, the principal amount of the aggregate contingent liability was $13,968. The Subsidiary was not approved a grant from the OCS for 2016 and 2017.

e. Lease Agreements:

1. The offices of the Company are rented under an operating lease agreement and committed through April 2019. Future minimum lease commitment under the existing operating lease agreement is $176.

2. The following table sets forth our lease payment obligations as of December 31, 2017 for the periods indicated below:

	Total	Less than 1 Year	1 – 3 Years	3 – 5 Years	More than 5 Years and Thereafter
Operating lease obligations	$ 176	$ 132	$ 44	$ -	$ -

f. Per the employment agreements of several executives, if terminated without cause, these executives will be entitled to severance pay in the aggregate amount of $2,627.

NOTE 7: STOCKHOLDERS' EQUITY

a. Common stock:

The common stock confers upon the holders the right to receive notice to participate and vote in annual and special meetings of the stockholders of the Company and the right to receive dividends, if declared.

b. Issuance of shares, stock options and warrants to investors:

1. In June 2016, the Company completed an underwritten public offering of 3,835,261 shares of common stock, including 195,261 shares sold pursuant to the partial exercise of an option granted to the underwriters to purchase additional shares of common stock. The shares were offered to the public at a price of $5.50 per share. Gross proceeds were $21,094 or approximately $19,563 in net proceeds after deducting underwriting discounts and commissions of $1,266 and other offering costs of approximately $265.

2. In October 2017, the Company completed a private offering of an aggregate of 22,222,222 shares of common stock, and warrants exercisable for up to an aggregate of 3,953,904 shares of common stock at a purchase price of $1.26 per share of common stock and accompanying warrants pursuant to that certain securities purchase agreement dated as of August 9, 2017. Each purchaser received a warrant exercisable to purchase a pro rata amount of shares of common stock at a purchase price of $2.84 per share, which will expire five years after the date of issuance. The Company has accounted for these warrants under the equity method in accordance with ASC 815. The aggregate gross proceeds from the offering to the Company were $28,000, of which $20,000 was proceeds received from the CHOP Foundation and $1,000 was proceeds received from directors and officers. The CHOP Foundation was issued 15,873,016 shares of common stock and accompanying warrants of 2,824,217. Net proceeds after deducting estimated offering expenses were $26,970.

   The Company also obtained approval from stockholders to increase the total number of authorized shares of Common Stock from 100,000,000 to 200,000,000 shares.

c. Issuance of stock options, warrants and restricted stock to employees and directors:

1. In 2006, the Company adopted a stock incentive plan (the "stock incentive plan") according to which options, restricted stock and other awards related to common stock of the Company may be granted to directors, employees and consultants (non-employees) of the Company and the Subsidiary, as determined by the Company's Board of Directors from time to time. The options outstanding are exercisable within a designated period from the date of grant and at an exercise price, each as determined by the Company's Board of Directors. The options outstanding to employees, directors and consultants will vest over a period of up to four years from the date of grant. Any option which is cancelled or forfeited before expiration becomes available for future grants.

2. In March 2013, the Company's Board of Directors approved an amendment to the stock incentive plan increasing the number of shares of common stock authorized for issuance thereunder to a total of 4,178,571 shares of common stock.

3. In the year ended December 31, 2015, upon retirement of two directors from the Board of Directors, the Compensation Committee of the Board of Directors approved to allow their options to vest pursuant to their original terms and all their options will have a 10 year contractual life from the date of being granted.

   As a result of the modification, the Company recorded incremental compensation cost of $1,427 on the modification date. The fair value was estimated using Binomial model with the following weighted-average assumptions: expected stock price volatility range of 70.5%-78.6%, risk-free interest rate of 1.6%-2.0% and expected dividend yield of 0%.

   No future compensation will be recorded on these options.

   In addition, in the year ended December 31, 2015, the Compensation Committee of the Board of Directors approved two employees' options to vest upon termination and all their options will have a 10 year contractual life from the date of being granted.

   As a result of the modification, the Company recorded incremental compensation cost of $266 and $188 in 2015 and 2016, respectively. The fair value was estimated using Binomial model with the following weighted-average assumptions: expected stock price volatility range of 70.1%-79.8%, risk-free interest rate of 1.4%-2.5% and expected dividend yield of 0%.

   The Company accounted for the above changes in terms of the options under the provisions of ASC 718 as modifications. A modification to the terms of an award should be treated as an exchange of the original award for a new award with total compensation cost equal to the grant-date fair value of the original award plus the incremental value measured at the same date. Under ASC 718, the calculation of the incremental value is based on the excess of the fair value of the (modified) award based on current circumstances over the fair value of the original option measured immediately before its terms are modified based on current circumstances. That is, the original (pre-modification) award will be valued based on current assumptions, without regard to the assumptions made on the grant date.

4. In April 2014, stockholders approved an amendment to the Company's Stock Incentive Plan, increasing the number of shares authorized to be issued under such plan by 2,000,000 shares. In April 2016, stockholders approved an amendment to the Company's Stock Incentive Plan, increasing the number of shares authorized to be issued under such plan by 3,000,000 shares. A summary of the Company's activity for options and warrants granted to employees and directors is as follows:

	Number of options and warrants	Weighted average exercise price	Weighted average remaining contractual terms (years)	Aggregate intrinsic value
Outstanding at December 31, 2016	10,433,396	$ 5.78	6.08	$ 3,383
Granted	3,749,110	$ 2.49		
Exercised	(6,200)	$ 3.14		
Forfeited	(3,065,944)	$ 6.97		
Outstanding at December 31, 2017	11,110,362	$ 4.34	6.43	$ 1
Vested and expected to vest, December 31, 2017	10,791,362	$ 4.43	6.33	$ 1
Exercisable at December 31, 2017	6,910,155	$ 5.12	4.80	$ -

As of December 31, 2017, there was $4,798 of total unrecognized compensation cost related to non-vested share-based compensation arrangements granted to employees and directors. That cost is expected to be recognized over a weighted-average period of 1.89 years.

d. Issuance of shares, stock options and warrants to consultants:

1. A summary of the Company's activity for options granted under the stock incentive plan and warrants to consultants is as follows:

	Number of options and warrants	Weighted average exercise price	Weighted average remaining contractual terms (years)	Aggregate intrinsic value
Outstanding at December 31, 2016	390,821	$ 6.95	1.43	$ 114
Granted	25,000	$ 1.23		
Exercised	-	$ -		
Forfeited	(255,821)	$ 8.47		
Outstanding at December 31, 2017	160,000	$ 3.62	2.45	$ -
Exercisable at December 31, 2017	135,000	$ 4.07	1.07	$ -

As of December 31, 2017, all compensation cost related to share-based compensation arrangements granted to consultants was recognized.

e. Compensation expenses:

Compensation expense related to shares, warrants and options granted to employees, directors and consultants was recorded in the Consolidated Statements of Operations in the following line items:

	Year ended December 31,		
	2017	2016	2015
Research and development expenses	$ 1,515	$ 2,170	$ 2,087
General and administrative expenses	1,853	4,618	7,101
	$ 3,368	$ 6,788	$ 9,188

f. Summary of shares to be issued upon exercise of options and warrants:

A summary of shares to be issued upon exercise of all the options and warrants, segregated into ranges, as of December 31, 2017 is presented in the following table:

		As of December 31, 2017		
Options / Warrants	Exercise Price per Share ($)	Shares to be Issued upon Exercise of Options and Warrants Outstanding	Shares to be Issued upon Exercise of Options and Warrants Exercisable	Weighted Average Remaining Contractual Terms of Options and Warrants Outstanding (in years)
Options:				
Granted to Employees and Directors				
	1.07-2.66	2,334,000	60,000	9.5
	3.14-5.07	5,822,567	4,191,668	5.8
	5.22-8.80	2,812,585	2,517,277	5.4
		10,969,152	6,768,945	
Granted to Consultants	1.23	25,000	-	9.9
	4.82	10,000	10,000	8.8
		35,000	10,000	
Total Shares to be Issued upon Exercise of Options		11,004,152	6,778,945	
Warrants:				
Granted to Employees and Directors	2.84	141,210	141,210	4.8
Granted to Consultants	3.76-4.99	125,000	125,000	0.4
Granted to Investors	2.84	3,812,694	3,812,694	4.8
	6.78	3,124,319	3,124,319	0.1
		6,937,013	6,937,013	
Total Shares to be Issued upon Exercise of Warrants		7,203,223	7,203,223	
Total Shares to be Issued upon Exercise of Options and Warrants		18,207,375	13,982,168	

NOTE 8: TAXES ON INCOME

On December 22, 2017, the U.S. government enacted comprehensive tax legislation commonly referred to as the Tax Cuts and Jobs Act (the "Tax Act"). The Tax Act makes broad and complex changes to the U.S. tax code, including, but not limited to, reducing the U.S. federal corporate tax rate from 35 percent to 21 percent; eliminating the corporate alternative minimum tax (AMT) and changing how existing AMT credits can be realized; creating a new limitation on deductible interest expense; changing rules related to uses and limitations of net operating loss carryforwards created in tax years beginning after December 31, 2017; limitations on the deductibility of certain executive compensation; and changes to the calculation of the orphan drug credit.

In December 2017, the SEC issued Staff Accounting Bulletin No. 118 ("SAB 118"), which addresses situations where the accounting is incomplete for the income tax effects of the Act. SAB 118 directs taxpayers to consider the impact of the Act as "provisional" when the Company does not have the necessary information available, prepared or analyzed (including computations) to finalize the accounting for the change in tax law. Companies are provided a measurement period of up to one year to obtain, prepare, and analyze information necessary to finalize the accounting for provisional amounts or amounts that cannot be estimated as of December 31, 2017.

With regards to the Tax Act impact on the tax provision as it relates to the company for period year-ending December 31, 2017, we have recognized the provisional impact of tax reform related to the revaluation of deferred tax assets and liabilities from 35% to 21% of $18,039 tax expense, which is offset by a reduction in the valuation allowance.

As a result of changes made by the Tax Act, starting with executive compensation paid in 2018, Section 162(m) will limit us from deducting compensation, including performance-based compensation, in excess of $1,000 paid to certain executives. The only exception to this rule is for compensation that is paid pursuant to a binding contract in effect on November 2, 2017 that would have otherwise been deductible under the prior Section 162(m) rules. The Company has not yet completed an analysis of the binding contract requirement on the various compensation plans to determine the impact of the law change that may affect its deferred tax asset for stock compensation.

With regards to the one-time transition tax, we did not record any tax liability as we estimate that the accumulated earnings and profits of foreign subsidiaries will be in a deficit position. Because of the complexity of the new international tax provisions included in the Act that are not applicable to the Company until 2018, the Company is continuing to evaluate these provisions of the Act and the application of ASC 740.

We will continue to refine our calculations as additional analysis is completed related to the Act. Additional information that may affect our provisional amounts would include further clarification and guidance on how the IRS will implement tax reform, including guidance with respect to the above, further clarification and guidance on how state taxing authorities will implement tax reform and the related effect on our state income tax returns, completion of our 2017 tax return filings, and the potential for additional guidance from the SEC or the FASB related to tax reform. The accounting is expected to be completed when the 2017 U.S. corporate income tax return is filed in 2018.

A reconciliation of income tax benefit computed at the statutory federal income tax rate to income taxes as reflected in the financial statements is as follows:

	December 31,	
	2017	2016
Rate reconciliation:		
Federal income tax benefit at statutory rate	35.0%	35.0%
State and local tax, net of federal benefit	5.3%	8.2%
Loss in earning of subsidiary	(1.0)%	(2.9)%
Permanent differences	(1.3)%	(2.2)%
Tax credits	2.1%	0.0%
Impact of tax reform	(50.6)%	-
Change in valuation allowance	10.5%	(38.1)%
Effective Income tax rate	0.0%	0.0%

Deferred taxes are recognized for temporary differences between the basis of assets and liabilities for financial statement and income tax purposes. The significant components of the Company's deferred tax assets are comprised of the following:

	December 31,	
	2017	2016
Deferred tax assets:		
Net operating loss and credit carryforwards	$ 45,548	$ 47,575
Stock Compensation	7,065	8,759
Accrued Expenses	1,497	1,503
Other	23	29
Total deferred tax assets before valuation allowance	54,133	57,866
Valuation allowance	(54,133)	(57,866)
Net deferred tax asset	$ -	$ -

As of December 31, 2017, the Company had U.S. federal net operating loss carryforwards of $119,251, which may be available to offset future income tax liabilities and will expire beginning in 2020. As of December 31, 2017, the Company also had U.S. state net operating loss carryforwards of $112,260 which may be available to offset future income tax liabilities and will expire beginning in 2018.

The Company has recorded a full valuation allowance against its net deferred tax assets as of December 31, 2017 and 2016, respectively, because the Company has determined that is it more likely than not that these assets will not be fully realized due to historic net operating losses incurred. The Company experienced a net increase of $17,383 and a net decrease of $3,733 in valuation allowance in the years ended December 31, 2016 and 2017, respectively.

As of December 31, 2017, the Company had federal research and development tax credit carryforwards of $1,799 available to reduce future tax liabilities which expire beginning in 2036.

Under the provisions of the Internal Revenue Code, the net operating loss and tax credit carryforwards are subject to review and possible adjustment by the Internal Revenue Service and state tax authorities. Net operating loss and tax credit carryforwards may become subject to an annual limitation in the event of certain cumulative changes in the ownership interest of significant shareholders over a three-year period in excess of 50%, as defined under Sections 382 and 383 of the Internal Revenue Code, respectively, as well as similar state provisions. This could limit the amount of tax attributes that can be utilized annually to offset future taxable income or tax liabilities. The amount of the annual limitation is determined based on the value of the Company immediately prior to the ownership change. Subsequent ownership changes may further affect the limitation in future years. The Company has completed financing since its inception which may have resulted in a change in control as defined by Sections 382 and 383 of the Internal Revenue Code, or could result in a change in control in the future.

The Company files tax returns as prescribed by the tax laws of the jurisdictions in which it operates. In the normal course of business, the Company is subject to examination by federal, state, and foreign jurisdictions, where applicable. The Company's tax years are still open under status from 2014 to present. All open years maybe be examined to the extent that tax credit or net operating loss carryforward are used in future periods. The Company will recognize interest and penalties related to uncertain tax positions in income tax expense. As of December 31, 2017, the Company had no accrued interest or penalties related to uncertain tax positions and no amounts have been recognized in the Company's consolidated statements of operations.

NOTE 9: FINANCIAL INCOME (EXPENSE)

	Year ended December 31,		
	2017	2016	2015
Financial expenses:			
Bank charges	(3)	$ (15)	$ (20)
Warrant valuation	-	-	(1,370)
Foreign currency remeasurement adjustments	(4)	-	(17)
Others	(34)	(9)	(1)
	(41)	$ (24)	$ (1,408)
Financial income:			
Foreign currency remeasurement adjustments	-	$ 5	$ -
Interest on cash equivalents, short-term bank deposits	22	10	1
Others	5	-	-
	27	$ 15	$ 1

NOTE 10: FAIR VALUE MEASUREMENTS

The Company classified certain warrants with down-round protection issued to investors through the years 2006 and 2007 and warrants issued to the purchasers of convertible debentures in 2010 as a liability at their fair value according to ASC 815-40-15-71. The liability in respect of these warrants was remeasured at each reporting period until exercised or expired. Changes in the fair value of these warrants were reported in the Consolidated Statements of Operations as financial income or expense. As of September 30, 2015, this class of warrants had all been exercised or expired, thus there were no need for additional warrant revaluation after that time period.

NOTE 11: LOSS PER SHARE

The Company computes basic net loss per share by dividing net loss by the weighted average number of shares outstanding, which includes stock issued and outstanding. The Company computes diluted net loss per share by dividing net loss by the weighted average number of shares and potential shares from outstanding stock options. Since the Company had a net loss for all periods presented, the effect of all potentially dilutive securities is anti-dilutive. Accordingly, basic and diluted net loss per share is the same for the year ended December, 2017, 2016 and 2015.

The following table presents anti-dilutive shares for the year ended December 31, 2017, 2016, and 2015:

	Year ended December 31,		
	2017	2016	2015
Weighted-average anti-dilutive shares related to:			
Outstanding stock options	11,105,065	10,678,393	8,917,389
Outstanding warrants	4,830,901	5,877,581	9,023,766
	15,935,966	16,555,974	17,941,155

NOTE 12: QUARTERLY FINANCIAL DATA

	Three Months Ended (Unaudited)			
	March 31	June 30	September 30	December 31
2017:				
Gross R&D expenses	$ (7,947)	$ (5,667)	$ (6,299)	$ (5,263)
Net R&D expenses	$ (7,947)	$ (5,667)	$ (6,299)	$ (5,263)
G&A expenses	$ (2,988)	$ (2,369)	$ (2,270)	$ (1,897)
Operating loss	$ (10,935)	$ (8,036)	$ (8,569)	$ (7,160)
Financial income (expense)	$ 18	$ 3	$ (36)	$ 1
Net loss	$ (10,917)	$ (8,033)	$ (8,605)	$ (7,159)
Basic loss per share	$ (0.29)	$ (0.22)	$ (0.23)	$ (0.13)
Diluted loss per share	$ (0.29)	$ (0.22)	$ (0.23)	$ (0.13)
Weighted average number of shares used in computing basic loss per share	37,108,261	37,110,043	37,110,043	55,225,985
Weighted average number of shares used in computing diluted loss per share	37,108,261	37,110,043	37,110,043	55,225,985
2016:				
Gross R&D expenses	$ (6,951)	$ (8,741)	$ (7,725)	$ (5,135)
Net R&D expenses	$ (6,951)	$ (8,741)	$ (7,529)	$ (5,135)
G&A expenses	$ (4,191)	$ (2,945)	$ (3,042)	$ (3,345)
Operating loss	$ (11,142)	$ (11,686)	$ (10,571)	$ (8,480)
Financial (expense) income	$ (2)	$ (18)	$ 14	$ (3)
Net loss	$ (11,144)	$ (11,707)	$ (10,570)	$ (8,483)
Basic loss per share	$ (0.34)	$ (0.35)	$ (0.29)	$ (0.23)
Diluted loss per share	$ (0.34)	$ (0.35)	$ (0.29)	$ (0.23)
Weighted average number of shares used in computing basic loss per share	32,953,542	33,469,789	37,080,789	37,100,778
Weighted average number of shares used in computing diluted loss per share	32,953,542	33,469,789	37,080,789	37,100,778

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

None.

ITEM 9A - Controls and Procedures.

Evaluation of Disclosure Controls and Procedures

As required by Exchange Act Rule 13a-15(b), in connection with the filing of this Annual Report on Form 10-K, we carried out an evaluation, under the supervision and with the participation of our management, including our principal executive officer and principal financial officer, of the effectiveness of the design and operation of our disclosure controls and procedures (as defined in Exchange Act Rules 13a-15(e) and 15d-15(e)) as of the end of the period covered by this Annual Report on Form 10-K. Based on this evaluation, our principal executive officer and principal financial officer concluded that our disclosure controls and procedures were effective as of December 31, 2017, the end of the period covered by this report.

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). Under the supervision and with the participation of our management, including our principal executive officer and principal financial officer, we conducted an evaluation of the effectiveness of our internal control over financial reporting based on the framework set forth in *Internal Control – Integrated Framework* issued by the Committee of Sponsoring Organizations of the Treadway Commission (2013 framework). Based on our evaluation under the framework set forth in *Internal Control –Integrated Framework*, our management concluded that our internal control over financial reporting was effective as of December 31, 2017.

Changes in Internal Control Over Financial Reporting

There were no changes in our internal control over financial reporting during the year ended December 31, 2017 that have materially affected, or are reasonably likely to materially affect, our internal control over financial reporting.

ITEM 9B - Other Information.

None.

PART III

ITEM 10 - Directors, Executive Officers and Corporate Governance.

Information required by Item 10 is incorporated by reference to our definitive proxy statement for our annual stockholders' meeting presently scheduled to be held in June 2018.

ITEM 11 - Executive Compensation.

Information required by Item 11 is incorporated by reference to our definitive proxy statement for our annual stockholders' meeting presently scheduled to be held in June 2018.

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

Information required by Item 12 is incorporated by reference to our definitive proxy statement for our annual stockholders' meeting presently scheduled to be held in June 2018.

ITEM 13 - Certain Relationships and Related Transactions, and Director Independence.

Information required by Item 13 is incorporated by reference to our definitive proxy statement for our annual stockholders' meeting presently scheduled to be held in June 2018.

ITEM 14 - Principal Accountant Fees and Services.

Information required by Item 14 is incorporated by reference to our definitive proxy statement for our annual stockholders' meeting presently scheduled to be held in June 2018.

PART IV

ITEM 15 - Exhibits and Financial Statement Schedules.

(a)(1) Financial Statements.

(a)(2) *Financial Statement Schedules*. No financial statement schedules are provided because the information called for is not required or is shown either in the financial statements or the notes thereto.

(a)(3) *Exhibits*. The list of exhibits filed with or incorporated by reference in this Annual Report on Form 10-K is set forth below.

Exhibit No.	Description
3.1	Amended and Restated Certificate of Incorporation (previously filed as Exhibit 3.1 to the Company's Registration Statement on Form S-1 filed November 5, 2010 and incorporated herein by reference).
3.2	Certificate of Amendment to Amended and Restated Certificate of Incorporation (previously filed as Exhibit 3.2 to the Company's Registration Statement on Form S-1 filed November 5, 2010 and incorporated herein by reference).
3.3	Certificate of Amendment to Amended and Restated Certificate of Incorporation dated as of February 14, 2011 (previously filed as Exhibit 4.3 to the Company's Post-Effective Amendment No. 1 to Form S-1 on Form S-3 filed July 16, 2012 and incorporated herein by reference).
3.4	Third Amended and Restated By-Laws (previously filed as Exhibit 3.2 to the Company's Current Report on Form 8-K filed December 15, 2016 and incorporated herein by reference).
3.5	Certificate of Amendment to the Amended and Restated Certificate of Incorporation (previously filed as Exhibit 3.1 to the Company's Current Report on Form 8-K filed October 18, 2017 and incorporated herein by reference).
4.1	Specimen Common Stock Certificate (previously filed as Exhibit 4.1 to the Company's Amendment No. 4 to Registration Statement on Form S-1 filed February 22, 2011 and incorporated herein by reference).
4.2	Registration Rights Agreement, dated as of May 25, 2009, between the Company and the person named therein (previously filed as Exhibit 4.2 to the Company's Registration Statement on Form S-1 filed November 5, 2010 and incorporated herein by reference).
4.3	Registration Rights Agreement, dated as of September 15, 2010, between the Company and the persons named therein (previously filed as Exhibit 4.3 to the Company's Registration Statement on Form S-1 filed November 5, 2010 and incorporated herein by reference).
4.4	Form of Warrant Certificate, dated as of June 18, 2012 (previously filed as Exhibit 10.2 to the Company's Current Report on Form 8-K filed June 19, 2012 and incorporated herein by reference).

Exhibit No.	Description
4.5	Warrant Agreement, dated as of June 18, 2012, between Medgenics, Inc. and Corporate Stock Transfer, Inc., as warrant agent (previously filed as Exhibit 10.3 to the Company's Current Report on Form 8-K filed June 19, 2012 and incorporated herein by reference).
4.6	Common Stock Purchase Warrant, dated as of June 18, 2012, issued to Maxim Partners LLC (previously filed as Exhibit 10.4 to the Company's Current Report on Form 8-K filed June 19, 2012 and incorporated herein by reference).
4.7	Registration Rights Agreement, dated as of June 18, 2012, by and among Medgenics, Inc. and the investors party thereto (previously filed as Exhibit 10.5 to the Company's Current Report on Form 8-K filed June 19, 2012 and incorporated herein by reference).
4.8	Warrant Agreement, dated as of February 8, 2013, between Medgenics, Inc. and Corporate Stock Transfer, Inc. (previously filed as Exhibit 4.1 to the Company's Current Report on Form 8-K filed February 8, 2013 and incorporated herein by reference).
4.9	Form of Series 2013-A Warrant Certificate (previously filed as Exhibit 4.2 to the Company's Current Report on Form 8-K filed February 8, 2013 and incorporated herein by reference).
4.10	Form of Warrant (previously filed as Exhibit 4.1 to the Company's Current Report on Form 8-K filed August 11, 2017 and incorporated herein by reference).
10.1†	Medgenics, Inc. Stock Incentive Plan, as amended and restated effective March 5, 2012 (previously filed as Exhibit 10.1 to the Company's Current Report on Form 8-K filed April 5, 2012 and incorporated herein by reference).
10.2†	First Amendment of the Medgenics, Inc. Stock Incentive Plan (previously filed as Exhibit 10.1 to the Company's Current Report on Form 8-K filed May 1, 2013 and incorporated herein by reference).
10.3†	Second Amendment of the Medgenics, Inc. Stock Incentive Plan (previously filed as Exhibit 10.1 to the Company's Current Report on Form 8-K filed April 9, 2014 and incorporated herein by reference).
10.4†	Third Amendment to Medgenics, Inc. Stock Incentive Plan (as amended and restated March 5, 2012), dated April 12, 2016 (previously filed as Exhibit 10.1 to the Company's Current Report on Form 8-K filed April 13, 2016 and incorporated herein by reference).
10.5†	Form of Non-Qualified Stock Option Award Agreement under the Medgenics, Inc. Stock Incentive Plan (previously filed as Exhibit 10.3 to the Company's Quarterly Report on Form 10-Q for the quarter ended March 31, 2013 and incorporated herein by reference).
10.6†	Form of Restricted Stock Award Agreement under the Medgenics, Inc. Stock Incentive Plan (previously filed as Exhibit 10.4 to the Company's Quarterly Report on Form 10-Q for the quarter ended March 31, 2013 and incorporated herein by reference).
10.7†	Form of Non-Qualified Stock Option Award Terms (Outside of Plan) (previously filed as Exhibit 4.7 to the Company's Registration Statement on Form S-8 filed October 15, 2013 and incorporated herein by reference).
10.8†	Employment Agreement, dated as of September 13, 2013, between Medgenics, Inc. and Michael Cola (previously filed as Exhibit 10.1 to the Company's Current Report on Form 8-K filed September 16, 2013 and incorporated herein by reference).

Exhibit No.	Description
10.9†	Executive Director Appointment Letter, dated as of September 13, 2013, between Medgenics, Inc. and Michael Cola (previously filed as Exhibit 10.2 to the Company's Current Report on Form 8-K filed September 16, 2013 and incorporated herein by reference).
10.10†	Employment Agreement, dated as of September 13, 2013, between Medgenics, Inc. and John Leaman (previously filed as Exhibit 10.3 to the Company's Current Report on Form 8-K filed September 16, 2013 and incorporated herein by reference).
10.11†	Employment Agreement, dated as of September 13, 2013, between Medgenics, Inc. and Garry Neil (previously filed as Exhibit 10.4 to the Company's Current Report on Form 8-K filed September 16, 2013 and incorporated herein by reference).
10.12†	Employment Agreement, dated as of September 8, 2014, between Medgenics, Inc. and Scott Applebaum (previously filed as Exhibit 10.1 to the Company's Quarterly Report on Form 10-Q for the quarter ended September 30, 2014 and incorporated herein by reference).
10.13†	Non-Executive Director Appointment Letter, dated as of November 14, 2007, for Eugene Andrew Bauer (previously filed as Exhibit 10.12 to the Company's Registration Statement on Form S-1 filed November 5, 2010 and incorporated herein by reference).
10.14†	Non-Executive Director Appointment Letter, dated as of June 6, 2011, for Isaac Blech (previously filed as Exhibit 10.1 to the Company's Current Report on Form 8-K filed July 5, 2011 and incorporated herein by reference).
10.15†	Medgenics, Inc. Non-Qualified Stock Option Award Terms between Medgenics, Inc. and Sol J. Barer (previously filed as Exhibit 4.7 to the Company's Registration Statement on Form S-8 filed August 1, 2012 and incorporated herein by reference).
10.16†	Director Appointment Letter, dated as of August 6, 2012, between Medgenics, Inc. and Sol J. Barer (previously filed as Exhibit 10.1 to the Company's Current Report on Form 8-K/A filed August 8, 2012 and incorporated herein by reference).
10.17†	Non-Executive Director Appointment Letter, dated as of March 8, 2013, between Medgenics, Inc. and Joseph J. Grano, Jr. (previously filed as Exhibit 10.1 to the Company's Current Report on Form 8-K filed March 14, 2013 and incorporated herein by reference).
10.18†	Non-Executive Director Appointment Letter, dated as of October 16, 2013, between Medgenics, Inc. and Wilbur H. Gantz (previously filed as Exhibit 10.1 to the Company's Current Report on Form 8-K filed October 18, 2013 and incorporated herein by reference).
10.19†	Non-Executive Director Appointment Letter, dated as of May 21, 2015, between Medgenics, Inc. and Barbara G. Duncan (previously filed as Exhibit 10.1 to the Company's Current Report on Form 8-K filed June 10, 2015 and incorporated herein by reference).
10.20*	Sponsored Research Agreement, dated as of November 12, 2014, between Medgenics Medical Israel Ltd. and The Children's Hospital of Philadelphia (previously filed as Exhibit 10.28 to the Company's Annual Report on Form 10-K for the year ended December 31, 2014 and incorporated herein by reference).
10.21	Amendment #1 to Sponsored Research Agreement, dated December 18, 2015, by and between Medgenics Medical Israel Ltd. and the Children's Hospital of Philadelphia (previously filed as Exhibit 10.1 to the Company's Current Report on Form 8-K filed December 22, 2015 and incorporated herein by reference).
10.22*	License Agreement, dated as of November 12, 2014, between Medgenics Medical Israel Ltd. and The Children's Hospital of Philadelphia (previously filed as Exhibit 10.29 to the Company's Annual Report on Form 10-K for the year ended December 31, 2014 and incorporated herein by reference).

Exhibit No.	Description
10.23*	Equity Interest Purchase Agreement, dated as of September 9, 2015, among Medgenics, Inc., neuroFix therapeutics, inc., neuroFix, LLC, The Children's Hospital Of Philadelphia, Philip Harper and Hakon Hakonarson (previously filed as Exhibit 10.1 to the Company's Quarterly Report on Form 10-Q for the quarter ended September 30, 2015 and incorporated herein by reference).
10.24*	License Agreement, dated as of September 9, 2015, between neuroFix, LLC and The Children's Hospital Of Philadelphia (previously filed as Exhibit 10.2 to the Company's Quarterly Report on Form 10-Q for the quarter ended September 30, 2015 and incorporated herein by reference).
10.25	Purchase Agreement dated October 1, 2015 by and among Medgenics, Inc. and Piper Jaffray & Co., as representative of the several underwriters set forth on Schedule I thereto (previously filed as Exhibit 1.1 to the Company's Current Report on Form 8-K filed October 7, 2015 and incorporated herein by reference).
10.26†	Letter Agreement, by and between Medgenics, Inc. and Brian Piper, dated February 1, 2016 (previously filed as Exhibit 10.1 to the Company's Current Report on Form 8-K filed February 3, 2016 and incorporated herein by reference).
10.27†	Agreement and Release and Waiver, by and between Medgenics, Inc. and John Leaman, dated February 1, 2016 (previously filed as Exhibit 10.2 to the Company's Current Report on Form 8-K filed February 3, 2016 and incorporated herein by reference).
10.28	Purchase Agreement, by and among Medgenics, Inc. and Jefferies LLC, as representative of the several underwriters set forth on Schedule I thereto, dated June 21, 2016 (previously filed as Exhibit 1.1 to the Company's Current Report on Form 8-K filed June 24, 2016 and incorporated herein by reference).
10.29*	Clinical Development and Option Agreement, by and between Medgenics, Inc. and Kyowa Hakko Kirin Co., Ltd., dated June 6, 2016 (previously filed as Exhibit 10.1 to the Company's Quarterly Report on Form 10-Q for the quarter ended June 30, 2016 and incorporated herein by reference).
10.30*	Amendment No. 1 to License Agreement, dated as of February 14, 2017, by and between The Children's Hospital of Philadelphia and Medgenics Medical Israel Ltd (previously filed as Exhibit 10.1 to the Company's Quarterly Report on Form 10-Q for the quarter ended March 31, 2017 and incorporated herein by reference).
10.31	Amendment No. 2 to Sponsored Research Agreement, dated as of February 16, 2017, by and between The Children's Hospital of Philadelphia and Medgenics Medical Israel, Ltd (previously filed as Exhibit 10.2 to the Company's Quarterly Report on Form 10-Q for the quarter ended March 31, 2017 and incorporated herein by reference).
10.32	Securities Purchase Agreement (previously filed as Appendix A to the Company's Definitive Proxy Statement on Schedule 14A filed on September 8, 2017 and incorporated herein by reference).
10.33	Registration Rights Agreement (previously filed as Appendix B to the Company's Definitive Proxy Statement on Schedule 14A filed on September 8, 2017 and incorporated herein by reference).
10.34	Form of Indemnification Agreement (previously filed as Exhibit 10.1 to the Company's Current Report on Form 8-K filed on December 4, 2017 and incorporated herein by reference).
16.1	Letter from Kost Forer Gabbay & Kasierer to the U.S. Securities and Exchange Commission, regarding change in certifying accountant of the Company, dated August 4, 2016 (previously filed as Exhibit 16.1 to the Company's Current Report on Form 8-K filed August 4, 2016 and incorporated herein by reference).

Exhibit No.	Description
21.1	Subsidiaries of the Company (filed herewith).
23.1	Consent of Ernst & Young LLP US (filed herewith).
23.2	Consent of KOST FORER GABBAY & KASIERER (filed herewith).
31.1	Certification of Principal Executive Officer pursuant to Section 302 of the Sarbanes-Oxley Act of 2002 (filed herewith).
31.2	Certification of Principal Financial Officer pursuant to Section 302 of the Sarbanes-Oxley Act of 2002 (filed herewith).
32.1	Certification of Principal Executive Officer and Principal Financial Officer pursuant to 18 U.S.C. Section 1350, as adopted pursuant to Section 906 of the Sarbanes-Oxley Act of 2002 (furnished herewith).
101	Interactive Data File (filed herewith).

† Indicates a management contract or compensatory plan or arrangement contemplated by Item 15(a)(3) of Form 10-K.

* Portions of this exhibit have been omitted pursuant to a request for confidential treatment on file with the Securities and Exchange Commission.

ITEM 16 – Form 10-K Summary.

We have opted to not provide a summary.

SIGNATURES

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

AEVI GENOMIC MEDICINE, INC.

Date: March 13, 2018

By: /s/ Michael F. Cola
Michael F. Cola
President and Chief Executive Officer

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

Name	Title	Date
/s/ Michael F. Cola Michael F. Cola	President, Chief Executive Officer and Director (Principal Executive Officer)	March 13, 2018
/s/ Brian D. Piper Brian D. Piper	Chief Financial Officer (Principal Financial Officer & Principal Accounting Officer)	March 13, 2018
/s/ Sol J. Barer Sol J. Barer	Chairman of the Board of Directors	March 13, 2018
/s/ Eugene A. Bauer Eugene A. Bauer	Director	March 13, 2018
/s/ Alastair Clemow Alastair Clemow	Director	March 13, 2018
/s/ Barbara Duncan Barbara Duncan	Director	March 13, 2018
/s/ Wilbur H. Gantz Wilbur H. Gantz	Director	March 13, 2018
/s/ Joseph J. Grano, Jr. Joseph J. Grano, Jr.	Director	March 13, 2018
/s/ Matthew D. Bayley Matthew D. Bayley	Director	March 13, 2018

Exhibit 21.1

SUBSIDIARIES OF AEVI GENOMIC MEDICINE, INC.

Medgenics Medical (Israel) Ltd., a company organized under the laws of the State of Israel neuroFix, LLC, a Delaware limited liability company.

Exhibit 23.1

CONSENT OF INDEPENDENT REGISTERED PUBLIC ACCOUNTING FIRM

We consent to the incorporation by reference in the Registration Statements on Form S-3 (File Nos. 333-221950, 333-209737, 333-182740 and 333-208586) and in the Registration Statements on Form S-8 (File Nos. 333-219788, 333-210737, 333-182992, 333-188709, 333-191733 and 333-195165) of Aevi Genomic Medicine, Inc. of our report dated March 13, 2018, with respect to the consolidated financial statements of Aevi Genomic Medicine, Inc. included in this Annual Report of Aevi Genomic Medicine, Inc. (Form 10-K) for the year ended December 31, 2017.

/s/ Ernst & Young LLP
Philadelphia, Pennsylvania
March 13, 2018

Exhibit 23.2

CONSENT OF INDEPENDENT REGISTERED PUBLIC ACCOUNTING FIRM

We consent to the incorporation by reference in the Registration Statements on Form S-3 (File Nos. 333-221950, 333-209737, 333-182740 and 333-208586) and in the Registration Statements on Form S-8 (File Nos. 333-219788, 333-210737, 333-182992, 333-188709, 333-191733 and 333-195165) of Aevi Genomic Medicine, Inc. (formerly Medgenics Inc.) of our report dated Febuary 26, 2016, with respect to the consolidated financial statements of Aevi Genomic Medicine, Inc. included in this Annual Report of Aevi Genomic Medicine, Inc. (Form 10-K) for the year ended December 31, 2017.

Haifa, Israel
March 13, 2018

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

Exhibit 31.1

CERTIFICATION OF PRINCIPAL EXECUTIVE OFFICER
PURSUANT TO SECTION 302 OF THE SARBANES-OXLEY ACT OF 2002

I, Michael F. Cola, certify that:

PART I I have reviewed this Annual Report on Form 10-K of Aevi Genomic Medicine, Inc.;

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

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

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

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

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

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

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

PART V The registrant's other certifying officer and I have disclosed, based on our most recent evaluation of internal control over financial reporting, to the registrant's auditors and the audit committee of the registrant's board of directors (or persons performing the equivalent functions):

A. All significant deficiencies and material weaknesses in the design or operation of internal control over financial reporting which are reasonably likely to adversely affect the registrant's ability to record, process, summarize and report financial information; and

B. Any fraud, whether or not material, that involves management or other employees who have a significant role in the registrant's internal control over financial reporting.

Date: March 13, 2018

AEVI GENOMIC MEDICINE, INC.
/s/ Michael F. Cola
Michael F. Cola
President and Chief Executive Officer
(Principal Executive Officer)

Exhibit 31.2

CERTIFICATION OF PRINCIPAL EXECUTIVE OFFICER
PURSUANT TO SECTION 302 OF THE SARBANES-OXLEY ACT OF 2002

I, Brian D. Piper, certify that:

PART I I have reviewed this Annual Report on Form 10-K of Aevi Genomic Medicine, Inc.;

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

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

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

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

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

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

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

PART V The registrant's other certifying officer and I have disclosed, based on our most recent evaluation of internal control over financial reporting, to the registrant's auditors and the audit committee of the registrant's board of directors (or persons performing the equivalent functions):

A. All significant deficiencies and material weaknesses in the design or operation of internal control over financial reporting which are reasonably likely to adversely affect the registrant's ability to record, process, summarize and report financial information; and

B. Any fraud, whether or not material, that involves management or other employees who have a significant role in the registrant's internal control over financial reporting.

Date: March 13, 2018

AEVI GENOMIC MEDICINE, INC.
/s/ Brian D. Piper
Brian D. Piper
Chief Financial Officer and Corporate Secretary
(Principal Financial Officer)

Exhibit 32.1

CERTIFICATION PURSUANT TO 18 U.S.C. SECTION 1350, AS ADOPTED PURSUANT TO SECTION 906 OF THE SARBANES-OXLEY ACT OF 2002

Pursuant to Section 906 of the Sarbanes-Oxley Act of 2002 (Chapter 63, Title 18 U.S.C. § 1350(a) and (b)), each of the undersigned hereby certifies that, to his knowledge, the Annual Report on Form 10-K for the fiscal year ended December 31, 2017 of Aevi Genomic Medicine, Inc. (the "Company") fully complies with the requirements of Section 13(a) or Section 15(d) of the Securities Exchange Act of 1934, as amended, and that the information contained in such report fairly presents, in all material respects, the financial condition and results of operations of the Company.

Date: March 13, 2018

/s/ Michael F. Cola
Michael F. Cola
President and Chief Executive Officer
(Principal Executive Officer)

Date: March 13, 2018

/s/ Brian D. Piper
Brian D. Piper and Corporate Secretary
Chief Financial Officer and Corporate Secretary
(Principal Financial Officer)