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, 2018

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

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

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

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, 2018, as of the last business day of the registrant's most recently completed second fiscal quarter was approximately $45.6 million.

As of March 26, 2019, the registrant had 64,766,882 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 2019 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, 2018.

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 and execute on our business strategy;
- the need to obtain and maintain 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;
- legal and regulatory developments in the United States and foreign countries, including any actions or advice that may affect the design, initiation, timing, continuation, progress, or outcome of clinical trials or result in the need for additional preclinical studies or clinical trials;
- our ability to commercialize our product candidates and the indication and labeling under any such approval;
- market acceptance of our product candidates, including the size and growth of the potential markets for our product candidates, and our ability to serve those markets;
- 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;
- recently enacted and future legislation regarding the healthcare system, including changes to the Patient Protection and Affordable Care Act, or Affordable Care Act;
- our dependency on third-party manufacturers to supply or manufacture our products;
- delays, interruptions or failures in the manufacture and supply of our product candidates;
- 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;
- our ability to attract and retain key personnel to manage our business effectively; and
- our ability to identify, evaluate and execute on strategic alternatives.

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., neuroFix, LLC and Aevi Genomic Medicine Europe BVBA/SPRL. 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's efforts focus on the discovery of 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 potential 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 try to 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 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	Status
AEVI-002* (anti-LIGHT mAb)	Severe Pediatric Onset Crohn's Disease				Initial Data Mid-year 2019
AEVI-004	Epilepsy				*in vitro* POC work
AEVI-005*	Undisclosed pediatric rare disease				*in vitro* POC work

* Partnered with Kyowa Hakko Kirin (KHK)

AEVI-001 (mGluR+ Genetic Subset ADHD)

The initial program from our genomic research collaboration with CHOP was 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.

Development of AEVI-001 in mGluR+ Genetic Subset ADHD

We completed an AEVI-001 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.

Our subsequent analysis of responder data from a subset of genomically identified patients in the SAGA trial identified eight genes (genetic subset) that appeared to be predictive of a 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.

We believed that these results clarified a path forward for the continued development of AEVI-001 in ADHD. As a result, we initiated a Phase 2 trial (the ASCEND trial) during the third quarter of 2017 to confirm genetic responders to AEVI-001. On January 2, 2019, we announced that the ASCEND trial, a genomically-guided Phase 2 double-blind, placebo-controlled clinical trial of orally-administered AEVI-001 (100 – 400 mg BID) in children aged 6 – 17 with Attention Deficit Hyperactivity Disorder (ADHD) with an mGluR copy number variant (Part A) or without an mGluR copy number variant (Part B), did not achieve statistical significance on the primary endpoint of reduction of ADHD-RS in either Part A or Part B after 6 weeks of treatment with AEVI-001. AEVI-001 was safe and well tolerated. Reported adverse events were minimal and similar across both Part A and Part B treatment groups. Given the negative outcomes of the ASCEND trial, we have terminated the AEVI-001 program.

AEVI-004 (novel co-crystal version of AEVI-001)

In July 2018, we announced the receipt of positive feedback from the United States Food and Drug Administration, which we refer to as the FDA, on an improved version of AEVI-001, identified as AEVI-004.

Following 2016 FDA regulatory guidance on co-crystalization of active drugs, we created a co-crystal of fasoracetam (AEVI-001) with enhanced physical and chemical properties. The new molecule, AEVI-004, has comparatively greater stability and a higher melting point than AEVI-001. The molecule was engineered to maintain solubility, dissolution and pharmacokinetics substantially similar to AEVI-001.

In July 2018, we announced the receipt of feedback from the FDA provisionally indicating that AEVI-004 is a co-crystal of AEVI-001 and eligible for new chemical entity (NCE) status. The FDA also provisionally indicated that existing toxicology and pathology studies would support clinical development with AEVI-004 with minimal preclinical bridging studies.

AEVI-004 is expected to have composition of matter patents extending to 2039 and should be listed as a NCE in the FDA Orange Book.

Given the negative outcomes of the ASCEND trial, there are no current clinical development plans for AEVI-004 in ADHD. However, the company is in discussions with the National Institutes of Health (NIH) regarding evaluating the potential anti-seizure activity of AEVI-004 in a preclinical model as part of the NIH Epilepsy Treatment Screening Program.

AEVI-002 (Anti-LIGHT Monoclonal Antibody)

AEVI-002, a first-in-class anti-LIGHT monoclonal antibody, or the Antibody, is in development 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."

A submission to reactivate the IND for AEVI-002 in Pediatric Crohn's Disease was filed with the FDA in 2017 and has passed the 30-day waiting period. An 8-week Phase Ib proof-of-concept study has been initiated, 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 mid-year 2019, at the earliest, 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 mid-year 2019 is highly dependent on timely recruiting; thus, continued difficulties in recruitment could cause an extended delay or an inability to deliver initial data for the program.

AEVI-005 (Monoclonal Antibody)

AEVI-005 is the second monoclonal antibody we are developing as part of our ongoing collaboration with KHK. We are studying AEVI-005 in an undisclosed ultra-orphan auto-immune pediatric disease. We initiated a preclinical research program with AEVI-005 in the second quarter of 2018.

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 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 with the intent of enrolling up to 12 patients with 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 mid-year 2019, at the earliest, 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.

In light of our decision to discontinue the AEVI-001 program in ADHD, our board of directors has commenced a review to explore and evaluate potential strategic alternatives to enhance stockholder value. These alternatives could include, among others, continuing to execute the Company's business plan, issuing or transferring shares of our common stock or other equity securities, the license, sale or disposition of certain assets or programs, the formation of a joint venture, a strategic business combination, a transaction that results in private ownership or the sale of the Company, or some combination of these. There can be no assurance that the review of strategic alternatives will result in the identification or consummation of any transaction or that our board of directors will determine that continuing our current business operations is in the best interests of our stockholders.

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, AEVI-002 and AEVI-005 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

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 and (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 the first extension was $197,603 with each subsequent extension costing $125,000. We have exercised such option in each of 2017 and 2018. The amendment also (1) granted us the first right to defend challenges to the patent rights licensed under the License Agreement, (2) clarified termination rights in favor of CHOP if we enter liquidation, have a receiver or administrator appointed over any assets related to the License Agreement, make any voluntary assignment of our assets for the benefit of creditors, cease to carry on business, file for bankruptcy under Chapter 7 of the US Bankruptcy Code or have an involuntary petition under Chapter 7 of the US Bankruptcy Code filed against us and (3) added a provision that tolls the right to terminate the License Agreement for default until completion of dispute resolutions proceedings concerning the alleged default and any remaining cure period, if any.

In December 2015, we entered into an amendment to the Research Agreement, which amendment (i) set the payment schedule under such agreement through March 2017 and (ii) granted us the right to extend the term of the Research Agreement until November 12, 2017**.** In February 2017, we entered into a second amendment to the Research Agreement, which extended the term of the Research Agreement through June 30, 2018. This amendment also granted us rights to continually extend the term of the Research Agreement by one year by giving CHOP written notice of extension no later than one year prior to the expiration of the then-current term of the Research Agreement. In June 2017, we extended the term of the Research Agreement through June 30, 2019, and in June 2018, we extended the term of Research Agreement through June 30, 2020.

For the year ended December 31, 2018, $5.94 million was due under the Research Agreement and $4.75 million will be due under the Research Agreement in 2019. In the first half of 2020, $2.38 million will be due under Research Agreement.

On March 25, 2019, we and CHOP agreed to, and on March 29, 2019 we and CHOP entered into definitive agreements to, further amend the Research Agreement and the License Agreement, or the CHOP Amendments. The CHOP Amendments allow us to defer the monthly payments due under the Research Agreement for the period from February 1, 2019 through September 30, 2019 in exchange for a non-interest bearing note in the amount of such deferral. Such note matures September 30, 2019 and is secured by all of Aevi's intellectual property and other assets, or the Note. At maturity, and at CHOP's option, the Note will be payable in cash or a number of shares of our common stock calculated based on the price of our common stock at such time; provided, however, if conversion upon such election would cause CHOP and its affiliates including the CHOP Foundation to own, in the aggregate, in excess of 47.5% of the then-outstanding shares of our common stock (after giving effect to such conversion), then CHOP would only receive the number of shares of our common stock such that CHOP and its affiliates including the CHOP Foundation would own, in the aggregate, 47.5% of the then outstanding shares of our common stock (after giving effect to such conversion), and the balance of the Note would be payable to CHOP in cash.

The CHOP Amendments with respect to the Research Agreement and the License Agreement prohibits the assignment or sublicense of CHOP's intellectual property without CHOP's prior written consent, allows CHOP to terminate the Research Agreement and the License Agreement upon a change of control without CHOP's prior written consent, reduces the period of time during which we have to exercise its options to license new intellectual property of CHOP and to negotiate the terms of any such license and requires us to meet certain diligence requirements related to acquiring rights to and commencing a clinical trial for a viable molecule that addresses the optioned intellectual property.

Furthermore, we have agreed that until and including June 23, 2019 the Company will not undertake any equity financing (including convertible notes) that would have a dilutive effect on the stockholders of Aevi. Thereafter, and until the later of repayment in full of the Note or June 30, 2020, Aevi has agreed to only undertake an equity financing (including convertible notes) if the net proceeds of such financing provide at least six month of cash to sustain our operations; provided, that CHOP will have a right of first refusal to purchase any or all equity proposed to be issued in such financing on equivalent terms.

Development and Option Agreement with Kyowa Hakko Kirin Co., Ltd. (KHK) related to AEVI-002

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.

Regarding AEVI-002, 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 (AEVI-002): 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 (AEVI-002): 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.

Research Collaboration and Option Agreement with Kyowa Hakko Kirin Co., Ltd. (KHK) related to AEVI-005

During 2018, we expanded our collaboration with KHK by entering a Research Collaboration and Option Agreement related to AEVI-005. AEVI-005 is the second monoclonal antibody we are developing as part of our ongoing collaboration with KHK. We are studying AEVI-005 in an undisclosed ultra-orphan auto-immune pediatric disease. We initiated a preclinical research program with AEVI-005 in the second quarter of 2018.

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 research, clinical and preclinical testing, manufacture, quality control, safety, effectiveness, approval, labeling, distribution, sale, import, export, storage, record-keeping, reporting, advertising, and promotion of our products. Although the discussion below focuses on regulation in the United States, we anticipate seeking approval for, and marketing of, our products in other countries. Generally, our activities in other countries will be subject to regulation that is similar in nature and scope as that imposed in the United States, although there can be important differences. Product development and approval within this regulatory framework, if successful, will take many years and involve the expenditure of substantial time and financial 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 in compliance with good laboratory practices, or GLP, and other regulations;
- submission to the FDA of an Investigational New Drug Application, or IND, for a new drug or biologic, which must become effective before human clinical trials may begin;
- approval by an Institutional Review Board, or IRB, before each trial may be initiated;
- performance of adequate and well-controlled human clinical trials in accordance with federal regulations and with current Good Clinical Practices, or GCPs, to establish the safety and efficacy of the proposed drug or biologic for its intended use;
- detailed information on product characterization and manufacturing process;
- satisfactory completion of an FDA inspection of the manufacturing facilities at which the investigational product candidate is produced to assess compliance with cGMP, and to assure that the facilities, methods and controls are adequate;
- submission of a New Drug Application, or NDA, for a drug or a Biologics License Application, or BLA, for a biologic;
- satisfactory completion of an FDA Advisory Committee review, if applicable; and
- review and approval of an NDA or a BLA.

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.

Clinical trials involve the administration of the investigational product candidate to healthy volunteers or patients under the supervision of qualified investigators. Clinical trials are conducted under protocols that detail, among other things, the parameters to be used in monitoring safety and the efficacy criteria to be evaluated. Each protocol involving testing on U.S. patients and subsequent protocol amendments must be submitted to the FDA as part of the IND. Clinical trials must be reviewed, approved and conducted under the auspices of an IRB. The sponsor, investigators, and IRB must, as applicable, obtain the informed written consent of each participating subject, comply with the protocol and investigational plan, adequately monitor the clinical trial, and timely report adverse events.

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 with the target disease or condition, 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.

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. In addition, there are requirements for the registration of certain ongoing clinical trials of product candidates on public registries and the disclosure of certain information pertaining to the trials as well as clinical trial results after completion. In the United States, sponsors are required to register this information on a website maintained by the U.S. National Institutes of Health, or NIH, at www.clinicaltrials.gov.

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 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 elements to assure safe use, or ETASU, such as 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.

From time to time, legislation is drafted, introduced and passed in the U.S. Congress that could significantly change the statutory provisions governing the approval, manufacturing and marketing of products regulated by the FDA. In addition to new legislation, the FDA regulations and policies are often revised or reinterpreted by the agency in ways that may significantly affect our business and our product candidates. It is impossible to predict whether further legislative or FDA regulation or policy changes will be enacted or implemented and what the impact of such changes, if any, may be. For example, in December 2016, the 21st Century Cures Act, or the Cures Act, became law. The Cures Act contains numerous provisions, including provisions designed to speed development of innovative therapies and encourage greater use of real-world evidence to support regulatory decision making for drugs.

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.

In August 2017, President Trump signed into law the Food & Drug Administration Reauthorization Act. 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.

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. A product cannot be commercially promoted before it is approved. After approval, 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. In addition to FDA restrictions on marketing of pharmaceutical products, state and federal fraud and abuse and consumer protection laws have been applied to restrict certain marketing practices in the pharmaceutical industry in recent years. Some of the pertinent laws have not been definitively interpreted by the regulatory authorities or the courts, and their provisions are open to a variety of interpretations. In addition, these laws and their interpretations are subject to change.

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 effect 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 Affordable Care Act. 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 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-002, may 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, which substantially changes the way healthcare will be financed by both governmental and private insurers, and significantly impacts the pharmaceutical industry. The Affordable Care Act 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 Affordable Care Act 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. Other legislative changes have been proposed and adopted since passage of the Affordable Care Act, and there have been significant ongoing efforts to modify or eliminate the under the current administration. Further legislation to repeal or revise Affordable Care Act, if enacted, may have a significant impact on the health care system.

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 Affordable Care Act, 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 Affordable Care Act 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. The False Statements Statute prohibits knowingly and willfully falsifying, concealing or covering up a material fact or making any materially false statement in connection with the delivery of or payment for healthcare benefits, items or services.

HIPAA, as amended by HITECH, 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.

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. Some state laws also require pharmaceutical companies to report expenses relating to the marketing and promotion of pharmaceutical products and to report gifts and payments to certain health care providers in those states. Some of these states also prohibit certain marketing-related activities including the provision of gifts, meals, or other items to certain health care providers

The federal Physician Payment Sunshine Act, being implemented as the Open Payments Program, requires certain pharmaceutical and biological manufacturers to engage in extensive tracking of payments or transfers of value to physicians and teaching hospitals and public reporting of the payment data. Pharmaceutical and biological manufacturers with products for which payment is available under Medicare, Medicaid or the State Children’s Health Insurance Program are required to track such payments, and must submit a report on or before the 90th day of each calendar year disclosing reportable payments made in the previous calendar year.

Moreover, we are subject to data protection laws and regulations (i.e., laws and regulations that address privacy and data security). In the U.S., numerous federal and state laws and regulations, including state data breach notification laws, state health information privacy laws, and federal and state consumer protection laws govern the collection, use, disclosure and protection of health-related and other personal information. Failure to comply with data protection laws and regulations could result in government enforcement actions and create liability for us (which could include civil and/or criminal penalties), private litigation and/or adverse publicity that could negatively affect our operating results and business. HIPAA, as amended by HITECH, and its implementing regulations imposes obligations, including mandatory contractual terms, with respect to safeguarding the privacy, security and transmission of individually identifiable health information. Although we are not directly subject to HIPAA other than with respect to providing certain employee benefits, we potentially could be subject to criminal penalties if we knowingly obtain or disclose individually identifiable health information maintained by a HIPAA-covered entity (e.g., a healthcare provider or a health plan) in a manner that is not authorized or permitted by HIPAA.

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, pharmaceutical sales and data protection.

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 14 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 reviewing strategic alternatives and there can be no assurance that we will be successful in identifying or completing any strategic transaction, that any such strategic transaction will result in additional value for our stockholders or that the process will not have an adverse impact on our business.

As a result of the negative outcomes of our ASCEND trial with AEVI-001 and our limited financial resources, we have begun exploring strategic alternatives, which could include, but are not limited to, issuing or transferring shares of our common stock or other equity securities, the license, sale or disposition of certain assets or programs, the formation of a joint venture, a strategic business combination, a transaction that results in private ownership or the sale of the Company, or some combination of these, in addition to other potential actions aimed at increasing stockholder value. There can be no assurance that the review of strategic alternatives will result in the identification or consummation of any transaction. Our board of directors may also determine that our most effective strategy is to continue to execute on our current development strategy or to cease our current drug development activities altogether. The process of reviewing strategic alternatives may be time consuming and disruptive to our business operations and, if we are unable to effectively manage the process, our business, financial condition and results of operations could be adversely affected. We could incur substantial expenses associated with identifying, evaluating and negotiating potential strategic alternatives. There can be no assurance that any potential transaction or other strategic alternative, if consummated, will provide greater value to our stockholders than that reflected in the current price of our common stock. Until the review process is concluded, perceived uncertainties related to our future may result in the loss of potential business opportunities and volatility in the market price of our common stock and may make it more difficult for us to attract and retain qualified personnel and business partners.

Additionally, we continue to pursue discussions related to potentially expanding the company's pipeline of development programs via the in-license or acquisition of future product development candidates. There can be no assurance that these discussions will results in completed transactions.

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 $30.78 million and $34.71 million for the years ended December 31, 2018 and 2017, respectively. As of December 31, 2018, we had stockholders' equity of approximately $7.93 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 fund the CHOP Research Agreement and continue 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 raise working capital or 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, 2018, our cash and cash equivalents were approximately $12.08 million. As of February 28, 2019, our cash and cash equivalents were approximately $8.34 million. Based upon current management projections, we expect the current cash balance to fund operations into early in the third 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 failure of our AEVI-001 trials in ADHD, which has caused us to terminate all activities related to this program. Those terminations could result in additional wind-down costs;
- 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 ongoing AEVI-002 proof-of-concept clinical trial in pediatric onset Crohn's Disease has been challenging. No patients have yet been recruited into the clinical trial. The ability to produce initial data by mid-year 2019 is directly based on timely recruiting; thus, continued difficulties in recruitment could further impact the Company's ability to generate initial 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;
- the effect of competing product and market developments;
- costs of recruiting and retaining qualified personnel;
- the timing and amount of milestone payments we are required to make under our license agreements;
- in-licensing and/or acquisition transaction costs (if any) for potential product development candidates;
- 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.

If our process to identify and evaluate potential business alternatives is not successful, our board of directors may decide to pursue a restructuring, which may include a reorganization or bankruptcy under federal bankruptcy laws, or a dissolution, liquidation and/or winding up of the Company.

There can be no assurance that the process to identify and evaluate potential business alternatives will result in a successful alternative for our business. If no transactions with respect to potential business alternatives are identified and completed, our board of directors may decide to pursue a restructuring, which may include a reorganization or bankruptcy under federal bankruptcy laws, or a dissolution, liquidation and/or winding up of our company. If our board of directors were to approve and recommend, and our stockholders were to approve, a dissolution and liquidation of the Company, we would be required under Delaware corporate law to pay our outstanding obligations, as well as to make reasonable provision for contingent and unknown obligations, prior to making any distributions in liquidation to our stockholders. Our commitments and contingent liabilities may include (i) obligations under our employment and separation agreements with certain members of its management that provide for severance and other payments following a termination of employment occurring for various reasons, including a change in control of our company, (ii) various claims and legal actions arising in the ordinary course of business, (iii) obligations to CHOP pursuant to the Research Agreement, and (iv) non-cancelable lease obligations. As a result of this requirement, a portion of our assets may need to be reserved pending the resolution of such obligations. In addition, we may be subject to litigation or other claims related to a dissolution and liquidation of the Company. If a dissolution and liquidation were pursued, our board of directors, in consultation with its advisors, would need to evaluate these matters and make a determination about a reasonable amount to reserve. Accordingly, holders of our common stock may lose their entire investment in the event of a reorganization, bankruptcy, liquidation, dissolution or winding up of the Company.

If we are successful in completing a strategic transaction, we may be exposed to other operational and financial risks.

Although there can be no assurance that a strategic transaction will result from the process we have undertaken to identify and evaluate strategic alternatives, the negotiation and consummation of any such transaction will require significant time on the part of our management, and the diversion of management's attention may disrupt our business.

The negotiation and consummation of any such transaction may also require more time or greater cash resources than we anticipate and expose us to other operational and financial risks, including:

- increased near-term and long-term expenditures;
- exposure to unknown liabilities;
- higher than expected acquisition or integration costs;
- incurrence of substantial debt or dilutive issuances of equity securities to fund future operations;
- write-downs of assets or goodwill or incurrence of non-recurring, impairment or other charges;
- increased amortization expenses;
- difficulty and cost in combining the operations and personnel of any acquired business with our operations and personnel;
- impairment of relationships with key suppliers or customers of any acquired business due to changes in management and ownership; and
- inability to retain key employees of our company or any acquired business.

Any of the foregoing risks could have a material adverse effect on our business, financial condition and prospects.

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-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-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 8-week Phase Ib proof-of-concept study of AEVI-002 in subjects with a diagnosis of severe pediatric-onset Crohn's disease. 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. 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. The basis 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 candidate, 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 historical efforts and expenses were focused on the development of AEVI-001, which was unsuccessful. A substantial portion of our efforts and expenses are currently focused on the development of 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-002, AEVI-005 and other product candidates we are in the early stages of developing. There is no guarantee that we will succeed in developing AEVI-002, AEVI-005 or any of our product candidates. If the development of AEVI-002 or other product candidates 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-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-002 or any other product development candidates. If subsequent clinical trials involving AEVI-002 or other product development candidates 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-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 medical affairs, 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 medical affairs, marketing and sales staff or distribution capabilities. Developing medical affairs as well as 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 medical affairs, marketing, sales and distribution operations. If we fail to establish successful medical affairs, 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 medical affairs, 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 medical affairs, 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.

We intend to expend our limited resources to pursue AEVI-002 for pediatric onset Crohn's disease and may fail to capitalize on other product candidates or other indications for AEVI-002 that may be more profitable or for which there is a greater likelihood of success.

Because we have limited financial and managerial resources, we are focusing on research programs relating to AEVI-002 for pediatric Crohn's disease, which concentrates the risk of product failure in the event it proves to be unsafe or ineffective or inadequate for clinical development or commercialization. As a result, we may forego or delay pursuit of opportunities with other product candidates or for other indications for AEVI-002 that could later prove to have greater commercial potential. We may also deem it advisable to refocus our clinical development programs based on clinical trial results. Our resource allocation decisions may cause us to fail to capitalize on viable commercial products or profitable market opportunities. Our spending on proprietary research and development programs relating to AEVI-002 may not yield any commercially viable products.

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 -002, are under development for indications for which off-label use is possible. 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.

If we receive regulatory approvals, we intend to market AEVI-002 in multiple jurisdictions where we have limited or no operating experience and may be subject to increased business and economic risks that could affect our financial results.

If we receive regulatory approvals, we may plan to market AEVI-002 in jurisdictions where we have limited or no experience in marketing, developing and distributing our products. Certain markets have substantial legal and regulatory complexities that we may not have experience navigating. We are subject to a variety of risks inherent in doing business internationally, including risks related to the legal and regulatory environment in non-U.S. jurisdictions, including with respect to privacy and data security, trade control laws and unexpected changes in laws, regulatory requirements and enforcement, as well as risks related to fluctuations in currency exchange rates and political, social and economic instability in foreign countries. If we are unable to manage our international operations successfully, our financial results could be adversely affected.

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-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-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 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;

- 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, 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.

Comparable laws and regulations exist in countries within the European Economic Area, or EEA. Although such laws are partially based upon European Union law, they may vary from country to country. Healthcare specific, as well as general European Union and national laws, regulations and industry codes constrain, for example, our interactions with government officials and healthcare professionals, and the collection and processing of personal health data. Non-compliance with any of these laws or regulations could lead to criminal or civil liability.

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 contractors, clinical data management organizations, independent contractors, medical institutions and clinical investigators to conduct our clinical trials of AEVI-002 and for our other development candidate 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 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.

Even if AEVI-002 advances through pre-clinical studies and clinical trials, we may experience difficulties in managing our growth and expanding our operations.

We have limited resources to carry out objectives for our current and future pre-clinical studies and clinical trials. In addition, while we have experienced management and expect to contract out many of the activities related to conducting these programs, we are a small company with only 14 employees and therefore have limited internal resources both to conduct pre-clinical studies and clinical trials and to monitor third-party providers. As our product candidates advance through pre-clinical studies and clinical trials, we will need to expand our development, regulatory and manufacturing operations, either by expanding our internal capabilities or contracting with other organizations to provide these capabilities for us. In the future, we expect to have to manage additional relationships with collaborators or partners, suppliers and other organizations. Our ability to manage our operations and future growth will require us to continue to improve our operational, financial and management controls, reporting systems and procedures.

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 candidate, AEVI-002, is still in development and is 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-002, AEVI-005 and other product development candidates we are in the early stages of pursuing. These personnel 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.

Our employees may engage in misconduct or other improper activities, including noncompliance with regulatory standards and requirements, which could subject us to significant liability and harm our reputation.

We are exposed to the risk of employee fraud or other misconduct. Misconduct by employees could include intentional failures to comply with FDA or to provide accurate information to the FDA. In addition, misconduct by employees could include intentional failures to comply with certain manufacturing standards, to comply with U.S. federal and state healthcare fraud and abuse laws and regulations, to report financial information or data accurately or to disclose unauthorized activities to us. Employee misconduct could also involve the improper use of information obtained in the course of clinical trials, which could result in regulatory sanctions and serious harm to our reputation. We have implemented, and will enforce, a Code of Business Conduct and Ethics, but it is not always possible to identify and deter employee misconduct, and the precautions we take to detect and prevent this activity, such as employee training on enforcement of the Code of Business Conduct and Ethics, may not be effective in controlling unknown or unmanaged risks or losses or in protecting us from governmental investigations or other actions or lawsuits stemming from a failure to be in compliance with such laws or regulations. If any such actions are instituted against us, and we are not successful in defending ourselves or asserting our rights, those actions could have a significant impact on our business and results of operations, including the imposition of significant fines or other sanctions.

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 FDARA into law, imposing 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.

Regardless of merit or eventual outcome, product liability claims may result in, among other things:

- withdrawal of patients from our clinical trials;
- substantial monetary awards to patients or other claimants;
- decreased demand for our product candidates following marketing approval, if obtained;
- damage to our reputation and exposure to adverse publicity;
- increased warnings on product labels imposed by regulators;
- litigation costs;
- distraction of management's attention from our primary business;
- loss of revenue; and
- the inability to successfully commercialize our product candidates following approval, if approved.

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. Cyber-attacks are becoming more sophisticated and frequent, and our systems could be the target of malware and other cyber-attacks. We have invested in our systems and the protection of our data to reduce the risk of an intrusion or interruption, and we monitor our systems on an ongoing basis for any current or potential threats. We can give no assurances that these measures and efforts will prevent interruptions or breakdowns. If we are unable to detect or prevent a security breach or cyber-attack or other disruption from occurring, then we could incur losses or damage to our data, or inappropriate disclosure of our confidential information or that of others; and we could sustain damage to our reputation, suffer disruptions to our research and development and incur increased operating costs including costs to mitigate any damage caused and protect against future damage, and be exposed to additional regulatory scrutiny or penalties and to civil litigation and possible financial liability. For instance, the loss of preclinical or clinical data could result in delays in our development and regulatory filing efforts and significantly increase our costs. 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.

Risks Related to Our Intellectual Property

If we are unable to obtain and maintain sufficient intellectual property protection for our product candidates, or if the scope of the intellectual property protection obtained is not sufficiently broad, our competitors could develop and commercialize product candidates similar or identical to ours, and our ability to successfully commercialize our product candidates may be impaired.

As is the case with other biopharmaceutical companies, our success depends in large part on our ability to obtain and maintain protection of the intellectual property we may own solely and jointly with others, particularly patents, in the United States and other countries with respect to our product candidates and technology. We seek to protect our proprietary position by in-licensing AEVI-002 IP, and by filing patent applications in the United States and abroad related to AEVI-004 or other product candidates that we may identify.

Obtaining and enforcing biopharmaceutical patents is costly, time consuming and complex, and we may not be able to file and prosecute all necessary or desirable patent applications, or maintain, enforce and license any patents that may issue from such patent applications, at a reasonable cost or in a timely manner. It is also possible that we will fail to identify patentable aspects of our research and development output before it is too late to obtain patent protection. We may not have the right to control the preparation, filing and prosecution of patent applications, or to maintain the rights to patents licensed to third parties. Therefore, these patents and applications may not be prosecuted and enforced in a manner consistent with the best interests of our business.

The patent position of biotechnology and pharmaceutical companies generally is highly uncertain, involves complex legal, technological and factual questions and has in recent years been the subject of much litigation. 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, the patents we own and license, or any further patents we may own or license, may not prevent other persons or companies from developing similar or therapeutically 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. Patents also will not protect our product candidates if competitors devise ways of making or using these product candidates without legally infringing our patents. Furthermore, our own issued and in-licensed patents may not be valid or enforceable or be able to provide our company with meaningful protection. 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. 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.

In addition, the laws of foreign countries may not protect our rights to the same extent as the laws of the United States, or vice versa. Further, we may not be aware of all third-party intellectual property rights potentially relating to our product candidates. Publications of discoveries in the scientific literature often lag behind the actual discoveries, and patent applications in the United States and other jurisdictions are typically not published until 18 months after filing or, in some cases, not at all. Therefore, we cannot know with certainty whether we were the first to make the inventions claimed in our patents or pending patent applications, or that we were the first to file for patent protection of such inventions. Moreover, we may be subject to a third-party preissuance submission of prior art to the United States Patent and Trademark Office, or the USPTO. As a result, the issuance, scope, validity, enforceability and commercial value of our patent rights are highly uncertain. Our pending and future patent applications may not result in patents being issued that protect our product candidates, in whole or in part, or which effectively prevent others from commercializing competitive product candidates. Even if our patent applications issue as patents, they may not issue in a form that will provide us with any meaningful protection, prevent competitors from competing with us or otherwise provide us with any competitive advantage. Our competitors may be able to circumvent our patents by developing similar or alternative product candidates in a non-infringing manner.

In addition, even if patents do issue to us or our licensors covering embodiments of our product candidates, devices, or methods of using them, the issuance of a patent is not conclusive as to its inventorship, scope, validity or enforceability, and those patents can be challenged by our competitors or other third parties in the courts or patent offices in the United States and abroad. For example, we may become involved in opposition, derivation, reexamination, *inter partes* review, post-grant review or interference proceedings challenging our patent rights or the patent rights of others. Such challenges may result in loss of exclusivity or freedom to operate or in patent claims being narrowed, invalidated or held unenforceable, in whole or in part, which could limit our ability to stop others from using or commercializing similar or identical product candidates, or limit the duration of the patent protection of our product candidates. An adverse determination in any such submission, proceeding or litigation could reduce the scope of, or invalidate, our patent rights, allow third parties to commercialize our product candidates and compete directly with us, without payment to us, or result in our inability to manufacture or commercialize drugs without infringing third-party patent rights. In addition, if the breadth or strength of protection provided by our patents and patent applications is threatened, regardless of the outcome, it could dissuade companies from collaborating with us to license, develop or commercialize current or future product candidates.

If we fail to comply with our obligations in the agreements under which we license intellectual property rights from third parties or these agreements are terminated or we otherwise experience disruptions to our business relationships with our licensors, we could lose intellectual property 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. For example, we are party to a Development and Option Agreement with KHK under which we may license certain technology related AEVI-002. We may need to obtain additional licenses from others in the future to advance our research and development activities or allow the commercialization of AEVI-002 or any other product candidates we may identify and pursue. See the section entitled “Business” for a more detailed description of our current license agreements.

Our license agreements impose, and we expect that future license agreements will impose, various diligence, milestone payment, royalty, insurance and other obligations on us. In spite of our efforts, our licensors might conclude that we have materially breached our obligations under such license agreements and might therefore terminate the license agreements, thereby removing or limiting our ability to develop and commercialize products and technology covered by these license agreements. 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-002, AEVI-005 or any future product development candidates. 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, competitors or other third parties would have the freedom to seek regulatory approval of, and to market, products identical to AEVI-002 and we may be required to cease our development and commercialization of AEVI-002, AEVI-005 or any future product development candidates. Any of the foregoing could have a material adverse effect on our competitive position, business, financial conditions, 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-002, AEVI-005 or any future product development candidates;

- Lose patent protection for AEVI-002, AEVI-005 or any future product development candidates;
- experience significant delays in the development or commercialization of AEVI-002, AEVI-005 or any future product development 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 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. Disputes may arise regarding intellectual property subject to a licensing agreement, including:

- the scope of rights granted under the license agreement and other interpretation-related issues;
- the extent to which our product candidates, technology and processes infringe on intellectual property of the licensor that is not subject to the licensing agreement;
- the sublicensing of patent and other rights under our collaborative development relationships;
- our diligence obligations under the license agreement and what activities satisfy those diligence obligations;
- the inventorship and ownership of inventions and know-how resulting from the joint creation or use of intellectual property by our licensors and us and our partners; and
- the priority of invention of patented technology.

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 agreement, either of which could have a material adverse effect on our business, financial condition, results of operations and prospects. Moreover, if disputes over intellectual property that we have licensed prevent or impair our ability to maintain our current licensing arrangements on commercially acceptable terms, we may be unable to successfully develop and commercialize the affected product candidates, which could have a material adverse effect on our business, financial conditions, results of operations and prospects.

As we develop our product candidates, we may need to obtain additional 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.

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

We are party to license agreements and sponsored research agreements with CHOP and CAG, and a Development and Option Agreement with KHK pursuant to which we exclusively license certain technology related to the development of AEVI-002 and AEVI-005. Under our license agreements and sponsored research 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 and have certain ongoing payment obligations with respect to our sponsored research agreement. 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 the 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. In addition, if we do not have sufficient funds to pay our ongoing obligations under the CHOP development agreement, we may lose our rights under that agreement, which would negatively impact our development capabilities.

Third-party claims of intellectual property infringement may prevent or delay our development and commercialization efforts.

Our commercial success depends in part on our avoiding infringement of the patents and proprietary rights of third parties. However, our research, development and commercialization activities may be subject to claims that we infringe or otherwise violate patents or other intellectual property rights owned or controlled by third parties. There is a substantial amount of litigation, both within and outside the United States, involving patent and other intellectual property rights in the biotechnology and pharmaceutical industries, including patent infringement lawsuits, interferences, oppositions and *inter partes* review and reexamination proceedings before the USPTO, and corresponding foreign patent offices. Numerous U.S. and foreign issued patents and pending patent applications, which are owned by third parties, exist in the fields in which we are pursuing development candidates. As the biotechnology and pharmaceutical industries expand and more patents are issued, the risk increases that AEVI-002, AEVI-005 or other product development candidates that we may identify may be subject to claims of infringement of the patent rights of third parties.

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. There may be third-party patents or patent applications with claims to materials, formulations, methods of manufacture or methods for treatment related to the use or manufacture of AEVI-002, AEVI-005 or other product development candidates that we may identify. Because patent applications can take many years to issue, there may be currently pending patent applications which may later result in issued patents that AEVI-002, AEVI-005 or other product development candidates that we may identify may infringe. In addition, third parties may obtain patents in the future and claim that use of our technologies infringes upon these patents. If any third-party patents were held by a court of competent jurisdiction to cover the manufacturing process of AEVI-002, AEVI-005 or other product development candidates that we may identify, any molecules formed during the manufacturing process or any final product itself, the holders of any such patents may be able to block our ability to commercialize such product candidate unless we obtained a license under the applicable patents, or until such patents expire.

Similarly, if any third-party patents were held by a court of competent jurisdiction to cover aspects of our formulations, processes for manufacture or methods of use, including combination therapy, the holders of any such patents may be able to block our ability to develop and commercialize the applicable product candidate unless we obtained a license or until such patent expires. In either case, such a license may not be available on commercially reasonable terms or at all, or it may be non-exclusive, which could result in our competitors gaining access to the same intellectual property.

Parties making claims against us may obtain injunctive or other equitable relief, which could effectively block our ability to further develop and commercialize AEVI-002, AEVI-005 or other product development candidates that we may identify. Defense of these claims, regardless of their merit, would involve substantial litigation expense and would be a substantial diversion of employee resources from our business. In the event of a successful claim of infringement against us, we may have to pay substantial damages, including treble damages and attorneys' fees for willful infringement, pay royalties, redesign our infringing products or obtain one or more licenses from third parties, which may be impossible or require substantial time and monetary expenditure.

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.

Parties making claims against us may be able to sustain the costs of complex patent litigation more effectively than we can because they have substantially greater resources. Furthermore, because of the substantial amount of discovery required in connection with intellectual property litigation or administrative proceedings, there is a risk that some of our confidential information could be compromised by disclosure. In addition, any uncertainties resulting from the initiation and continuation of any litigation could have material adverse effect on our ability to raise additional funds or otherwise have a material adverse effect on our business, results of operations, financial condition and prospects.

Patent terms may be inadequate to protect our competitive position on our product candidates for an adequate amount of time.

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. In the United States, if all maintenance fees are timely paid, the natural expiration of a patent is generally 20 years from its earliest U.S. non-provisional filing date. Various extensions may be available, but the life of a patent, and the protection it affords, is limited. Even if patents covering our product candidates are obtained, once the patent life has expired, we may be open to competition from competitive products, including generics or biosimilars. Given the amount of time required for the development, testing and regulatory review of new product candidates, patents protecting such candidates might expire before or shortly after such candidates are commercialized. As a result, our owned and licensed patent portfolio may not provide us with sufficient rights to exclude others from commercializing products similar or identical to ours.

If we are not able to obtain patent term extension or non-patent exclusivity in the United States under the Hatch-Waxman Act and in foreign countries under similar legislation, thereby potentially extending the term of our marketing exclusivity for AEVI-002, AEVI-005 or other product development candidates that we may identify, our business may be materially harmed.

Depending upon the timing, duration and specifics of FDA marketing approval of AEVI-002, AEVI-005 or other product development candidates that we may identify, one of the U.S. patents covering each of such product candidates or the use or manufacturing method thereof may be eligible for up to five years of patent term extension under the Hatch-Waxman Act. The Hatch-Waxman Act allows a maximum of one patent to be extended per FDA approved product as compensation for the patent term lost during the FDA regulatory review process. A patent term extension cannot extend the remaining term of a patent beyond a total of 14 years from the date of product approval and only those claims covering such approved drug product, a method for using it or a method for manufacturing it may be extended. Patent term extension also may be available in certain foreign countries upon regulatory approval of our product candidates. Nevertheless, we may not be granted patent term extension either in the United States or in any foreign country because of, for example, failing to exercise due diligence during the testing phase or regulatory review process, failing to apply within applicable deadlines, failing to apply prior to expiration of relevant patents or otherwise failing to satisfy applicable requirements. Moreover, the term of extension, as well as the scope of patent protection during any such extension, afforded by the governmental authority could be less than we request.

If we are unable to obtain patent term extension or restoration, or the term of any such extension is less than we request, the period during which we will have the right to exclusively market our product may be shortened and our competitors may obtain approval of competing products following our patent expiration sooner, and our revenue could be reduced, possibly materially.

It is possible that we will not obtain patent term extension under the Hatch-Waxman Act for a U.S. patent covering AEVI-002 or other product candidates that we may identify even where that patent is eligible for patent term extension, or if we obtain such an extension, it may be for a shorter period than we had sought. Further, for certain of our licensed patents, we do not have the right to control prosecution, including filing with the USPTO, a petition for patent term extension under the Hatch-Waxman Act. Thus, if one of our licensed patents is eligible for patent term extension under the Hatch-Waxman Act, we may not be able to control whether a petition to obtain a patent term extension is filed, or obtained, from the USPTO.

Also, there are detailed rules and requirements regarding the patents that may be submitted to the FDA for listing in the Approved Drug Products with Therapeutic Equivalence Evaluations, or the Orange Book. We may be unable to obtain patents covering our product candidates that contain one or more claims that satisfy the requirements for listing in the Orange Book. Even if we submit a patent for listing in the Orange Book, the FDA may decline to list the patent, or a manufacturer of generic drugs may challenge the listing. If one of our product candidates is approved and a patent covering that product candidate is not listed in the Orange Book, a manufacturer of generic drugs would not have to provide advance notice to us of any abbreviated new drug application filed with the FDA to obtain permission to sell a generic version of such product candidate.

If we are unable to protect the confidentiality of our trade secrets, the value of our technology could be materially adversely effected and our business would be harmed.

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 patents, we also rely on trade secrets, technical know-how, licensing opportunities, and continuing innovation to develop and maintain our competitive position especially where we do not believe that patent protection is appropriate or obtainable. Trade secrets are by nature difficult to protect. 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. We also require confidentiality or material transfer agreements from third parties that receive our confidential data or materials. These agreements are designed to protect our proprietary information. However, we cannot be certain that such agreements have been entered into with all relevant parties, and even if they are all in place, there can still be no guarantee that agreements have not been or will not be violated or that there will be an adequate remedy available for a violation of an agreement. Accordingly, we cannot be certain that our trade secrets and other confidential proprietary information will not be disclosed or that competitors will not otherwise gain access to our trade secrets or independently develop substantially equivalent information and techniques. For example, if 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, others, including our competitors, may independently develop substantially equivalent proprietary information and techniques or otherwise gain access to our trade secrets or technology.

We also seek to preserve the integrity and confidentiality of our confidential proprietary information by maintaining physical security of our premises and physical and electronic security of our information technology systems, but it is possible that these security measures could be breached. If any of our confidential proprietary information were to be lawfully obtained or independently developed by a competitor, we would have no right to prevent such competitor from using that technology or information to compete with us, which could harm our competitive position.

We anticipate that we will spend both time and management resources to develop and file trademark applications in the future. However, third parties may have trademarks or pending trademark 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.

Although we are not currently involved in any intellectual property litigation, we may become involved in lawsuits to protect or enforce our patents or other intellectual property, which could be expensive, time consuming and unsuccessful.

Unauthorized parties may infringe our patents or other intellectual property, 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.

Although we are not currently involved in any litigation, if we were to initiate legal proceedings against a third party to enforce a patent covering product candidates that we may identify, the defendant could counterclaim that the patent covering our product candidate is invalid and/or unenforceable. In patent litigation in the United States, defendant counterclaims alleging invalidity and/or unenforceability are commonplace. Grounds for a validity challenge could be an alleged failure to meet any of several statutory requirements, including lack of novelty, obviousness, written description or non-enablement. Grounds for an unenforceability assertion could be an allegation that someone connected with prosecution of the patent withheld relevant information from the USPTO, or made a misleading statement, during prosecution. The outcome following legal assertions of invalidity and unenforceability is unpredictable. Interference or derivation proceedings provoked by third parties or brought by us or declared by the USPTO may be necessary to determine the priority of inventions with respect to our patents or patent applications. An unfavorable outcome could require us to cease using the related technology or to attempt to license rights to it from the prevailing party. Our business could be harmed if the prevailing party does not offer us a license on commercially reasonable terms or at all, or if a non-exclusive license is offered and our competitors gain access to the same technology. Our defense of litigation or interference or derivation proceedings may fail and, even if successful, may result in substantial costs and distract our management and other employees. In addition, the uncertainties associated with litigation could have a material adverse effect on our ability to raise the funds necessary to continue our clinical trials, continue our research programs, license necessary technology from third parties, or enter into development partnerships that would help us bring AEVI-002 or other product candidates that we may identify to market. Furthermore, because of the substantial amount of discovery required in connection with intellectual property litigation, there is a risk that some of our confidential information could be compromised by disclosure during this type of litigation. There could also be public announcements of the results of hearings, motions, or other interim proceedings or developments. If securities analysts or investors perceive these results to be negative, it could have a material adverse effect on the price of our common stock.

We may be subject to claims challenging the inventorship of our patents and other intellectual property.

We or our licensors may be subject to claims that former employees, collaborators or other third parties have an interest in our owned or in-licensed patents, trade secrets, or other intellectual property as an inventor or co-inventor. For example, we or our licensors may have inventorship disputes arise from conflicting obligations of employees, consultants or others who are involved in developing our product candidates. Litigation may be necessary to defend against these and other claims challenging inventorship or our or our licensors' ownership of our owned or in-licensed patents, trade secrets or other intellectual property. If we or our licensors fail in defending any such claims, in addition to paying monetary damages, we may lose valuable intellectual property rights, such as exclusive ownership of, or right to use, intellectual property that is important to our product candidates. Even if we are successful in defending against such claims, litigation could result in substantial costs and be a distraction to management and other employees. Any of the foregoing could have a material adverse effect on our business, financial condition, results of operations and prospects.

Any issued patents that may cover our product candidates could be found invalid or unenforceable if challenged in court.

Third parties may claim that our owned or in-licensed patents relating to AEVI-004 or other product candidates that we may identify, are invalid and/or unenforceable. In patent litigation in the United States, defendant counterclaims alleging invalidity and/or unenforceability are commonplace. Grounds for a validity challenge could be an alleged failure to meet any of several statutory requirements, including lack of novelty, obviousness or non-enablement. Grounds for an unenforceability assertion could be an allegation that someone connected with prosecution of the patent withheld relevant information from the USPTO, or made a misleading statement, during prosecution. Third parties may also raise similar claims before administrative bodies in the United States or abroad, even outside the context of litigation. Such mechanisms include re-examination, post grant review, and equivalent proceedings in foreign jurisdictions (e.g., opposition proceedings). Such proceedings could result in revocation or amendment to our patents in such a way that they no longer cover AEVI-002 or other product candidates that we may identify. The outcome following legal assertions of invalidity and unenforceability is unpredictable. With respect to the validity question, for example, we cannot be certain that there is no invalidating prior art, of which we and the patent examiner were unaware during prosecution. If a defendant were to prevail on a legal assertion of invalidity and/or unenforceability, we would lose at least part, and perhaps all, of the patent protection on our product candidates. Such a loss of patent protection would have a material adverse impact on our business.

We may be subject to claims that our employees, consultants or independent contractors have wrongfully used or disclosed confidential information of third parties or that our employees have wrongfully used or disclosed alleged trade secrets of their former employers.

As is common in the biotechnology and pharmaceutical industry, we employ individuals who were previously employed at universities or other biotechnology or pharmaceutical companies, including our competitors or potential competitors. Agreements with our employees aim to prevent employees from bringing any proprietary rights of third parties to us. Although we try to ensure that our employees, consultants and independent contractors do not use the proprietary information or know-how of others in their work for us, we may be subject to claims that we or our employees, consultants or independent contractors have inadvertently or otherwise used or disclosed intellectual property, including trade secrets or other proprietary information, of any of our employee's former employer or other third parties. Litigation may be necessary to defend against these claims. If we fail in defending any such claims, in addition to paying monetary damages, we may lose valuable intellectual property rights or personnel, which could adversely impact our business. Even if we are successful in defending against such claims, litigation could result in substantial costs and be a distraction to management and other employees.

Obtaining and maintaining our patent protection depends on compliance with various procedural, document submission, fee payment and other requirements imposed by governmental patent agencies, and our patent protection could be reduced or eliminated for non-compliance with these requirements.

Periodic maintenance fees, renewal fees, annuity fees and various other governmental fees on patents and/or applications will be due to be paid to the USPTO and various governmental patent agencies outside of the United States in several stages over the lifetime of the patents and/or applications. We have systems in place to remind us to pay these fees, and we employ an outside agency and rely on our outside agency to pay these fees due to U.S. and non-U.S. patent agencies. The USPTO and various non-U.S. governmental patent agencies require compliance with a number of procedural, documentary, fee payment and other similar provisions during the patent application process. We employ reputable professionals to help us comply, and in many cases, an inadvertent lapse can be cured by payment of a late fee or by other means in accordance with the applicable rules. However, there are situations in which non-compliance can result in abandonment or lapse of the patent or patent application, resulting in partial or complete loss of patent rights in the relevant jurisdiction. In such an event, our competitors might be able to enter the market and this circumstance would have a material adverse effect on our business.

We may not be able to protect our intellectual property rights throughout the world.

Filing, prosecuting and defending patents on our product candidates in all countries throughout the world would be prohibitively expensive, and our intellectual property rights in some countries outside the United States can be less extensive than those in the United States. In addition, the laws of some foreign countries do not protect intellectual property rights to the same extent as federal and state laws in the United States. For example, 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. 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.

Consequently, we may not be able to prevent third parties from practicing our inventions in all countries outside the United States, or from selling or importing products made using our inventions in and into the United States or other jurisdictions. Competitors may use our technologies in jurisdictions where we have not obtained patent protection to develop their own products and may also export infringing products to territories where we have patent protection, but enforcement is not as strong as that in the United States. These products may compete with our products and our patents or other intellectual property rights may not be effective or sufficient to prevent them from competing.

Many companies have encountered significant problems in protecting and defending intellectual property rights in foreign jurisdictions. The legal systems of certain countries, particularly certain developing countries, do not favor the enforcement of patents, trade secrets, and other intellectual property protection, particularly those relating to biotechnology products, which could make it difficult for us to stop the infringement of our patents or marketing of competing products in violation of our proprietary rights generally. Proceedings to enforce our patent rights in foreign jurisdictions, whether or not successful, could result in substantial costs and divert our efforts and attention from other aspects of our business, could put our patents at risk of being invalidated or interpreted narrowly and our patent applications at risk of not issuing and could provoke third parties to assert claims against us. We may not prevail in any lawsuits that we initiate and the damages or other remedies awarded, if any, may not be commercially meaningful. Accordingly, our efforts to enforce our intellectual property rights around the world may be inadequate to obtain a significant commercial advantage from the intellectual property that we develop or license.

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

Changes in either the patent laws or interpretation of the patent laws in the United States and abroad could increase the uncertainties and costs surrounding the prosecution of patent applications and the enforcement or defense of issued patents. For example, assuming that other requirements for patentability are met, prior to March 2013, in the United States, in general, the first to invent was entitled to the patent. After March 2013, under the Leahy-Smith America Invents Act, enacted in September 2011, the United States transitioned to a first inventor to file system in which, assuming that other requirements for patentability are met, the first inventor to file a patent application will be entitled to the patent on an invention regardless of whether a third party was the first to invent the claimed invention. A third party that files a patent application in the USPTO after March 2013, but before us could therefore be awarded a patent covering an invention of ours even if we had made the invention before it was made by such third party. This will require us to be cognizant of the time from invention to filing of a patent application. Since patent applications in the United States and most other countries are confidential for a period of time after filing or until issuance, we cannot be certain that we or our licensors were the first to either (i) file any patent application related to our product candidates or (ii) invent any of the inventions claimed in our or our licensor's patents or patent applications.

The America Invents Act also includes a number of significant changes that affect the way patent applications will be prosecuted and also may affect patent litigation. These include allowing third party submission of prior art to the USPTO during patent prosecution and additional procedures to attack the validity of a patent by USPTO administered post-grant proceedings, including post-grant review, *inter partes* review, and derivation proceedings. Because of a lower evidentiary standard in USPTO proceedings compared to the evidentiary standard in United States federal courts necessary to invalidate a patent claim, a third party could potentially provide evidence in a USPTO proceeding sufficient for the USPTO to hold a claim invalid even though the same evidence would be insufficient to invalidate the claim if first presented in a district court action. Accordingly, a third party may attempt to use the USPTO procedures to invalidate our patent claims that would not have been invalidated if first challenged by the third party as a defendant in a district court action. Therefore, the America Invents Act and its implementation could increase the uncertainties and costs surrounding the prosecution of our owned or in-licensed patent applications and the enforcement or defense of our owned or in-licensed issued patents, all of which could have a material adverse effect on our business, financial condition, results of operations, and prospects.

In addition, the patent positions of companies in the development and commercialization of pharmaceuticals are particularly uncertain. Recent U.S. Supreme Court rulings have narrowed the scope of patent protection available in certain circumstances and weakened the rights of patent owners in certain situations. This combination of events has created uncertainty with respect to the validity and enforceability of patents, once obtained. Depending on future actions by the U.S. Congress, the federal courts, and the USPTO, the laws and regulations governing patents could change in unpredictable ways that could have a material adverse effect on our existing patent portfolio and our ability to protect and enforce our intellectual property in the future.

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 $0.17 per share to $2.29 per share during the 52-week trading period ending March 26, 2019. 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, specifically the 8-week signal finding study of AEVI-002 in patients with severe pediatric onset Crohn's disease, for which initial data from a small number of patients is expected by mid-year 2019, if enrollment in the trial is successful, although enrollment into the study has been extremely challenging and no patients have been enrolled to date,
- 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, 2018, there were outstanding options and warrants to purchase an aggregate of 10,177,118 and 3,953,904 shares, respectively. Of the 10,177,118 outstanding options, ranging in exercise price from $1.07 per share to $8.80 per share, 6,027,586 shares were exercisable as of December 31, 2018. 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, or the CHOP Foundation, is our largest stockholder. As of March 26, 2019, the CHOP Foundation and certain of its related parties beneficially owned 21,311,586 shares of our common stock. The shares of common stock beneficially owned by the CHOP Foundation and certain of its related parties represent approximately 31.5% 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.

If we fail to comply with the continued listing requirements of the Nasdaq Global Market, our common stock may be delisted and the price of our common stock and our ability to access the capital markets could be negatively impacted.

Our common stock is listed for trading on the Nasdaq Global Market. We must satisfy Nasdaq's continued listing requirements, including, among other things, a minimum closing bid price requirement of $1.00 per share for our common stock. On February 6, 2019, we received a notification from Nasdaq that the closing bid price for our common stock had been below $1.00 for 30 consecutive business days.

If a company trades for 30 consecutive business days below the $1.00 minimum closing bid price requirement, Nasdaq will send a deficiency notice to the company, advising that it has been afforded a "compliance period" of 180 calendar days to regain compliance with the applicable requirements. Thereafter, if such a company does not regain compliance with the bid price requirement, a second 180-day compliance period may be available. If Nasdaq does not grant such a company a second 180-day compliance period or such company does not regain compliance with the bid price requirement, such company may be delisted from the Nasdaq Global Market.

A delisting of our common stock from Nasdaq could materially reduce the liquidity of our common stock and result in a corresponding material reduction in the price of our common stock. In addition, delisting could harm our ability to raise capital through alternative financing sources on terms acceptable to us, or at all, and may result in the potential loss of confidence by investors, employees and fewer business development opportunities.

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".

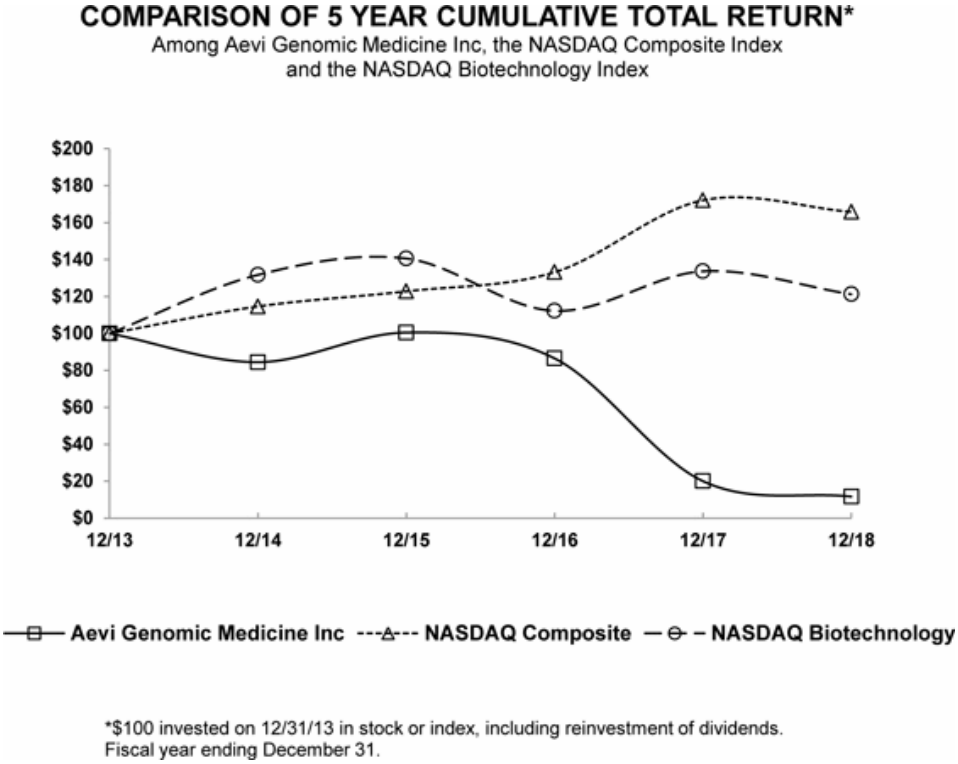
On October 10, 2016, we provided written notice to the NYSE MKT of our intention to voluntarily delist our common stock from the NYSE MKT and to list our common stock on the Nasdaq Global Market. The listing and trading of our common stock on the NYSE MKT ceased at market close on October 20, 2016, and 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". In order to maintain that listing on the Nasdaq Global Market, we must satisfy minimum financial and other requirements. On February 6, 2019, we received a notice from the Nasdaq Stock Market stating that, for 30 consecutive business days preceding the notice date, the closing bid price for our common stock had been below the minimum $1.00 per share requirement for continued listing on the Nasdaq Global Market as set forth in the applicable Nasdaq Listing Rule. We actively monitor the price of our common stock and will consider all available options to regain compliance with the continued listing standards of Nasdaq.

Holders of Record

As of March 26, 2019, there were 239 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, 2014 through December 31, 2018 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, 2014 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, 2018 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	7,477,118[2]	$ 3.73	4,674,838
Equity compensation plans not approved by security holders	2,700,000[3]	$ 4.21	—
Total	10,177,118	$ 3.86	4,674,838

(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 and Dr. Neil (900,000 options).

(ii) 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 16, 2026; and

(iii) 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.

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, 2018, 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,				
	2018	2017	2016	2015	2014
Statement of Operations Data:					
Operating expenses:					
Research and development expenses, net	$ 22,299	$ 25,176	$ 28,356	$ 15,444	$ 8,253
Non-recurring research and development expenses resulting from acquisition	-	-	-	8,170	-
General and administrative expenses	8,663	9,524	13,523	12,954	10,686
Operating loss	(30,962)	(34,700)	(41,879)	(36,568)	(18,939)
Financial expenses	(1)	(41)	(24)	(1,408)	(68)
Financial income	188	27	15	1	586
Loss before taxes on income	(30,775)	(34,714)	(41,888)	(37,975)	(18,421)
Taxes on income	-	-	16	17	12
Net loss	(30,775)	(34,714)	(41,904)	(37,992)	(18,433)
Basic loss per share	$ (0.50)	$ (0.83)	$ (1.19)	$ (1.42)	$ (0.96)
Diluted loss per share	$ (0.50)	$ (0.83)	$ (1.19)	$ (1.45)	$ (1.00)
Weighted average number of shares used in computing basic loss per share	61,381,611	41,675,814	35,161,823	26,783,623	19,246,611
Weighted average number of shares used in computing diluted loss per share	61,381,611	41,675,814	35,161,823	26,846,270	19,294,259
Statement of Cash Flows Data:					
Net cash used in operating activities	$ (26,649)	$ (33,246)	$ (32,749)	$ (24,347)	$ (12,195)
Net cash provided by (used in) investing activities	-	148	(221)	(187)	(363)
Net cash provided by financing activities	4,996	26,989	19,744	44,310	23,456
(Decrease) increase in cash and cash equivalents	(21,653)	(6,109)	(13,226)	19,776	10,898
Balance Sheet Data:					
Cash and cash equivalents	$ 12,076	$ 33,729	$ 39,838	$ 53,064	$ 33,288
Current assets	12,246	34,622	40,173	53,811	33,603
Long-term assets	31	139	388	447	677
Total assets	12,277	34,761	40,561	54,258	34,280
Current liabilities	4,345	4,140	5,583	3,908	3,638
Long-term liabilities	-	-	-	-	980

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 potential 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 $30.78 million and $34.71 million for the years ended December 31, 2018 and 2017, respectively. As of December 31, 2018 and February 28, 2019, we had cash and cash equivalents of $12.08 million and approximately $8.34 million, respectively, which we believe will provide funding for us into early in the third quarter of 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 initial program from our genomic research collaboration with CHOP was the development candidate AEVI-001, an oral, non-stimulant glutamatergic neuromodulator. Given the negative outcomes of the ASCEND trial with respect to the AEVI-001 program, we have terminated the AEVI-001 program.

AEVI-004 (novel co-crystal version of AEVI-001)

AEVI-004 is a co-crystal of AEVI-001, with enhanced physical and chemical properties. The new molecule has comparatively greater stability and a higher melting point than AEVI-001. The molecule was engineered to maintain solubility, dissolution and pharmacokinetics substantially similar to AEVI-001.

Given the negative outcomes of the ASCEND trial, there are no current clinical development plans for AEVI-004 in ADHD. However, the company is in discussions with the National Institutes of Health (NIH) regarding evaluating the potential anti-seizure activity of AEVI-004 in a preclinical model as part of the NIH Epilepsy Treatment Screening Program.

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

We are developing AEVI-002, a first-in-class anti-LIGHT monoclonal antibody, or the Antibody, 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."

A submission to reactivate the IND for AEVI-002 in Pediatric Crohn's Disease was filed with the FDA in 2017 and has passed the 30-day waiting period. An 8-week Phase Ib proof-of-concept study has been initiated at multiple clinical sites, 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 has been underway for over 12 months, and we have not enrolled any 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 by mid-year 2019, 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 mid-year 2019 is highly dependent on timely recruiting; thus, continued difficulties in recruitment could cause an extended delay or an inability to deliver the initial data for the program.

AEVI-005 *(Monoclonal Antibody)*

AEVI-005 is the second monoclonal antibody we are developing as part of our ongoing collaboration with KHK. We are studying AEVI-005 in an undisclosed ultra-orphan auto-immune pediatric disease. We initiated a preclinical research program with AEVI-005 in the second quarter of 2018.

Current Strategy

In light of our decision to discontinue the AEVI-001 program in ADHD, our board of directors has commenced a review to explore and evaluate potential strategic alternatives to enhance stockholder value. These alternatives could include, among others, continuing to execute the Company's business plan, issuing or transferring shares of our common stock or other equity securities, the license, sale or disposition of certain assets or programs, the formation of a joint venture, a strategic business combination, a transaction that results in private ownership or the sale of the Company, or some combination of these. There can be no assurance that the review of strategic alternatives will result in the identification or consummation of any transaction or that our board of directors will determine that continuing our current business operations is in the best interests of our stockholders.

Furthermore, we continue to pursue discussions related to potentially expanding the Company's pipeline of development programs through the in-license or acquisition of future product development candidates. There can be no assurance that these discussions will be successful.

Following the negative outcomes from the ASCEND trial early in the first quarter of 2019, the Company took numerous actions to closeout clinical research operations for the AEVI-001 program in ADHD and to scale back the scope of operations for the Company in general. These combined actions have significantly reduced the Company's monthly operating spend, thus providing a limited runway for the review of strategic alternatives described above.

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.

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 and decrease as personnel increase and decrease.

Financial Income and Expense

Financial income and expense consist primarily interest earned on cash and cash equivalents.

Results of Operations for the Year Ended December 31, 2018 and 2017

Research and Development Expenses

Research and development expenses for year ended December 31, 2018 decreased to $22.30 million from $25.18 million in 2017. This decrease was primarily driven by a reduction of expenses relating to development of AEVI-001 in ADHD.

General and Administrative Expenses

General and administrative expenses for the year ended December 31, 2018 were $8.66 million, decreasing from $9.52 million in 2017, due in part to a reduction in the scale of the Company's operations.

Financial Income and Expenses

Financial income and expenses for the years ended December 31, 2018 and 2017 were 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, 2018 and 2017, options and warrants were exercised in consideration of $0.03 million and $0.02 million, respectively, and 8,466 and 6,200 shares of common stock were issued upon such exercises, respectively.

On May 15, 2018, we entered into an Equity Distribution Agreement pursuant to which we may from time-to-time issue and sell shares of our common stock having an aggregate offering price of up to $20,000,000 in an "at the market offering" as defined in Rule 415(a)(4) promulgated under the Securities Act (the "ATM Facility"). For the year ended December 31, 2018, we sold 5,426,151 shares of common stock at an average purchase price of $0.97 per share of common stock for gross proceeds of $5.28 million and net proceeds after deducting estimated offering expenses of approximately $4.96 million under the ATM Facility.

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.

Cash Flows

We had cash and cash equivalents of $12.08 million at December 31, 2018 and $33.73 million at December 31, 2017. The decrease in our cash balance during 2018 was primarily related to advancement of our AEVI-001 ADHD program, offset by the 2018 funding activities.

Net cash used in operating activities of $26.65 million and $33.25 million for the years ended December 31, 2018 and 2017, respectively, primarily reflected our net cash expenses for our operations.

Net cash provided by investing activities for the year ended December 31, 2018 was de minimis.

Net cash provided by financing activities was $5.00 million and $26.99 million for the years ended December 31, 2018 and 2017, respectively resulting primarily from the issuance of shares of common stock.

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 early in the third quarter of 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 2019. 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 sell additional equity or debt securities or obtain a bank credit facility. The sale of additional equity or debt securities, if convertible, would 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.

In light of our decision to discontinue the AEVI-001 program in ADHD, our board of directors has commenced a review to explore and evaluate potential strategic alternatives to enhance stockholder value. These alternatives could include, among others, continuing to execute the Company's business plan, issuing or transferring shares of our common stock or other equity securities, the license, sale or disposition of certain assets or programs, the formation of a joint venture, a strategic business combination, a transaction that results in private ownership or the sale of the Company, or some combination of these. There can be no assurance that the review of strategic alternatives will result in the identification or consummation of any transaction or that our board of directors will determine that continuing our current business operations is in the best interests of our stockholders.

Contractual Obligations

The following table sets forth our contractual payment obligations as of December 31, 2018 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	$ 44,000	$ 44,000	$ -	$ -	$ -
Purchase obligations	$ 7,125,000	$ 4,750,000	$ 2,375,000	$ -	$ -
Total	$ 7,169,000	$ 4,794,000	$ 2,375,000	$ -	$ -

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. The purchase obligations presented consist solely of our obligations under the Sponsored Research Agreement with CHOP as of December 31, 2018. Per the employment agreements of several executives, if terminated without cause, these executives will be entitled to severance pay in the aggregate amount of $2.63 million.

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, 2018 and 2017, 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.

Off-Balance Sheet Arrangements

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. 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 the first extension was $197,603 with each subsequent extension costing $125,000. We have exercised such option in each of 2017 and 2018. 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.

In December 2015, we entered into an amendment to the Research Agreement, which amendment (i) set the payment schedule under such agreement through March 2017 and (ii) granted us the right to extend the term of the Research Agreement until November 12, 2017**.** In February 2017, we entered into a second amendment to the Research Agreement, which extended the term of the Research Agreement through June 30, 2018. This amendment also granted us rights to continually extend the term of the Research Agreement by one year by giving CHOP written notice of extension no later than one year prior to the expiration of the then-current term of the Research Agreement. In June 2017, we extended the term of the Research Agreement through June 30, 2019, and in June 2018, we extended the term of Research Agreement through June 30, 2020. $5.94 million was due for the Research Agreement in 2018. $4.75 million will be due under the Research Agreement in 2019, and in the first half of 2020, $2.38 million will be due.

In March 2019, we reached agreement with CHOP to further amend the Research Agreement and the License Agreement ("the CHOP Amendments"). The CHOP Amendments allow us to defer the monthly payments due under the Research Agreement for the period from February 1, 2019 through September 30, 2019 in exchange for a non-interest bearing note in the amount of such deferral. Such note matures September 30, 2019 and is secured by all of Aevi's intellectual property and other assets ("the Note"). At maturity, and at CHOP's option, the Note will be payable in cash or a number of shares of our common stock calculated based on the price of our common stock at such time; provided, however, if conversion upon such election would cause CHOP and its affiliates including the CHOP Foundation to own, in the aggregate, in excess of 47.5% of the then-outstanding shares of our common stock (after giving effect to such conversion), then CHOP would only receive the number of shares of our common stock such that CHOP and its affiliates including the CHOP Foundation would own, in the aggregate, 47.5% of the then outstanding shares of our common stock (after giving effect to such conversion), and the balance of the Note would be payable to CHOP in cash.

The CHOP Amendments with respect to the Research Agreement and the License Agreement prohibits the assignment or sublicense of CHOP's intellectual property without CHOP's prior written consent, allows CHOP to terminate the Research Agreement and the License Agreement upon a change of control without CHOP's prior written consent, reduces the period of time during which we have to exercise its options to license new intellectual property of CHOP and to negotiate the terms of any such license and requires us to meet certain diligence requirements related to acquiring rights to and commencing a clinical trial for a viable molecule that addresses the optioned intellectual property.

Furthermore, we have agreed that until and including June 23, 2019 the Company will not undertake any equity financing (including convertible notes) that would have a dilutive effect on the stockholders of Aevi. Thereafter, and until the later of repayment in full of the Note or June 30, 2020, Aevi has agreed to only undertake an equity financing (including convertible notes) if the net proceeds of such financing provide at least six month of cash to sustain our operations; provided, that CHOP will have a right of first refusal to purchase any or all equity proposed to be issued in such financing on equivalent terms.

Development and Option Agreement, with Kyowa Hakko Kirin Co., Ltd. (KHK) related to AEVI-002

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 (AEVI-002): 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 (AEVI-002): 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.

Research Collaboration and Option Agreement with Kyowa Hakko Kirin Co., Ltd. (KHK) related to AEVI-005

During 2018, we expanded our collaboration with KHK by entering a Research Collaboration and Option Agreement related to AEVI-005. AEVI-005 is the second monoclonal antibody we are developing as part of our ongoing collaboration with KHK. We are studying AEVI-005 in an undisclosed ultra-orphan auto-immune pediatric disease. We initiated a preclinical research program with AEVI-005 in the second quarter of 2018.

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 Israeli subsidiary as participation in the research and development program, plus interest at LIBOR. The obligation to pay these royalties is contingent on actual income. Proceeds from any potential transactions relating to the Israeli Subsidiary's research and development program may be subject to the terms and conditions of the OCS agreement. As of December 31, 2018, 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 SUBSIDIARIES

CONSOLIDATED FINANCIAL STATEMENTS
AS OF DECEMBER 31, 2018

REPORT OF INDEPENDENT REGISTERED PUBLIC ACCOUNTING FIRM

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

Opinion on the Financial Statements

We have audited the accompanying consolidated balance sheets of Aevi Genomic Medicine, Inc. and its subsidiaries (the Company) as of December 31, 2018 and 2017, and the related consolidated statements of operations, stockholders' equity and cash flows for the years then ended, 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, 2018 and 2017, and the consolidated results of their operations and their cash flows for the years then ended, 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 2016.

Philadelphia, Pennsylvania

March 29, 2019

AEVI GENOMIC MEDICINE, INC. AND ITS SUBSIDIARIES

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

	Note	December 31, 2018	December 31, 2017
ASSETS			
CURRENT ASSETS:			
Cash and cash equivalents	3	$ 12,076	$ 33,729
Prepaid expenses and other current assets		170	893
Total current assets		12,246	34,622
LONG-TERM ASSETS:			
Lease deposits	6(d)	11	11
Property and equipment, net	4	20	85
Other long-term assets		-	43
Total long-term assets		31	139
Total assets		$ 12,277	$ 34,761
LIABILITIES AND STOCKHOLDERS' EQUITY			
CURRENT LIABILITIES:			
Trade payables		$ 1,582	$ 943
Other accounts payable and accrued expenses	5	2,763	3,197
Total current liabilities		4,345	4,140
Total liabilities		4,345	4,140
COMMITMENTS AND CONTINGENCIES	6		
STOCKHOLDERS' EQUITY:	7		
Common stock - $0.0001 par value; 200,000,000 shares authorized; 64,766,882 shares issued and outstanding at December 31, 2018; 59,332,265 shares issued and outstanding at December 31, 2017		7	6
Additional paid-in capital		253,678	245,593
Accumulated deficit		(245,753)	(214,978)
Total stockholders' equity		7,932	30,621
Total liabilities and stockholders' equity		$ 12,277	$ 34,761

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

AEVI GENOMIC MEDICINE, INC. AND ITS SUBSIDIARIES

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

	Note	Year ended December 31, 2018	2017
Research and development expenses		$ 22,299	$ 25,176
General and administrative expenses		8,663	9,524
Operating loss		(30,962)	(34,700)
Financial expenses		(1)	(41)
Financial income		188	27
Loss before taxes on income		(30,775)	(34,714)
Taxes on income	8	-	-
Net loss		$ (30,775)	$ (34,714)
Basic loss per share	10	$ (0.50)	$ (0.83)
Diluted loss per share	10	$ (0.50)	$ (0.83)
Weighted average number of shares of common stock used in computing basic loss per share		61,381,611	41,675,814
Weighted average number of shares of common stock used in computing diluted loss per share		61,381,611	41,675,814

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

AEVI GENOMIC MEDICINE, INC. AND ITS SUBSIDIARIES

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, 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
Issuance of common stock at an average of $0.97 per share, net	5,426,151	1	4,961	-	4,962
Stock-based compensation related to options and warrants granted to consultants, directors and employees	-	-	3,090	-	3,090
Exercise of warrants and options	8,466	(*)	34	-	34
Net loss	-	-	-	(30,775)	(30,775)
Balance as of December 31, 2018	64,766,882	$ 7	$ 253,678	$ (245,753)	7,932

(*) Represents an amount lower than $1.

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

AEVI GENOMIC MEDICINE, INC. AND ITS SUBSIDIARIES

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

	Year ended December 31	
	2018	**2017**
CASH FLOWS FROM OPERATING ACTIVITIES:		
Net loss	$ (30,775)	$ (34,714)
Adjustments to reconcile loss to net cash used in operating activities:		
Depreciation	65	112
Loss from disposal of property and equipment	-	32
Stock-based compensation	3,090	3,368
Change in operating assets and liabilities:		
Prepaid and other current assets	723	(558)
Trade payables	639	806
Other accounts payable and accrued expenses	(434)	(2,249)
Lease deposits	-	-
Other long-term assets	43	(43)
Net cash used in operating activities	$ (26,649)	$ (33,246)
CASH FLOWS FROM INVESTING ACTIVITIES:		
Purchase of property and equipment	$ -	$ (4)
Proceeds from disposal of property and equipment	-	152
Net cash provided by (used in) investing activities	$ -	$ 148
CASH FLOWS FROM FINANCING ACTIVITIES:		
Proceeds from issuance of common stock and warrants, net	$ 4,962	$ 26,970
Proceeds from exercise of options and warrants	34	19
Net cash provided by financing activities	$ 4,996	$ 26,989
Increase (decrease) in cash and cash equivalents	(21,653)	(6,109)
Balance of cash and cash equivalents at the beginning of the period	33,729	39,838
Balance of cash and cash equivalents at the end of the period	$ 12,076	$ 33,729
Supplemental disclosure of cash flow information:		
Cash paid during the period for taxes	$ -	$ -

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 two wholly-owned subsidiaries (the "Subsidiaries"): Medgenics Medical Israel Ltd. (the "Israeli Subsidiary"), which was incorporated in Israel in March 2000; and Aevi Genomics Medicine Europe BVBA/SPRL, which was incorporated in Belgium in December 2018. 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, 2018 of $30,775 and had negative cash flow from operating activities of $26,649 during the twelve month period ended December 31, 2018. The accumulated deficit as of December 31, 2018 is $245,753. The Company and the Subsidiaries 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 Subsidiaries operate. Thus, the functional currency of the Company and its Subsidiaries 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, which will replace existing leasing guidance. ASU 2016-02 requires lessees to recognize operating and financing lease liabilities and related right-of-use assets, in addition to increased disclosures as to the nature of cash flows arising from a lease. We will adopt the new standard effective January 1, 2019, at which time we will not restate comparative periods. Adoption will not change the classification of any of our leases. We do not expect the new standard to have a material impact on our consolidated 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 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.

In June 2018, the FASB issued ASU 2018-07, Compensation–Stock Compensation (Topic 718): Improvements to Nonemployee Shared-Based Payment Accounting. This guidance is intended to simplify the accounting for share-based payments to nonemployees by aligning it with the accounting for share-based payments to employees, with certain exceptions. This guidance will be effective for annual reporting periods beginning after December 15, 2018, including interim periods within those annual reporting periods, and early adoption is permitted. The Company does not anticipate a material impact to the consolidated financial statements as a result of the adoption of this guidance.

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 Subsidiaries. Intercompany transactions and balances have been eliminated upon consolidation.

e. Cash equivalents:

The Company and the Subsidiaries 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, 2018.

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, 2018, 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, 2017 and 2018, 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 2017 and 2018, the Company estimated the fair value of stock options granted to employees and directors using the Binominal options pricing model with the following assumptions:

	2018	2017
Dividend yield	0%	0%
Expected volatility	77.5-77.9%	72.0-78.6%
Risk-free interest rate	2.7-3.1%	2.2-2.5%
Suboptimal exercise factor	1.5-2.5	1.5-2.5
Contractual life (years)	10	10
Exit rate	6%	6-8%

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.

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 the end of each reporting period up until the date of vesting and at the date of vesting, using the Binomial option pricing model with the following assumptions:

	2018	2017
Dividend yield	0%	0%
Expected volatility	77.9-77.9%	78.0-78.6%
Risk-free interest rate	2.7-2.7%	2.3-2.4%
Contractual life (years)	9.7-9.8	9.0-9.9

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:

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.

l. Concentrations of credit risks:

Financial instruments that potentially subject the Company and the Subsidiaries 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.

m. 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 third parties. As of December 31, 2018, the Company had cash and cash equivalents of $12,076 and liabilities of $4,345. The Company has incurred recurring operating losses since inception. For the year ended December 31, 2018, the Company incurred a net loss of $30,775 and as of December 31, 2018 the Company has an accumulated deficit of $245,753. 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, the board of directors has commenced a review to explore and evaluate potential strategic alternatives to enhance stockholder value. These alternatives could include, among others, continuing to execute the Company's business plan, issuing or transferring shares of its common stock or other equity securities, the license, sale or disposition of certain assets or programs, the formation of a joint venture, a strategic business combination, a transaction that results in private ownership or the sale of the Company, or some combination of these. There can be no assurance that the review of strategic alternatives will result in the identification or consummation of any transaction or that our board of directors will determine that continuing our current business operations is in the best interest of the Company's stockholders. 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-002, AEVI-005 and other product candidates, 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 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.

In light of our decision to discontinue the AEVI-001 program in ADHD, our board of directors has commenced a review to explore and evaluate potential strategic alternatives to enhance stockholder value. These alternatives could include, among others, continuing to execute the Company's business plan, issuing or transferring shares of our common stock or other equity securities, the license, sale or disposition of certain assets or programs, the formation of a joint venture, a strategic business combination, a transaction that results in private ownership or the sale of the Company, or some combination of these. There can be no assurance that the review of strategic alternatives will result in the identification or consummation of any transaction or that our board of directors will determine that continuing our current business operations is in the best interests of our stockholders.

NOTE 4: PROPERTY AND EQUIPMENT, NET

Composition of property and equipment is as follows:

	December 31,	
	2018	**2017**
Cost:		
Furniture and office equipment	$ -	$ -
Computers and peripheral equipment	35	35
Laboratory equipment	-	-
Leasehold improvements	157	157
Total cost	192	192
Total accumulated depreciation	172	107
Depreciated cost	$ 20	$ 85

Depreciation expense for the years ended December 31, 2018 and 2017 amounted to $65 and $112, 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. There were no disposals during the year ended December 31, 2018.

NOTE 5: OTHER ACCOUNTS PAYABLE AND ACCRUED EXPENSES

	December 31,	
	2018	2017
Employees and payroll accruals	$ 47	$ 1,297
R&D accruals	2,222	1,539
Accrued expenses, other	494	361
Other accounts payable and accrued expenses	$ 2,763	$ 3,197

NOTE 6: COMMITMENTS AND CONTINGENCIES

a. The Children's Hospital of Philadelphia (CHOP) Arrangements

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. A License Issuance Fee of $500 was paid and expensed in 2014. Beginning in 2016 and continuing through 2020, the Company paid, and is contractually required to pay, to CHOP an annual license maintenance fee of $100. This annual license maintenance fee increases to $200 beginning in 2021. The Company is required to 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 entering into liquidation, having a receiver or administrator appointed over any assets related to the License Agreement, makes any voluntary assignment of our assets for the benefit of creditors, ceases to carry on business, files for bankruptcy under Chapter 7 of the US Bankruptcy Code or has an involuntary petition under Chapter 7 of the US Bankruptcy Code filed against us.

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 the first extension was $198 with each subsequent extension costing $125. The Company has exercised such option in each of 2017 and 2018.

In December 2015, the Company entered into an amendment to the Research Agreement, which amendment, amongst other things, granted it the right to extend the term of the Research Agreement until November 12, 2017**.** In February 2017, the Company entered into a second amendment to the Research Agreement, which extended the term of the Research Agreement through June 30, 2018. This amendment also granted the Company rights to continually extend the term of the Research Agreement by one year by giving CHOP written notice of extension no later than one year prior to the expiration of the then-current term of the Research Agreement. In June 2017, the Company extended the term of the Research Agreement through June 30, 2019, and in June 2018, it extended the term of the Research Agreement through June 30, 2020. $5,937 was due under the Research Agreement in 2018. $4,750 will be due under the Research Agreement in 2019, and in the first half of 2020, $2,375 will be due.

In March 2019, the Company reached agreement with CHOP to further amend the Research Agreement and the License Agreement ("the CHOP Amendments"). The CHOP Amendments allow the Company to defer the monthly payments due under the Research Agreement for the period from February 1, 2019 through September 30, 2019 in exchange for a non-interest bearing note in the amount of such deferral. Such note matures September 30, 2019 and is secured by all of Aevi's intellectual property and other assets ("the Note"). At maturity, and at CHOP's option, the Note will be payable in cash or a number of shares of the Company's common stock calculated based on the price of the Company's common stock at such time; provided, however, if conversion upon such election would cause CHOP and its affiliates including the CHOP Foundation to own, in the aggregate, in excess of 47.5% of the then-outstanding shares of the Company's common stock (after giving effect to such conversion), then CHOP would only receive the number of shares of the Company common stock such that CHOP and its affiliates including the CHOP Foundation would own, in the aggregate, 47.5% of the then outstanding shares of the Company's common stock (after giving effect to such conversion), and the balance of the Note would be payable to CHOP in cash.

The CHOP Amendments with respect to the Research Agreement and the License Agreement prohibits the assignment or sublicense of CHOP's intellectual property without CHOP's prior written consent, allows CHOP to terminate the Research Agreement and the License Agreement upon a change of control without CHOP's prior written consent, reduces the period of time during which the Company has to exercise its options to license new intellectual property of CHOP and to negotiate the terms of any such license and requires the Company to meet certain diligence requirements related to acquiring rights to and commencing a clinical trial for a viable molecule that addresses the optioned intellectual property.

Furthermore, the Company has agreed that until and including June 23, 2019 the Company will not undertake any equity financing (including convertible notes) that would have a dilutive effect on the stockholders of Aevi. Thereafter, and until the later of repayment in full of the Note or June 30, 2020, Aevi has agreed to only undertake an equity financing (including convertible notes) if the net proceeds of such financing provide at least six month of cash to sustain the Company's operations; provided, that CHOP will have a right of first refusal to purchase any or all equity proposed to be issued in such financing on equivalent terms.

CHOP is the Company's largest shareholder and also has a seat on the Company's Board of Directors. Expenses related to CHOP, within the Research Agreement or otherwise, were $7,111 and $7,780 for the years ended December 31, 2018 and December 31, 2017, respectively. As of December 31, 2018, the Company had total payables related to CHOP, inclusive of those related to the Research Agreement, of $1,218, allocated between accrued expenses and trade payables.

b. License Agreements

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.

c. Office of the Chief Scientist (OCS):

Under agreements with the OCS in Israel regarding research and development projects, the Israeli 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 Israeli Subsidiary as participation in the research and development program, plus interest at LIBOR. The obligation to pay these royalties is contingent on actual income. The proceeds from any potential transactions relating to the Israeli Subsidiary's research and development program may be subject to the terms and conditions of the OCS agreement. As of December 31, 2018, the principal amount of the aggregate contingent liability was $13,968. The Israeli Subsidiary was not approved a grant from the OCS for 2017 and 2018.

d. 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 $44.

2. The following table sets forth our lease payment obligations as of December 31, 2018 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	$ 44	$ 44	$ -	$ -	$ -

e. 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 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.

2. On May 15, 2018, we entered into an Equity Distribution Agreement pursuant to which we may from time-to-time issue and sell shares of our common stock having an aggregate offering price of up to $20,000 in an "at the market offering" as defined in Rule 415(a)(4) promulgated under the Securities Act (the "ATM Facility"). For the year ended December 31, 2018, we sold 5,426,151 shares of common stock at an average purchase price of $0.97 per share of common stock for gross proceeds of $5,285 and net proceeds after deducting estimated offering expenses of approximately $4,962 under the ATM Facility.

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 Subsidiaries, 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. 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. In June 2018, stockholders approved an amendment to the Company's Stock Incentive Plan, increasing the number of shares authorized to be issued under such plan by 4,000,000 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, 2017	11,110,362	$ 4.34	6.43	$ 1
Granted	2,943,930	$ 1.54		
Exercised	(17,334)	$ 1.24		
Forfeited	(3,728,630)	$ 3.51		
Outstanding at December 31, 2018	10,308,328	$ 3.84	6.85	$ -
Vested and expected to vest, December 31, 2018	10,308,328	$ 3.84	6.85	$ -
Exercisable at December 31, 2018	6,158,796	$ 4.85	5.50	$ -

As of December 31, 2018, there was $2,679 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 1.41 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, 2017	160,000	$ 3.62	2.45	$ -
Granted	40,000	$ 1.52		
Exercised	-	$ -		
Forfeited	(190,000)	$ 3.12		
Outstanding at December 31, 2018	10,000	$ 4.82	7.84	$ -
Exercisable at December 31, 2018	10,000	$ 4.82	7.84	$ -

As of December 31, 2018, all compensation cost related to share-based compensation arrangements granted to consultants was recognized.

e. Compensation expense:

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,	
	2018	2017
Research and development expenses	$ 1,260	$ 1,515
General and administrative expenses	1,830	1,853
	$ 3,090	$ 3,368

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, 2018 is presented in the following table:

		As of December 31, 2018		
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	3,619,280	447,641	9.1
	3.14-4.91	4,403,900	3,564,632	5.9
	5.22-8.80	2,143,938	2,005,313	5.2
		10,167,118	6,017,586	
Granted to Consultants	4.82	10,000	10,000	7.8
Total Shares to be Issued upon Exercise of Options		10,177,118	6,027,586	
Warrants:				
Issued to Employees and Directors	2.84	141,210	141,210	3.8
Issued to Investors	2.84	3,812,694	3,812,694	3.8
Total Shares to be Issued upon Exercise of Warrants		3,953,904	3,953,904	
Total Shares to be Issued upon Exercise of Options and Warrants		14,131,022	9,981,490	

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.

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,	
	2018	2017
Rate reconciliation:		
Federal income tax benefit at statutory rate	21.0%	35.0%
State and local tax, net of federal benefit	5.7%	5.3%
Loss in earning of subsidiaries	0.0%	(1.0)%
Permanent differences	(0.9)%	(1.3)%
Tax credits	1.6%	2.1%
Tax attribute revaluations	(7.7)%	0.0%
Impact of tax reform	0.0%	(50.6)%
Change in valuation allowance	(19.7)%	10.5%
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,	
	2018	2017
Deferred tax assets:		
Net operating loss and credit carryforwards	$ 55,184	$ 45,548
Stock Compensation	4,969	7,065
Accrued Expenses	-	1,497
Other	36	23
Total deferred tax assets before valuation allowance	60,189	54,133
Valuation allowance	(60,189)	(54,133)
Net deferred tax asset	$ -	$ -

As of December 31, 2018, the Company had U.S. federal net operating loss carryforwards of $145,614, which may be available to offset future income tax liabilities and will expire beginning in 2020. As of December 31, 2018, the Company also had U.S. state net operating loss carryforwards of $138,622 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 deferred tax assets as of December 31, 2018 and 2017, 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 change in valuation allowance of $6,056 and $17,383 in the years ended December 31, 2018 and 2017, respectively.

As of December 31, 2018, the Company had federal research and development tax credit carryforwards of $2,630 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 2015 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, 2018, 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,	
	2018	**2017**
Financial expenses:		
Bank charges	(1)	$ (3)
Foreign currency remeasurement adjustments	-	(4)
Others	-	(34)
	(1)	$ (41)
Financial income:		
Foreign currency remeasurement adjustments	-	$ -
Interest on cash equivalents, short-term bank deposits	207	22
Others	(19)	5
	188	$ 27

NOTE 10: 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, 2018 and 2017.

The following table presents anti-dilutive shares for the year ended December 31, 2018 and 2017:

	Year ended December 31,	
	2018	**2017**
Weighted-average anti-dilutive shares related to:		
Outstanding stock options	10,221,139	11,105,065
Outstanding warrants	4,386,288	4,830,901
	14,607,427	15,935,966

NOTE 11: QUARTERLY FINANCIAL DATA

	Three Months Ended (Unaudited)			
	March 31	**June 30**	**September 30**	**December 31**
2018:				
R&D expenses	$ (6,561)	$ (5,747)	$ (5,125)	$ (4,866)
G&A expenses	$ (2,174)	$ (2,504)	$ (2,174)	$ (1,811)
Operating loss	$ (8,735)	$ (8,251)	$ (7,299)	$ (6,677)
Financial income (expense)	$ 26	$ 60	$ 50	$ 51
Net loss	$ (8,709)	$ (8,191)	$ (7,249)	$ (6,626)
Basic loss per share	$ (0.15)	$ (0.14)	$ (0.12)	$ (0.10)
Diluted loss per share	$ (0.15)	$ (0.14)	$ (0.12)	$ (0.10)
Weighted average number of shares used in computing basic loss per share	59,334,821	59,338,255	62,019,780	64,766,882
Weighted average number of shares used in computing diluted loss per share	59,334,821	59,338,255	62,019,780	64,766,882
2017:				
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

* * * * * * * * * * * * * * * * * * *

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, 2018, 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, 2018.

Changes in Internal Control Over Financial Reporting

There were no changes in our internal control over financial reporting during the year ended December 31, 2018 that have materially affected, or are reasonably likely to materially affect, our internal control over financial reporting.

ITEM 9B - Other Information.

Subsequent Events

On March 25, 2019, we and CHOP agreed to, and on March 29, 2019 we and CHOP entered into definitive agreements to, further amend the Research Agreement and the License Agreement. The CHOP Amendments allow the Company to defer the monthly payments due under the Research Agreement for the period from February 1, 2019 through September 30, 2019 in exchange for a non-interest bearing note in the amount of such deferral. Such note matures September 30, 2019 and is secured by all of Aevi's intellectual property and other assets. At maturity, and at CHOP's option, the Note will be payable in cash or a number of shares of the Company's common stock calculated based on the price of the Company's common stock at such time; provided, however, if conversion upon such election would cause CHOP and its affiliates including the CHOP Foundation to own, in the aggregate, in excess of 47.5% of the then-outstanding shares of the Company's common stock (after giving effect to such conversion), then CHOP would only receive the number of shares of the Company common stock such that CHOP and its affiliates including the CHOP Foundation would own, in the aggregate, 47.5% of the then outstanding shares of the Company's common stock (after giving effect to such conversion), and the balance of the Note would be payable to CHOP in cash.

The CHOP Amendments with respect to the Research Agreement and the License Agreement prohibits the assignment or sublicense of CHOP's intellectual property without CHOP's prior written consent, allows CHOP to terminate the Research Agreement and the License Agreement upon a change of control without CHOP's prior written consent, reduces the period of time during which the Company has to exercise its options to license new intellectual property of CHOP and to negotiate the terms of any such license and requires the Company to meet certain diligence requirements related to acquiring rights to and commencing a clinical trial for a viable molecule that addresses the optioned intellectual property.

Furthermore, the Company has agreed that until and including June 23, 2019 the Company will not undertake any equity financing (including convertible notes) that would have a dilutive effect on the stockholders of Aevi. Thereafter, and until the later of repayment in full of the Note or June 30, 2020, Aevi has agreed to only undertake an equity financing (including convertible notes) if the net proceeds of such financing provide at least six month of cash to sustain the Company's operations; provided, that CHOP will have a right of first refusal to purchase any or all equity proposed to be issued in such financing on equivalent terms.

As of March 26, 2019, and without giving effect to any potential conversion of the Note, the CHOP Foundation and certain related parties beneficially owned approximately 31.5% of our outstanding shares of common stock. The Company has other business relationships with CHOP, including the Research Agreement and the License Agreement, as further described in the Company's public filings with the SEC, including in this Annual Report on Form 10-K.

The sale and issuance of the Note is exempt from the registration requirements of the Securities Act pursuant to the exemption for transactions by an issuer not involving any public offering under Section 4(a)(2) of the Securities Act and Rule 506 of Regulation D of the Securities Act and in reliance on similar exemptions under applicable state laws. CHOP has represented that it is an accredited investor within the meaning of Rule 501(a) of Regulation D, and was acquiring the securities for investment only and not with a view towards, or for resale in connection with, the public sale or distribution thereof. The securities were offered without any general solicitation by the Company or its representatives. The securities sold and issued have not been registered under the Securities Act or any state securities laws and may not be offered or sold in the United States absent registration with the SEC or an applicable exemption from the registration requirements.

On March 26, 2019, the Company entered into a Retention Bonus Agreement with Brian Piper, the Company's Chief Financial Officer, that provides for a cash bonus to be paid to Mr. Piper in the amount of $82,500 if Mr. Piper remains continuously employed by the Company through April 30, 2019.

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 2019.

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 2019.

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 2019.

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 2019.

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 2019.

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 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).
10.11†	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).
10.12†	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).
10.13†	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).
10.14†	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).
10.15†	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).
10.16*	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).
10.17	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).
10.18*	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).
10.19*	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).
10.20*	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).
10.21	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).
10.22†	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).

Exhibit No.	Description
10.23*	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).
10.24*	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).
10.25	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 June 30, 2018 and incorporated herein by reference).
10.26	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).
10.27	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).
10.28	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).
10.29	Equity Distribution Agreement, dated May 15, 2018, by and between Aevi Genomic Medicine, Inc. and JMP Securities LLC (previously filed as Exhibit 1.1 to the Company's Current Report on Form 8-K filed on May 15, 2018 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).
21.1	Subsidiaries of the Company (filed herewith).
23.1	Consent of Ernst & Young LLP US (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).

Exhibit No.	Description
	† 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 29, 2019

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 29, 2019
/s/ Brian D. Piper Brian D. Piper	Chief Financial Officer (Principal Financial Officer & Principal Accounting Officer)	March 29, 2019
/s/ Sol J. Barer Sol J. Barer	Chairman of the Board of Directors	March 29, 2019
/s/ Eugene A. Bauer Eugene A. Bauer	Director	March 29, 2019
/s/ Alastair Clemow Alastair Clemow	Director	March 29, 2019
/s/ Barbara Duncan Barbara Duncan	Director	March 29, 2019
/s/ Joseph J. Grano, Jr. Joseph J. Grano, Jr.	Director	March 29, 2019
/s/ Matthew D. Bayley Matthew D. Bayley	Director	March 29, 2019

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

Aevi Genomic Medicine Europe BVBA/SPRL, a company organized under the laws of Belgium

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. 333-224929, 333-221950, 333-209737, 333-182740 and 333-208586) and in the Registration Statements on Form S-8 (File Nos. 333-226134, 333-219788, 333-210737, 333-182992, 333-188709, 333-191733 and 333-195165) of Aevi Genomic Medicine, Inc. of our report dated March 29, 2019, 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, 2018.

/s/ Ernst & Young LLP
Philadelphia, Pennsylvania
March 29, 2019

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 29, 2019

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 29, 2019

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, 2018 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 29, 2019

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

Date: March 29, 2019

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