

VistaGen Therapeutics, Inc.
Form S-1/A
December 07, 2017

As filed with the Securities and Exchange Commission on December 7, 2017

Registration No. 333-221009

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

FORM S-1
(Amendment No. 1)

REGISTRATION STATEMENT
UNDER
THE SECURITIES ACT OF 1933

VISTAGEN THERAPEUTICS, INC.
(Exact name of registrant as specified in its charter)

Nevada	3841	20-5093315
(State or other jurisdiction of incorporation or organization)	(Primary Standard Industrial Classification Code Number)	(I.R.S. Employer Identification Number)

VistaGen Therapeutics, Inc.
343 Allerton Avenue
South San Francisco, CA 94080
(650) 577-3600
(Address, including zip code, and telephone number, including area code, of registrant's principal executive offices)

Shawn K. Singh
Chief Executive Officer
VistaGen Therapeutics, Inc.
343 Allerton Avenue
South San Francisco, CA 94080
(650) 577-3600
(Name, address, including zip code, and telephone number, including area code, of agent for service)

Copies of all communications to:

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Approximate date of commencement of proposed sale to the public: As soon as practicable after this registration statement becomes effective.

If any of the securities being registered on this Form are to be offered on a delayed or continuous basis pursuant to Rule 415 under the Securities Act, check the following box. ☐

If this Form is filed to register additional securities for an offering pursuant to Rule 462(b) under the Securities Act, please check the following box and list the Securities Act registration statement number of the earlier effective registration statement for the same offering. ☐

If this Form is a post-effective amendment filed pursuant to Rule 462(c) under the Securities Act, check the following box and list the Securities Act registration statement number of the earlier effective registration statement for the same offering. ☐

If this Form is a post-effective amendment filed pursuant to Rule 462(d) under the Securities Act, check the following box and list the Securities Act registration statement number of the earlier effective registration statement for the same offering. ☐

Indicate by check mark whether the registrant is a large accelerated filer, an accelerated filer, a non-accelerated filer, or a smaller reporting company. See the definitions of “large accelerated filer,” “accelerated filer,” “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	☒
(Do not check if a smaller reporting company)		Emerging growth company	☐

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

CALCULATION OF REGISTRATION FEE

Title of each class of securities to be registered	Proposed maximum aggregate offering price (1)	Amount of registration fee
Common stock, \$0.001 par value (1)	\$ 15,000,000	\$ 1,867.50
Warrants to purchase shares of common stock (2)	-	-
Shares of common stock issuable upon exercise of warrants (1)(2)(3)	15,000,000	1,867.50
Total	\$ 30,000,000	\$ 3,735.00(4)

(1) Estimated solely for the purpose of calculating the amount of the registration fee pursuant to Rule 457(o) under the Securities Act of 1933, as amended.

(2) No fee required pursuant to Rule 457(g).

(3) Pursuant to Rule 416, there is also being registered such indeterminable additional securities as may be issued to prevent dilution as a result of stock splits, stock dividends or similar transactions.

(4) \$2,863.50 previously paid.

The Registrant hereby amends this Registration Statement on such date or dates as may be necessary to delay its effective date until the Registrant shall file a further amendment which specifically states that this Registration Statement shall thereafter become effective in accordance with Section 8(a) of the Securities Act of 1933, as amended, or until the Registration Statement shall become effective on such date as the Commission, acting pursuant to said Section 8(a), may determine.

The information in this preliminary prospectus is not complete and may be changed. These securities may not be sold until the registration statement filed with the Securities and Exchange Commission is effective. This preliminary prospectus is not an offer to sell these securities nor does it seek an offer to buy these securities in any jurisdiction where the offer or sale is not permitted.

PRELIMINARY PROSPECTUS SUBJECT TO COMPLETION DATED DECEMBER , 2017

8,333,333 Shares of Common Stock

Warrants to Purchase up to 8,333,333 Shares of Common Stock

We are offering 8,333,333 shares of our common stock and warrants to purchase up to 8,333,333 shares of our common stock. Each share of common stock will be accompanied by a warrant to purchase one share of common stock for an assumed combined public offering price of \$1.80 per share and related warrant (the last reported sale price of our common stock on the NASDAQ Capital Market on December 6, 2017). The common stock and warrants are immediately separable but can only be purchased together in this offering. The warrants are exercisable immediately and expire five years from the date of issuance, at an exercise price per share equal to 100% of the combined public offering price per share of common stock and related warrant in this offering. The shares of our common stock issuable from time to time upon exercise of the warrants are also being offered pursuant to this prospectus.

Our common stock is presently traded on the NASDAQ Capital Market under the symbol “VTGN.” On December 6, 2017, the last reported sale price of our common stock was \$1.80 per share. The actual combined public offering price will be as determined by us and the underwriters at the time of pricing, and may be at a discount to the current market price. Accordingly, the recent market price used throughout this prospectus may not be indicative of the actual combined public offering price. There is no established public trading market for the warrants being offered by this prospectus and we do not intend to have the warrants listed on a national securities exchange or any other recognized trading system in the future. Without an active market, the liquidity of any warrants sold by means of this prospectus will be limited.

Investing in our securities involves risks. See “Risk Factors” beginning on page 7 of this prospectus.

	Per Share and Related Warrant	Total
Combined public offering price	\$	\$
Underwriting discount (1)	\$	\$
Proceeds, before expenses, to us	\$	\$

(1) Please see the section titled “Underwriting” beginning on page 105 of this prospectus for additional information regarding the total compensation to be received by the underwriters.

Neither the Securities and Exchange Commission nor any state securities commission has approved or disapproved of these securities or determined if this prospectus is truthful or complete. Any representation to the contrary is a criminal offense.

The underwriters expect to deliver the shares of common stock and related warrants against payment on or about December , 2017.

Sole Book-Running Manager

Oppenheimer & Co.

Lead Manager

Chardan

The date of this prospectus is , 2017

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ABOUT THIS PROSPECTUS

Neither we nor the underwriters have authorized anyone to provide you with information that is different from that contained in this prospectus or in any free writing prospectus we may authorize to be delivered or made available to you. We take no responsibility for, and can provide no assurance as to the reliability of, any other information that others may give you. We and the underwriters are offering to sell shares of common stock and related warrants and seeking offers to buy shares of common stock and related warrants only in jurisdictions where offers and sales are permitted. The information contained in this prospectus is accurate only as of the date on the front of this prospectus, regardless of the time of delivery of this prospectus or any sale of shares of our common stock and related warrants.

Neither we nor the underwriters have done anything that would permit this offering or possession or distribution of this prospectus in any jurisdiction where action for that purpose is required, other than the U.S. You are required to inform yourself about, and to observe any restrictions relating to, this offering and the distribution of this prospectus.

This prospectus includes industry and market data that we obtained from industry publications, internal estimates and other third-party sources. These sources may include government and industry sources. Industry publications and surveys generally state that the information contained therein has been obtained from sources believed to be reliable. Although we believe the industry and market data to be reliable as of the date of this prospectus, this information could prove to be inaccurate. Industry and market data could be wrong because of the method by which sources obtained their data and because information cannot always be verified with complete certainty due to the limits on the availability and reliability of raw data, the voluntary nature of the data gathering process and other limitations and uncertainties. In addition, we do not know all of the assumptions regarding general economic conditions or growth that were used in preparing the forecasts from the sources relied upon or cited herein.

We further note that the representations, warranties and covenants made by us in any agreement that is filed as an exhibit to the registration statement of which this prospectus forms a part were made solely for the benefit of the parties to such agreement, including, in some cases, for the purpose of allocating risk among the parties to such agreements, and should not be deemed to be a representation, warranty or covenant to you. Moreover, such representations, warranties or covenants were accurate only as of the date when made. Accordingly, such representations, warranties and covenants should not be relied on as accurately representing the current state of our affairs.

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PROSPECTUS SUMMARY

This summary highlights information contained elsewhere in this prospectus and does not contain all of the information you should consider in making your investment decision. Before deciding to invest in our common stock, you should read this entire prospectus carefully, including the sections of this prospectus entitled “Risk Factors” and “Management’s Discussion and Analysis of Financial Condition and Results of Operations” and our consolidated financial statements and related notes contained elsewhere in this prospectus.

Unless the context otherwise requires, the words “VistaGen Therapeutics, Inc.” “VistaGen,” “we,” “the Company,” “us” and “our” refer to VistaGen Therapeutics, Inc., a Nevada corporation. “VistaStem Therapeutics, Inc.” and “VistaGen California” refers to our wholly owned subsidiary, VistaGen Therapeutics, Inc., a California corporation doing business as VistaStem Therapeutics, Inc.

Business Overview

We are a clinical-stage biopharmaceutical company focused on developing new generation medicines for depression and other central nervous system (CNS) disorders. Unless the context otherwise requires, the words “VistaGen Therapeutics, Inc.” “VistaGen,” “we,” “the Company,” “us” and “our” refer to VistaGen Therapeutics, Inc., a Nevada corporation. All references to future quarters and years in this prospectus refer to calendar quarters and calendar years, unless reference is made otherwise.

AV-101 is our oral CNS product candidate in Phase 2 clinical development in the United States, initially as a new generation adjunctive treatment for Major Depressive Disorder (MDD) in patients with an inadequate response to standard antidepressants approved by the U.S. Food and Drug Administration (FDA). AV-101’s mechanism of action (MOA) involves both NMDA (N-methyl-D-aspartate) and AMPA (alpha-amino-3-hydroxy-5-methyl-4-isoxazolepropionic acid) receptors in the brain responsible for fast excitatory synaptic activity throughout the CNS. AV-101’s MOA is fundamentally differentiated from all FDA-approved antidepressants, as well as all atypical antipsychotics,

such as aripiprazole, often used adjunctively to augment them. We believe AV-101 also has potential as a non-opioid treatment alternative for neuropathic pain, as well as several additional CNS indications where modulation of the NMDA receptors, activation of AMPA pathways and/or key active metabolites of AV-101 may achieve therapeutic benefit, including Parkinson's disease levodopa -induced dyskinesia (PD LID), epilepsy and Huntington's disease.

Clinical studies conducted at the U.S. National Institute of Mental Health (NIMH), part of the U.S. National Institutes of Health (NIH), by Dr. Carlos Zarate, Jr., Chief of the NIMH's Experimental Therapeutics & Pathophysiology Branch and its Section on Neurobiology and Treatment of Mood and Anxiety Disorders, have focused on the antidepressant effects of ketamine hydrochloride injection (ketamine), an ion-channel blocking NMDA receptor antagonist approved by the FDA as an anesthetic, in MDD patients with inadequate responses to multiple standard antidepressants. These NIMH studies, as well as clinical research at Yale University and other academic institutions, have demonstrated ketamine's robust antidepressant effects in treatment-resistant MDD patients within twenty-four hours of a single sub-anesthetic dose administered by intravenous (IV) injection.

We believe AV-101 may have potential to deliver ketamine-like antidepressant effects, without the need for IV administration and ketamine's psychological and other negative side effects. As published in the October 2015 issue of the peer-reviewed, Journal of Pharmacology and Experimental Therapeutics, in an article titled, The prodrug 4-chlorokynurenine causes ketamine-like antidepressant effects, but not side effects, by NMDA/glycineB-site inhibition, using well-established preclinical models of depression, AV-101 was shown to induce fast-acting, dose-dependent, persistent and statistically significant antidepressant-like responses following a single treatment. These responses were equivalent to those seen with a single sub-anesthetic control dose of ketamine. In addition, these studies confirmed that the fast-acting antidepressant effects of AV-101 were mediated through both inhibiting the GlyB site of the NMDA receptor and activating the AMPA receptor pathway in the brain.

Pursuant to our Cooperative Research and Development Agreement (CRADA) with the NIMH, the NIMH is

currently funding, and Dr. Zarate, as Principal Investigator, and his team are currently conducting, a small Phase 2 clinical study of AV-101 as a monotherapy in subjects with treatment-resistant MDD (the NIMH AV-101 MDD Phase 2 Monotherapy Study). In October 2017, we received FDA authorization to launch our 180-patient Phase 2 multi-center, multi-dose, double blind, placebo-controlled efficacy and safety study of AV-101 as a new generation adjunctive treatment of MDD in adult patients with an inadequate therapeutic response to standard, FDA-approved antidepressants (the AV-101 MDD Phase 2 Adjunctive Treatment Study). Subject to completion of this offering, we intend to launch the AV-101 MDD Phase 2 Adjunctive Treatment Study in the first quarter of 2018 with Dr. Maurizio Fava, Professor of Psychiatry at Harvard Medical School and Director, Division of Clinical Research, Massachusetts General Hospital (MGH) Research Institute, as the Principal Investigator. Dr. Fava was the co-Principal Investigator with Dr. A. John Rush of the STAR*D study, the largest clinical trial conducted in depression to date, whose findings were published in journals such as the New England Journal of Medicine (NEJM) and the Journal of the American Medical Association (JAMA). We expect to complete this study by the end of 2018, with top line results available in the first quarter of 2019.

VistaStem Therapeutics (VistaStem) is our wholly owned subsidiary focused on applying human pluripotent stem cell (hPSC) technology to discover, rescue, develop and commercialize (i) proprietary new chemical entities (NCEs) for CNS and other diseases and (ii) regenerative medicine (RM) involving hPSC-derived blood, cartilage, heart and liver cells. Our internal drug rescue programs are designed to utilize CardioSafe 3D, our customized cardiac bioassay system, to develop small molecule NCEs for our pipeline. To advance potential RM applications of our cardiac stem cell technology, in December 2016, we exclusively sublicensed to BlueRock Therapeutics LP, a next generation RM company established by Bayer AG and Versant Ventures (BlueRock Therapeutics), rights to certain proprietary technologies relating to the production of cardiac stem cells for the treatment of heart disease (the BlueRock Agreement). In a manner similar to our exclusive sublicense agreement with BlueRock

Therapeutics, we may pursue additional RM collaborations or licensing transactions involving blood, cartilage, and/or liver cells derived from hPSCs for (A) cell-based therapy, (B) cell repair therapy, and/or (C) tissue engineering.

AV-101 and Major Depressive Disorder

Background

The World Health Organization (WHO) estimates that 300 million people worldwide are affected by depression. According to the NIH, major depression is one of the most common mental disorders in the U.S. The NIMH reports that, in 2014, approximately 16 million adults in the U.S. had at least one major depressive episode in the past year. According to the U.S. Centers for Disease Control and Prevention (CDC) one in 10 Americans over the age of 12 takes a standard, FDA-approved antidepressant.

Most standard antidepressants target neurotransmitter reuptake inhibition – either serotonin (antidepressants known as SSRIs) or serotonin/norepinephrine (antidepressants known as SNRIs). Even when

effective, these standard antidepressants take many weeks to achieve adequate therapeutic effects. Nearly two out of every three drug-treated depression patients do not obtain adequate therapeutic benefit from initial treatment with a standard antidepressant. Even after treatment with many different standard antidepressants, nearly one out of every three drug-treated depression patients still do not achieve adequate therapeutic benefits from their antidepressant medication. Such patients with an inadequate response to standard antidepressants often seek to augment their treatment regimen by adding an atypical antipsychotic (drugs such as aripiprazole), despite only modest potential therapeutic benefit and the significant risk of additional side effects.

All standard antidepressants have risks of side effects, including, among others, anxiety, metabolic syndrome, sleep disturbance and sexual dysfunction. Adjunctive use of atypical antipsychotics to augment inadequately performing standard antidepressants may increase the risk of significant side effects, including, tardive dyskinesia, substantial weight gain, diabetes and

heart disease, while offering only a modest potential increase in therapeutic benefit.

AV-101

AV-101 is our oral CNS product candidate in Phase 2 development in the United States, initially focused as a new generation antidepressant for the adjunctive treatment of MDD patients with an inadequate therapeutic response to standard, FDA-approved antidepressants. As published in the October 2015 issue of the peer-reviewed, Journal of Pharmacology and Experimental Therapeutics, in an article titled, “The prodrug 4-chlorokynurenine causes ketamine-like antidepressant effects, but not side effects, by NMDA/glycineB-site inhibition,” using well-established preclinical models of depression, AV-101 was shown to induce fast-acting, dose-dependent, persistent and statistically significant ketamine-like antidepressant effects following a single treatment, responses equivalent to those seen with a single sub-anesthetic control dose of ketamine, but without the negative side effects seen with ketamine. In addition,

these studies confirmed that the antidepressant effects of AV-101 were mediated through both inhibition of the GlyB site of NMDA receptors and activation of the AMPA receptor pathway in the brain, a key final common pathway feature of certain new generation antidepressants such as ketamine and AV-101, each with a MOA that is fundamentally different from all standard antidepressants and atypical antipsychotics used adjunctively to augment them.

We have completed two NIH-funded, randomized, double blind, placebo-controlled AV-101 Phase 1 safety studies. Currently, pursuant to our CRADA with the NIMH and Dr. Carlos Zarate, Jr., the NIMH is currently funding, and Dr. Zarate, as Principal Investigator, and his team are currently conducting, the NIMH AV-101 MDD Phase 2 Monotherapy Study. Although we are not involved in conducting this study, we currently anticipate that the NIMH will complete the NIMH AV-101 MDD Phase 2 Monotherapy Study during the first half of 2018.

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We are preparing to begin the AV-101 MDD Phase 2 Adjunctive Treatment Study, which will test the safety, efficacy and tolerability of AV-101 as an adjunctive treatment of MDD in adult patients with an inadequate therapeutic response to standard, FDA-approved antidepressants. Subject to completion of this offering, we intend to launch the AV-101 MDD Phase 2 Adjunctive Treatment Study in the first quarter of 2018. In connection with our preparation for this study, as well as potential Phase 3 development and commercialization of AV-101, we, together with our contract manufacturing organization (CMO), developed a novel process for the production of AV-101 drug substance. We believe our new proprietary

production process will significantly improve AV-101 manufacturing efficiency, thereby reducing the current and future cost of manufacturing AV-101 drug substance and improving the yield of AV-101 drug substance manufactured.

While developing our new manufacturing process, our CMO produced a batch of AV-101 drug substance that contained certain impurities not within the limits set out by the International Conference on Harmonisation of Technical Requirements for Registration of Pharmaceuticals for Human Use (the ICH Guidelines). The FDA routinely utilizes the ICH Guidelines as an industry standard for development stage programs such as our AV-101 Phase 2 program. Consequently, the FDA placed a clinical hold on the launch of the AV-101 MDD Phase 2 Adjunctive

Treatment Study until we either (a) further improved our AV-101 manufacturing process to remove the impurities or reduce the impurities below applicable limits in the ICH Guidelines, or (b) conducted a bridging toxicology study to qualify the impurities as safe for clinical use. In response to the FDA's requests, we did both. We further improved our AV-101 manufacturing process, produced a new batch of AV-101 drug substance using the improved process, and now have analytical results from that batch showing that the impurities were reduced to a level below the limits of the ICH Guidelines. In addition, we conducted a bridging toxicology study, the results of which confirmed that the impurities were safe for clinical use.

The FDA also requested additional

contraceptive protection in the upcoming clinical study until we complete preclinical reproductive toxicology studies that are routinely conducted later in stages of clinical development.

Although previous toxicology studies for AV-101 do not suggest any reproductive organ involvement, and we have confirmed with the FDA that our proposed study is a Phase 2 study, we have

implemented additional contraceptive measures in the revised protocol for the AV-101 MDD Phase 2

Adjunctive Treatment Study that will remain in effect until standard reproductive toxicology studies are completed in the ordinary course prior to commencement of Phase 3

development. We have confirmed with the FDA that our recently implemented contraceptive measures are appropriate for the current stage of

development of
AV-101.

In September 2017, we requested, and were granted, a pre-IND Type A meeting with the FDA's Division of Psychiatry to discuss certain matters pertaining to our AV-101 development program and the AV-101 MDD Phase 2 Adjunctive Treatment Study. Subsequent to our Type A Meeting with the FDA, we submitted the supplemental data from our new manufacturing process, the bridging toxicology report and our study protocol, revised to address the FDA's comments. These documents adequately addressed all concerns raised by the FDA to date. Consequently, on October 26, 2017, the FDA authorized us to proceed, under our Investigational New Drug (IND) application, with the AV-101 MDD Phase 2 Adjunctive Treatment Study.

We believe preclinical studies and Phase 1 safety studies support our hypothesis that AV-101 also has potential as a non-opioid treatment alternative for neuropathic pain, as well as several additional CNS indications where modulation of the NMDA receptors, activation of AMPA pathways and/or key active metabolites of AV-101 may achieve therapeutic benefit, including PD LID, epilepsy, and Huntington's disease. We are beginning to plan additional Phase 2 clinical studies to further evaluate the therapeutic potential of AV-101 beyond MDD.

CardioSafe 3D™;
NCE Drug Rescue
and Regenerative
Medicine

VistaStem
Therapeutics is our
wholly owned
subsidiary focused
on applying hPSC
technology to
discover, rescue,
develop and
commercialize
proprietary small
molecule NCEs for

CNS and other diseases, as well as potential cellular therapies involving stem cell-derived blood, cartilage, heart and liver cells.

CardioSafe 3D™ is our customized in vitro cardiac bioassay system capable of predicting potential human heart toxicity of small molecule NCEs in vitro, long before they are ever tested in animal and human studies. Potential commercial applications of our stem cell technology platform involve using CardioSafe 3D internally for NCE drug discovery and drug rescue to expand our proprietary drug candidate pipeline.

Drug rescue involves leveraging substantial prior research and development investments by pharmaceutical companies and others related to public domain NCE programs terminated before FDA approval due to heart toxicity risks and RM and cellular therapies.

To advance potential RM applications of our cardiac stem cell technology, in December 2016, we exclusively sublicensed to BlueRock Therapeutics LP, a next generation regenerative medicine company established by Bayer AG and Versant Ventures, rights to certain proprietary technologies relating to the production of cardiac stem cells for the treatment of heart disease. In a manner similar to the BlueRock Agreement, we may also pursue additional potential RM applications using blood, cartilage, and/or liver cells derived from hPSCs for (A) cell-based therapy (injection of stem cell-derived mature organ-specific cells obtained through directed differentiation), (B) cell repair therapy (induction of regeneration by biologically active molecules administered alone or produced by infused genetically

engineered cells),
or (C) tissue
engineering
(transplantation
of in vitro grown
complex tissues)
using
hPSC-derived
blood, bone,
cartilage, and/or
liver cells.

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Risk Factors

Our business is subject to substantial risk. Please carefully consider the section titled “Risk Factors” on page 7 of this prospectus for a discussion of the factors you should carefully consider before deciding to purchase the securities offered by this prospectus. These risks include, among others:

we are a development stage biopharmaceutical company with no current revenues or approved products, and limited experience developing new drug, biological and/or regenerative medicine candidates, which makes it difficult to assess our future viability;

we depend heavily on the success of AV-101, and we cannot be certain that we will be able to obtain regulatory approval for, or successfully commercialize, AV-101, or any product candidate;

failures or delays in the commencement or completion of our planned clinical trials could delay, prevent or limit our ability to generate revenue and continue our business;

we face significant competition, and if we are unable to compete effectively, we may not be able to achieve or maintain significant market penetration or improve our results of operations;

some of our programs have been partially supported by government grants, which may not be available to us in the future;

if we are unable to adequately protect our proprietary technology, or obtain and maintain issued patents that are sufficient to protect our product candidates, others could compete against us more directly, which would have a material adverse impact on our business, results of operations, financial condition and prospects; and

we have incurred significant net losses since inception and we will continue to incur substantial operating losses for the foreseeable future.

Additional risks and uncertainties not presently known to us or that we currently deem immaterial may also impair our business operations. You should be able to bear a complete loss of your investment.

Corporate information

VistaGen Therapeutics, Inc., a Nevada corporation, is the parent of VistaGen Therapeutics, Inc. (dba VistaStem Therapeutics, Inc.), a wholly-owned California corporation founded in 1998. Our principal executive offices are located at 343 Allerton Avenue, South San Francisco, California 94080, and our telephone number is (650) 577-3600. Our website address is www.vistagen.com. The information contained on our website is not part of this prospectus. We have included our website address as a factual reference and do not intend it to be an active link to our website.

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THE OFFERING

Issuer: VistaGen Therapeutics, Inc.

Common

stock 8,333,333 shares, assuming a combined public offering price of \$1.80 per share and related warrant (the last reported sale price of our common stock on the NASDAQ Capital Market on December 6, 2017).
offered
by
us

Common

stock 20,182,307 shares (assuming no exercise of the warrants offered hereby), assuming a combined public offering price of \$1.80 per share and related warrant (the last reported sale price of our common stock on the NASDAQ Capital Market on December 6, 2017).
immediately
after
this
offering

Warrants to purchase up to 8,333,333 shares of common stock. Each share of common stock sold in this offering will be accompanied by a warrant to purchase one share of our common stock, at an exercise price per share equal to 100% of the combined public offering price in this offering. The warrants will be immediately exercisable, and may be exercised for a period of five years following the date of issuance.

Use of We intend to use the net proceeds from this offering for research and development, primarily related to the AV-101 MDD Phase 2 Adjunctive Treatment Study, ordinary course working capital needs and other general corporate purposes. See “Use of Proceeds” for additional information regarding the intended use of proceeds from this offering.
proceeds
this offering.

Risks An investment in our securities involves a high degree of risk. See “Risk Factors” beginning on page 7 of this prospectus for a discussion of factors you should carefully consider before investing in our securities.

Listing Our common stock is listed on the NASDAQ Capital Market under the symbol “VTGN.” There is no established public trading market for the warrants being offered by this prospectus and we do not intend to have the warrants listed on a national securities exchange or any other recognized trading system in the future. Without an active market, the liquidity of any warrants sold by means of this prospectus will be limited.

The number of shares of common stock that will be outstanding after this offering is based on 11,848,974 shares outstanding as of December 6, 2017 and excludes, as of that date, the following:

- 3,279,871 shares of common stock issuable upon the exercise of outstanding stock options, having a weighted average exercise price of \$3.23 per share;
- 7,107,157 shares of common stock issuable upon the exercise of outstanding warrants, having a weighted average exercise price of \$4.70 per share;
- 750,000 shares of common stock issuable upon conversion of all outstanding shares of our Series A Preferred;
- 1,160,240 shares of common stock issuable upon conversion of all outstanding shares of our Series B Preferred and 663,460 shares of common stock reserved for issuance as payment of accrued dividends on outstanding

shares of Series B Preferred;
2,318,012 shares of common stock issuable upon conversion of all outstanding shares of our Series C Preferred;
6,012,162 shares of common stock reserved for future issuance in connection with future grants under our
Amended and Restated 2016 Stock Incentive Plan; and
shares of common stock issuable upon the exercise of the warrants offered hereby

Unless otherwise indicated, this prospectus reflects and assumes the following:

no exercise of options or warrants outstanding as of December 6, 2017.

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SUMMARY
CONSOLIDATED
FINANCIAL DATA

The following tables summarize our consolidated financial data. We have derived the summary consolidated statement of operations data for the years ended March 31, 2017 and 2016 from our audited consolidated financial statements included elsewhere in this prospectus. We have derived the summary consolidated statement of operations data for the six-months ended September 30, 2017 and 2016 and our balance sheet data as of September 30, 2017 from our unaudited interim consolidated financial statements included elsewhere in this prospectus. The unaudited interim consolidated financial statements have been prepared on the same basis as the audited consolidated financial statements and reflect, in our opinion, only adjustments of a

normal, recurring nature that are necessary for a fair statement of the unaudited interim consolidated financial statements. Our results for the six-months ended September 30, 2017 are not necessarily indicative of results to be expected for the full year or any other period. The following summary consolidated financial data should be read in conjunction with the section titled “Management’s Discussion and Analysis of Financial Condition and Results of Operations” and our consolidated financial statements and related notes included elsewhere in this prospectus.

Fiscal Year Ended March 31,		Six-Months Ended September 30,	
2017	2016	2017	2016

Consolidated Statement of Operations Data:

(in thousands, except share and per share amounts)

Sublicense revenue	\$ 1,250	\$ –	\$ –	\$ –
Operating expenses:				
Research and development	5,204	3,932	3,523	2,432
General and administrative	6,295	13,919	3,731	2,631
Total operating expenses	11,499	17,851	7,254	5,063
Loss from operations	(10,249)	(17,851)	(7,254)	(5,063)
Other expenses, net:				
Interest expense, net	(5)	(771)	(6)	(3)
Change in warrant liabilities	–	(1,895)	–	–

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Loss on early extinguishment of debt	—	(26,700)	—	—
Other expense	—	(2)	—	—
Loss before income taxes	(10,254)	(47,219)	(7,260)	(5,066)
Income taxes	(2)	(2)	(2)	(2)
Net loss	(10,256)	(47,221)	(7,262)	(5,068)
Accrued dividend on Series B Preferred Stock	(1,257)	(2,140)	(504)	(781)
Deemed dividend on Series B Preferred Units	(111)	(2,058)	—	(111)
Net loss attributable to common stockholders	\$ (11,624)	\$ (51,419)	\$ (7,766)	\$ (5,960)

Basic and diluted net loss attributable to common stockholders per common share	\$ (1.54)	\$ (29.08)	\$ (0.82)	\$ (0.91)
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Weighted average shares used in computing:

Basic and diluted net loss attributable to common stockholders per common share	7,531,642	1,767,957	9,465,459	6,577,769
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March 31, September 30, 2017

2017 Actual Pro Forma(1)

(in thousands)

Consolidated Balance Sheet Data:

Cash and cash equivalents	\$ 2,921	\$ 1,764	\$ 15,374
Total assets	\$ 3,712	\$ 2,618	\$ 16,228
Current notes payable	\$ 55	\$ 105	\$ 105
Working capital	\$ 2,010	\$ 443	\$ 14,053
Common stock, preferred stock, treasury stock and additional paid-in capital	\$ 142,615	\$ 147,589	\$ 161,199
Total stockholders' equity (deficit)	\$ 616	\$ (1,672)	\$ 11,938

(1) The pro forma column gives effect to the assumed sale and issuance by us of 8,333,333 shares of our common stock and related warrants to purchase up to 8,333,333 shares of

common
stock in this
offering,
assuming a
combined
public
offering price
of \$1.80 per
share and
related
warrant (the
last reported
sale price of
our common
stock on the
NASDAQ
Capital
Market on
December 6,
2017), after
deducting the
estimated
underwriting
discount and
estimated
offering
expenses
payable by
us assuming
no value is
attributed to
the warrants,
and the
warrants are
accounted for
and classified
as equity.

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RISK FACTORS

Investing in our securities involves a high degree of risk. You should consider carefully the risks and uncertainties described below, together with all of the other information in this prospectus, including our financial statements and related notes, before deciding whether to purchase securities in the offering. If any of the following risks are realized, our business, financial condition and results of operations could be materially and adversely affected.

Risks Related to Product Development, Regulatory Approval and Commercialization

We depend heavily on the success of AV-101. We cannot be certain that we will be able to obtain regulatory approval for, or successfully commercialize AV-101, or any product candidate.

We currently have no drug products for sale and may never be able to develop and commercialize marketable drug products. Our business depends heavily on the successful development, regulatory approval and commercialization of AV-101 for depression, including for MDD, and, potentially, various other diseases and disorders involving the CNS, as well as, but to a more limited extent, our ability to produce, develop and commercialize NCEs from our drug rescue programs. AV-101 will require substantial additional nonclinical and clinical testing and regulatory approval before it may be commercialized. It is unlikely to achieve regulatory approval, if at all, until at least 2021. Each drug rescue NCE will require substantial nonclinical development, all phases of clinical development, and regulatory approval before it may be commercialized. The nonclinical and clinical development of our product candidates are, and the manufacturing and marketing of our product candidates will be, subject to extensive and rigorous review and regulation by numerous government authorities in the United States and in other countries where we intend to test and, if approved, market any product candidate. Before obtaining regulatory approvals for the commercial sale of any product candidate, we must demonstrate through numerous nonclinical and clinical studies that the product candidate is safe and effective for use in each target indication. Drug development is a long, expensive and uncertain process, and delay or failure can occur at any stage of any of our nonclinical or clinical studies. This process takes many years and may also include post-marketing studies and surveillance obligations, which would require the expenditure of substantial resources beyond the proceeds we have raised to date. Of the large number of drugs in development in the United States, only a small percentage will successfully complete the FDA regulatory approval process and will be commercialized. Accordingly, we cannot assure you that AV-101, any drug rescue NCE, or any other future product candidate will be successfully developed or commercialized.

We are not permitted to market our product candidates in the United States until we receive approval of a New Drug Application (NDA) from the FDA, or in any foreign countries until we receive the requisite approval from such countries. We expect the FDA to require us to complete the planned AV-101 MDD Phase 2 Adjunctive Treatment Study and at least two pivotal Phase 3 clinical trials in order to submit an NDA for AV-101 as an adjunctive treatment for MDD patients with an inadequate response to standard, FDA-approved antidepressants. Also, we anticipate that the FDA will require that we conduct additional toxicology studies, additional nonclinical and certain small clinical studies before submitting an NDA for AV-101. The results of all of these studies are not known until after the studies are concluded.

Obtaining FDA approval of an NDA is a complex, lengthy, expensive and uncertain process, and the FDA may delay, limit or deny approval of AV-101 or any other product candidate we may seek to develop for many reasons, including, among others:

if we submit an NDA and it is reviewed by an advisory committee, the FDA may have difficulties scheduling an advisory committee meeting in a timely manner or the advisory committee may recommend against approval of our

application or may recommend that the FDA require, as a condition of approval, additional non-clinical or clinical studies, limitations on approved labeling or distribution and use restrictions;

the FDA may require development of a Risk Evaluation and Mitigation Strategy (REMS) as a condition of approval or post-approval;

the FDA or the applicable foreign regulatory agency may determine that the manufacturing processes or facilities of third-party contract manufacturers with which we contract do not conform to applicable requirements, including current Good Manufacturing Practices (cGMPs); or

the FDA or applicable foreign regulatory agency may change its approval policies or adopt new regulations.

Any of these factors, many of which are beyond our control, could jeopardize our ability to obtain regulatory approval for and successfully commercialize AV-101 or any other product candidate we may develop, including drug rescue NCEs. Any such setback in our pursuit of regulatory approval for any product candidate would have a material adverse effect on our business and prospects.

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We have applied for a Fast Track designation from the FDA for AV-101, initially for adjunctive treatment of MDD patients with an inadequate response to standard antidepressants. Even if the FDA approves Fast Track designation for AV-101 for this indication, it may not actually lead to a faster development or regulatory review or approval process.

The Fast Track designation is a program offered by the FDA pursuant to certain mandates under the FDA Modernization Act of 1997, designed to facilitate drug development and to expedite the review of new drugs that are intended to treat serious or life threatening conditions. Compounds selected must demonstrate the potential to address unmet medical needs. The Fast Track designation allows for close and frequent interaction with the FDA. A designated Fast Track drug may also be considered for priority review with a shortened review time, rolling submission, and accelerated approval if applicable. The designation does not, however, guarantee approval or expedited approval of any application for the product.

In October 2017 we applied for FDA Fast Track designation for AV-101, initially for adjunctive treatment of MDD patients with an inadequate response to standard antidepressants, and we may do so for other CNS indications, as well as for other product candidates. The FDA has broad discretion whether or not to grant a Fast Track designation, and even if we believe AV-101 and other product candidates are eligible for this designation, we cannot be sure that the review or approval will compare to conventional FDA procedures. Even if granted, the FDA may withdraw Fast Track designation if it believes that the designation is no longer supported by data from our clinical development programs.

The number of patients suffering from MDD has not been established with precision. If the actual number of patients with MDD is smaller than we anticipate, we or our collaborators may encounter difficulties in enrolling patients in AV-101 clinical trials, including the NIMH AV-101 MDD Phase 2 Monotherapy Study and our planned AV-101 MDD Phase 2 Adjunctive Treatment Study, thereby delaying completion such studies or preventing additional clinical development. Further, if AV-101 is approved for adjunctive treatment of MDD patients with an inadequate response to standard antidepressants, and the market for this indication is smaller than we anticipate, our ability to achieve profitability could be limited.

Results of earlier clinical trials may not be predictive of the results of later-stage clinical trials.

The results of preclinical studies and early clinical trials of AV-101 and/or other product candidates, including positive results, may not be predictive of the results of later-stage clinical trials. AV-101 or other product candidates in later stages of clinical trials may fail to show the desired safety and efficacy results despite having progressed through preclinical studies and initial clinical trials. Many companies in the biopharmaceutical industry have suffered significant setbacks in advanced clinical trials due to adverse safety profiles or lack of efficacy, notwithstanding promising results in earlier studies. Similarly, our future clinical trial results may not be successful for these or other reasons.

Moreover, preclinical and clinical data are often susceptible to varying interpretations and analyses, and many companies that believed their product candidates performed satisfactorily in preclinical studies and clinical trials nonetheless failed to obtain FDA approval. We have not yet completed a Phase 2 clinical trial for AV-101, and if the NIMH AV-101 MDD Phase 2 Monotherapy Study and/or our AV-101 MDD Phase 2 Adjunctive Treatment Study fail(s) to produce positive results, the development timeline and regulatory approval and commercialization prospects for AV-101 and, correspondingly, our business and financial prospects, could be materially adversely affected.

This drug candidate development risk is heightened by any changes in planned timing or nature of clinical trials compared to completed clinical trials. As product candidates are developed through preclinical to early and late stage clinical trials towards approval and commercialization, it is customary that various aspects of the development program, such as manufacturing and methods of administration, are altered along the way in an effort to optimize

processes and results. While these types of changes are common and are intended to optimize the product candidates for later stage clinical trials, approval and commercialization, such changes do carry the risk that they will not achieve these intended objectives.

For example, the results of planned clinical trials may be adversely affected if we or our collaborator seek to optimize and scale-up production of a product candidate. In such case, we will need to demonstrate comparability between the newly manufactured drug substance and/or drug product relative to the previously manufactured drug substance and/or drug product. Demonstrating comparability may cause us to incur additional costs or delay initiation or completion of our clinical trials, including the need to initiate a dose escalation study and, if unsuccessful, could require us to complete additional nonclinical or clinical studies of our product candidates.

If serious adverse events or other undesirable side effects are identified during the use of AV-101 in clinical trials, it may adversely affect our development of AV-101 for MDD and other CNS indications.

AV-101 as a monotherapy is currently being tested by the NIMH in the NIMH AV-101 MDD Phase 2 Monotherapy Study and may be subjected to testing in the future for other CNS indications in additional investigator sponsored clinical trials. If serious adverse events or other undesirable side effects, or unexpected characteristics of AV-101 are observed in investigator-sponsored clinical trials of AV-101 or our clinical trials of AV-101, it may adversely affect or delay our clinical development of AV-101, and the occurrence of these events would have a material adverse effect on our business and financial prospects.

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Failures or delays in the commencement or completion of our planned clinical trials and nonclinical studies of our product candidates could result in increased costs to us and could delay, prevent or limit our ability to generate revenue and continue our business.

Under our CRADA with the NIMH, the NIMH is conducting and funding the NIMH AV-101 MDD Phase 2 Monotherapy Study. We will need to complete the planned AV-101 MDD Phase 2 Adjunctive Treatment Study, at least two additional large Phase 3 pivotal clinical trials, additional toxicology and other nonclinical studies and certain smaller clinical studies prior to the submission of an NDA for AV-101 as a new generation adjunctive treatment for MDD. Successful completion of our nonclinical and clinical trials is a prerequisite to submitting an NDA to the FDA and, consequently, the ultimate approval required before commercial marketing of AV-101 for MDD and any other product candidates we may develop. Except as disclosed herein, we do not know whether the NIMH AV-101 MDD Phase 2 Monotherapy Study, our AV-101 MDD Phase 2 Adjunctive Treatment Study or any of our future-planned nonclinical and clinical trials will be completed on schedule, if at all, as the commencement and completion of nonclinical and clinical trials can be delayed or prevented for a number of reasons, including, among others:

the FDA may deny permission to proceed with our planned clinical trials or any other clinical trials we may initiate, or may place a planned or ongoing clinical trial on hold;

delays in filing or receiving approvals of additional INDs that may be required;

negative results from our ongoing nonclinical studies;

delays in reaching or failing to reach agreement on acceptable terms with prospective CROs, investigators and clinical trial sites, the terms of which can be subject to extensive negotiation and may vary significantly among different CROs, investigators and clinical trial sites;

delays in the manufacturing of, or insufficient supply of, AV-101 or other product candidates necessary to conduct nonclinical or clinical trials, including delays in the manufacturing of sufficient supply or finished drug product resulting from our new manufacturing process for AV-101;

inability to manufacture or obtain clinical supplies of a product candidate meeting required quality standards;

difficulties obtaining Institutional Review Board (IRB) approval to conduct a clinical trial at a prospective clinical site or sites;

challenges in recruiting and enrolling patients to participate in clinical trials, including the proximity of patients to clinical trial sites;

eligibility criteria for a clinical trial, the nature of a clinical trial protocol, the availability of approved effective treatments for the relevant disease and competition from other clinical trial programs for similar indications;

severe or unexpected drug-related side effects experienced by patients in a clinical trial;

delays in validating any endpoints utilized in a clinical trial;

the FDA may disagree with our clinical trial design and our interpretation of data from prior nonclinical studies or clinical trials, or may change the requirements for approval even after it has reviewed and commented on the design for our clinical trials;

reports from nonclinical or clinical testing of other CNS indications or therapies that raise safety or efficacy concerns; and

difficulties retaining patients who have enrolled in a clinical trial but may be prone to withdraw due to rigors of the clinical trial, lack of efficacy, side effects, personal issues or loss of interest.

Clinical trials may also be delayed or terminated prior to completion as a result of ambiguous or negative interim results. In addition, a clinical trial may be suspended or terminated by us, the FDA, the IRBs at the sites where the IRBs are overseeing a clinical trial, a data and safety monitoring board (DSMB), overseeing the clinical trial at issue or other regulatory authorities due to a number of factors, including, among others:

failure to conduct the clinical trial in accordance with regulatory requirements or approved clinical protocols;

inspection of the clinical trial operations or trial sites by the FDA or other regulatory authorities that reveals deficiencies or violations that require us to undertake corrective action, including the imposition of a clinical hold;

unforeseen safety issues, including any that could be identified in our ongoing nonclinical carcinogenicity studies, adverse side effects or lack of effectiveness;

changes in government regulations or administrative actions;

problems with clinical supply materials that may lead to regulatory actions; and

lack of adequate funding to continue nonclinical or clinical studies.

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Changes in regulatory requirements, FDA guidance or unanticipated events during our nonclinical studies and clinical trials of AV-101 or other product candidates may occur, which may result in changes to nonclinical studies and clinical trial protocols or additional nonclinical studies and clinical trial requirements, which could result in increased costs to us and could delay our development timeline.

Changes in regulatory requirements, FDA guidance or unanticipated events during our nonclinical studies and clinical trials may force us to amend nonclinical studies and clinical trial protocols or the FDA may impose additional nonclinical studies and clinical trial requirements. Amendments or changes to our clinical trial protocols would require resubmission to the FDA and IRBs for review and approval, which may adversely impact the cost, timing or successful completion of clinical trials. Similarly, amendments to our nonclinical studies may adversely impact the cost, timing, or successful completion of those non-clinical studies. If we experience delays completing, or if we terminate, any of our nonclinical studies or clinical trials, or if we are required to conduct additional nonclinical studies or clinical trials, the commercial prospects for AV-101 or other product candidates may be harmed and our ability to generate product revenue will be delayed.

We rely, and expect that we will continue to rely, on third parties to conduct non-clinical and clinical trials of AV-101 and any other product candidates. If these third parties do not successfully carry out their contractual duties or meet expected deadlines, completion of non-clinical and clinical trials and development of AV-101 and other product candidates may be delayed and we may not be able to obtain regulatory approval for or commercialize AV-101 or other product candidates and our business could be substantially harmed.

We do not have the internal staff resources to independently conduct nonclinical and clinical trials completely on our own. We rely on our network of strategic relationships with various medical institutions, nonclinical and clinical investigators, contract laboratories and other third parties, such as contract research and development organizations (CROs), to conduct nonclinical and clinical trials of our product candidates. We enter into agreements with third-party CROs to provide monitors for and to manage data for our clinical trials, as well as provide other services necessary to prepare for, conduct and complete clinical trials. We rely heavily on these and other third-parties for execution of nonclinical and clinical trials for our product candidates and control only certain aspects of their activities. As a result, we have less direct control over the conduct, timing and completion of these nonclinical and clinical trials and the management of data developed through nonclinical and clinical trials than would be the case if we were relying entirely upon our own staff. Communicating with outside parties can also be challenging, potentially leading to mistakes as well as difficulties in coordinating activities. Outside parties may:

have staffing difficulties and/or undertake obligations beyond their anticipated capabilities and resources;

fail to comply with contractual obligations;

experience regulatory compliance issues;

undergo changes in priorities or become financially distressed; or

form relationships with other entities, some of which may be our competitors.

These factors may materially adversely affect the willingness or ability of third parties to conduct our nonclinical and clinical trials and may subject us to unexpected cost increases that are beyond our control. Nevertheless, we are responsible for ensuring that each of our nonclinical studies and clinical trials is conducted in accordance with the applicable protocol, legal, regulatory and scientific requirements and standards, and our reliance on CROs or the NIH does not relieve us of our regulatory responsibilities. We and our CROs and the NIMH are required to comply with regulations and guidelines, including current cGCPs for conducting, monitoring, recording and reporting the results of clinical trials to ensure that the data and results are scientifically credible and accurate, and that the trial patients are adequately informed of the potential risks of participating in clinical trials. These regulations are enforced by the FDA, the Competent Authorities of the Member States of the European Economic Area and comparable foreign regulatory authorities for any products in clinical development. The FDA enforces cGCP regulations through periodic inspections of clinical trial sponsors, principal investigators and trial sites. If we or any of our CROs fail to comply with applicable cGCPs, the clinical data generated in our clinical trials may be deemed unreliable and the FDA or comparable foreign regulatory authorities may require us to perform additional clinical trials before approving our marketing applications. We cannot assure you that, upon inspection, the FDA will determine that any of our clinical trials comply with cGCPs. In addition, our clinical trials must be conducted with product candidates produced under cGMPs regulations and will require a large number of test patients. Our failure or the failure of our CROs to comply with these regulations may require us to repeat clinical trials, which would delay the regulatory approval process and could also subject us to enforcement action up to and including civil and criminal penalties.

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Although we design our clinical trials for our product candidates, we plan to have CROs, and in the case of the NIMH AV-101 MDD Phase 2 Monotherapy Study, the NIMH, conduct the AV-101 Phase 2 and Phase 3 clinical trials. As a result, many important aspects of our drug development programs are outside of our direct control. In addition, the CROs or the NIMH, as the case may be, may not perform all of their obligations under arrangements with us or in compliance with regulatory requirements, but we remain responsible and are subject to enforcement action that may include civil penalties up to and including criminal prosecution for any violations of FDA laws and regulations during the conduct of our clinical trials. If the NIMH or CROs do not perform clinical trials in a satisfactory manner, breach their obligations to us or fail to comply with regulatory requirements, the development and commercialization of AV-101 and other product candidates may be delayed or our development program materially and irreversibly harmed. We cannot control the amount and timing of resources these CROs or the NIMH devote to our program or our clinical products. If we are unable to rely on non-clinical and clinical data collected by our CROs or the NIMH, we could be required to repeat, extend the duration of, or increase the size of our clinical trials and this could significantly delay commercialization and require significantly greater expenditures.

If any of our relationships with these third-party CROs or the NIMH terminate, we may not be able to enter into arrangements with alternative CROs or collaborators. If CROs or the NIMH do not successfully carry out their contractual duties or obligations or meet expected deadlines, if they need to be replaced or if the quality or accuracy of the clinical data they obtain is compromised due to the failure to adhere to our clinical protocols, regulatory requirements or for other reasons, any clinical trials that such CROs or the NIMH are associated with may be extended, delayed or terminated, and we may not be able to obtain regulatory approval for or successfully develop and commercialize our product candidates. As a result, we believe that our financial results and the commercial prospects for our product candidates in the subject indication would be harmed, our costs would increase and our ability to generate revenue would be delayed.

We rely completely on third-parties to manufacture and prepare supplies of AV-101 for all nonclinical and clinical studies of AV-101, and we intend to continue to rely on third parties to produce all nonclinical, clinical and commercial supplies of AV-101 in the future.

We do not currently have, nor do we plan to acquire or develop, the necessary infrastructure internal resources or technical capabilities to manufacture and prepare supplies of AV-101, or any future product candidates, for use in nonclinical and clinical studies, and we lack the internal resources and the capability to manufacture any product candidate on a clinical or commercial scale. As a result, we rely completely on third party contract manufacturing organizations (CMOs) to manufacture AV-101 active pharmaceutical ingredient (API) and prepare AV-101 final drug product. The facilities used by our CMOs to manufacture AV-101 API and final drug product must complete a pre-approval inspection by the FDA and other comparable foreign regulatory agencies to assess compliance with applicable requirements, including cGMPs, after we submit our AV-101 INDs, NDAs or relevant foreign regulatory submission equivalent to the applicable regulatory agency.

We do not directly control either the supply or quality of materials used in the manufacturing and preparation of AV-101 or the AV-101 manufacturing process, and we are completely dependent on our CMOs to comply with all cGMPs for manufacture of both AV-101 API and AV-101 finished drug product. If our CMOs cannot secure adequate supplies of suitable raw materials or successfully manufacture AV-101 API that conforms to our specifications and the strict regulatory requirements of the FDA or applicable foreign regulatory agencies, production of sufficient supplies of AV-101 API and finished drug product may be delayed and our CMOs may not be able to secure and/or maintain regulatory approval for their manufacturing facilities, or the FDA may take other actions, including the imposition of a clinical hold. In addition, we have no direct control over our CMOs' ability to maintain adequate quality control, quality assurance and qualified personnel. All of our CMOs are engaged with other companies to supply and/or manufacture materials or products for such other companies, which exposes our CMOs to regulatory

risks for the production of such materials and products. As a result, failure to satisfy the regulatory requirements for the production of those materials and products may affect the regulatory clearance of our CMO's facilities generally or affect the timing of manufacture of AV-101 for required or planned nonclinical and/or clinical studies of AV-101. If the FDA or an applicable foreign regulatory agency determines now or in the future that our CMOs' facilities are noncompliant, we may need to find alternative manufacturing facilities, which would adversely impact our ability to develop, obtain regulatory approval for or market AV-101. Our reliance on CMOs also exposes us to the possibility that they, or third parties with access to their facilities, will have access to and may appropriate our trade secrets or other proprietary information.

We do not yet have long-term AV-101 supply agreements in place with our CMOs and each batch of AV-101 is individually contracted under a separate supply agreement. If we engage new CMOs, such contractors must complete an inspection by the FDA and other applicable foreign regulatory agencies. We plan to continue to rely upon CMOs and, potentially, collaboration partners, to manufacture research, development and, if approved, commercial quantities of AV-101 and any other product candidates we may seek to develop in the future. Although we believe our current scale of manufacturing for AV-101 and current and projected supply of AV-101 API and finished drug product will be adequate to support our planned nonclinical and clinical studies of AV-101, no assurance can be given that unanticipated AV-101 supply shortages, CMO-related delays in manufacture and production of AV-101 API and finished drug product will not occur in the future.

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Even if we receive marketing approval for AV-101 or any other product candidate in the United States, we may never receive regulatory approval to market AV-101 or any other product candidate outside of the United States.

We have not yet selected any markets outside of the United States where we intend to seek regulatory approval to market our product candidates. In order to market any product outside of the United States, however, we must establish and comply with the numerous and varying safety, efficacy and other regulatory requirements of other countries. Approval procedures vary among countries and can involve additional product candidate testing and additional administrative review periods. The time required to obtain approvals in other countries might differ from that required to obtain FDA approval. The marketing approval processes in other countries may implicate all of the risks detailed above regarding FDA approval in the United States as well as other risks. In particular, in many countries outside of the United States, products must receive pricing and reimbursement approval before the product can be commercialized. Obtaining this approval can result in substantial delays in bringing products to market in such countries. Marketing approval in one country does not ensure marketing approval in another, but a failure or delay in obtaining marketing approval in one country may have a negative effect on the regulatory process in others. Failure to obtain marketing approval in other countries or any delay or other setback in obtaining such approval would impair our ability to market our product candidates in such foreign markets. Any such impairment would reduce the size of our potential market, which could have a material adverse impact on our business, results of operations and prospects.

If any of our product candidates are ultimately regulated as controlled substances, we, our contract manufacturers, as well as future distributors, prescribers, and dispensers will be required to comply with additional regulatory requirements which could delay the marketing of our product candidates, and increase the cost and burden of manufacturing, distributing, dispensing, and prescribing our product candidates.

Before we can commercialize our product candidates, the United States Drug Enforcement Administration (DEA) may need to determine the controlled substance schedule, taking into account the recommendation of the FDA. This may be a lengthy process that could delay our marketing of a product candidate and could potentially diminish any regulatory exclusivity periods for which we may be eligible, which would increase the cost associated with commercializing such products and, in turn, may have an adverse impact on our results of operations. While we currently do not know whether any of our product candidates will be considered to be controlled substances, certain of our product candidates, including AV-101, may be regulated as controlled substances.

If any of our product candidates are regulated as controlled substances, depending on the controlled substance schedule in which the product candidates are placed, we, our contract manufacturers, and any future distributors, prescribers, and dispensers of the scheduled product candidates may be subject to significant regulatory requirements, such as registration, security, recordkeeping, reporting, storage, distribution, importation, exportation, inventory, quota and other requirements administered by the DEA. Moreover, if any of our product candidates are regulated as controlled substances, we and our contract manufacturers would be subject to initial and periodic DEA inspection. If we or our contract manufacturers are not able to obtain or maintain any necessary DEA registrations, we may not be able to commercialize any product candidates that are deemed to be controlled substances or we may need to find alternative contract manufacturers, which would take time and cause us to incur additional costs, delaying or limit our commercialization efforts.

Because of their restrictive nature, these laws and regulations could limit commercialization of our product candidates, should they be deemed to contain controlled substances. Failure to comply with the applicable controlled substance laws and regulations can also result in administrative, civil or criminal enforcement. The DEA may seek civil penalties, refuse to renew necessary registrations, or initiate administrative proceedings to revoke those registrations. In some circumstances, violations could result in criminal proceedings or consent decrees. Individual states also independently regulate controlled substances.

If we are unable to establish sales and marketing capabilities or enter into agreements with third parties to market and sell our product candidates, we may not be able to generate any revenue.

We do not currently have an infrastructure for the sale, marketing and distribution of pharmaceutical products, nor do we intend to create such capabilities. Therefore, in order to market our product candidates, if approved by the FDA or any other regulatory body, we must make contractual arrangements with third parties to perform services related to sales, marketing, managerial and other non-technical capabilities relating to the commercialization of our product candidates. If we are unable to establish adequate contractual arrangements for such sales, marketing and distribution capabilities, or if we are unable to do so on commercially reasonable terms, our business, results of operations, financial condition and prospects will be materially adversely affected.

Even if we receive marketing approval for our product candidates, our product candidates may not achieve broad market acceptance, which would limit the revenue that we generate from their sales.

The commercial success of our product candidates, if approved by the FDA or other applicable regulatory authorities, will depend upon the awareness and acceptance of our product candidates among the medical community, including physicians, patients and healthcare payors. Market acceptance of our product candidates, if approved, will depend on a number of factors, including, among others:

the efficacy and safety of our product candidates as demonstrated in clinical trials, and, if required by any applicable regulatory authority in connection with the approval for the applicable indications, to provide patients with incremental health benefits, as compared with other available therapies;

limitations or warnings contained in the labeling approved for our product candidates by the FDA or other applicable regulatory authorities;

the clinical indications for which our product candidates are approved;

availability of alternative treatments already approved or expected to be commercially launched in the near future;

the potential and perceived advantages of our product candidates over current treatment options or alternative treatments, including future 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 and timing of market introduction of competitive products;

publicity concerning our products or competing products and treatments;

pricing and cost effectiveness;

the effectiveness of our sales and marketing strategies;

our ability to increase awareness of our product candidates through marketing efforts;

our ability to obtain sufficient third-party coverage or reimbursement; or

the willingness of patients to pay out-of-pocket in the absence of third-party coverage.

If our product candidates are approved but do not achieve an adequate level of acceptance by patients, physicians and payors, we may not generate sufficient revenue from our product candidates to become or remain profitable. Before granting reimbursement approval, healthcare payors may require us to demonstrate that our product candidates, in addition to treating these target indications, also provide incremental health benefits to patients. Our efforts to educate the medical community and third-party payors about the benefits of our product candidates may require significant resources and may never be successful.

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Our product candidates may cause undesirable safety concerns and side effects that could delay or prevent their regulatory approval, limit the commercial profile of an approved label, or result in significant negative consequences following marketing approval, if any.

Undesirable safety concerns and side effects caused by our product candidates could cause us or regulatory authorities to interrupt, delay or halt non-clinical studies and clinical trials and could result in a more restrictive label or the delay or denial of regulatory approval by the FDA or other regulatory authorities.

Further, clinical trials by their nature utilize a sample of potential patient populations. With a limited number of patients and limited duration of exposure, rare and severe side effects of our product candidates may only be uncovered with a significantly larger number of patients exposed to the product candidate. If our product candidates receive marketing approval and we or others identify undesirable safety concerns or side effects caused by such product candidates (or any other similar products) after such approval, a number of potentially significant negative consequences could result, including:

regulatory authorities may withdraw or limit their approval of such product candidates;

regulatory authorities may require the addition of labeling statements, such as a “black box” warning or a contraindication;

we may be required to change the way such product candidates are distributed or administered, conduct additional clinical trials or change the labeling of the product candidates;

we may be subject to regulatory investigations and government enforcement actions;

we may decide to remove such product candidates from the marketplace;

we could be sued and held liable for injury caused to individuals exposed to or taking our product candidates; and

our reputation may suffer.

We believe that any of these events could prevent us from achieving or maintaining market acceptance of the affected product candidates and would substantially increase the costs of commercializing our product candidates and significantly impact our ability to successfully commercialize our product candidates and generate revenues.

Even if we receive marketing approval for our product candidates, we may still face future development and regulatory difficulties.

Even if we receive marketing approval for our product candidates, regulatory authorities may still impose significant restrictions on our product candidates, indicated uses or marketing or impose ongoing requirements for potentially costly post-approval studies. Our product candidates will also be subject to ongoing regulatory requirements

governing the labeling, packaging, storage and promotion of the product and record keeping and submission of safety and other post-market information. The FDA has significant post-marketing authority, including, for example, the authority to require labeling changes based on new safety information and to require post-marketing studies or clinical trials to evaluate serious safety risks related to the use of a drug. The FDA also has the authority to require, as part of an NDA or post-approval, the submission of a REMS. Any REMS required by the FDA may lead to increased costs to assure compliance with new post-approval regulatory requirements and potential requirements or restrictions on the sale of approved products, all of which could lead to lower sales volume and revenue.

Manufacturers of drug products and their facilities are subject to continual review and periodic inspections by the FDA and other regulatory authorities for compliance with cGMPs and other regulations. If we or a regulatory agency discover problems with our product candidates, such as adverse events of unanticipated severity or frequency, or problems with the facility where our product candidates are manufactured, a regulatory agency may impose restrictions on our product candidates, the manufacturer or us, including requiring withdrawal of our product candidates from the market or suspension of manufacturing. If we, our product candidates or the manufacturing facilities for our product candidates fail to comply with applicable regulatory requirements, a regulatory agency may, among other things:

issue warning letters or untitled letters;

seek an injunction or impose civil or criminal penalties or monetary fines;

suspend or withdraw marketing approval;

suspend any ongoing clinical trials;

refuse to approve pending applications or supplements to applications submitted by us;

suspend or impose restrictions on operations, including costly new manufacturing requirements; or

seize or detain products, refuse to permit the import or export of products, or require that we initiate a product recall.

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Competing therapies could emerge adversely affecting our opportunity to generate revenue from the sale of our product candidates.

The pharmaceuticals industry is highly competitive. There are many public and private pharmaceutical companies, universities, governmental agencies and other research organizations actively engaged in the research and development of product candidates that may be similar to our product candidates or address similar markets. It is probable that the number of companies seeking to develop product candidates similar to our product candidates will increase.

Currently, management is unaware of any FDA-approved oral adjunctive therapy for MDD patients with an inadequate response to standard antidepressants having the same mechanism of action and safety profile as AV-101. However, new antidepressant products with other mechanisms of action or products approved for other indications, including the anesthetic ketamine hydrochloride, are being or may be used off-label for treatment of MDD, as well as other CNS indications for which AV-101 may have therapeutic potential. Additionally, other non-pharmaceutical treatment options, such as psychotherapy and electroconvulsive therapy (ECT) are sometimes used before or instead of standard antidepressant medications to treat patients with MDD.

In the field of new generation, orally available, adjunctive treatments of adult MDD patients with an inadequate response to standard antidepressants, we believe our principal competitor is Alkermes' orally available drug candidate in Phase 3 development, ALKS-5461.

Many of our potential competitors, alone or with their strategic partners, have substantially greater financial, technical and human resources than we do and significantly greater experience in the discovery and development of product candidates, obtaining FDA and other regulatory approvals of treatments and the commercialization of those treatments. We believe that a range of pharmaceutical and biotechnology companies have programs to develop small molecule drug candidates for the treatment of depression, including MDD, Parkinson's disease levodopa induced dyskinesia, neuropathic pain, epilepsy, and other neurological conditions and diseases, including, but not limited to, Abbott Laboratories, Acadia, Allergan, Alkermes, Astra Zeneca, Eli Lilly, GlaxoSmithKline, IntraCellular, Johnson & Johnson/Janssen, Lundbeck, Merck, Novartis, Ono, Otsuka, Pfizer, Roche, Sage, Sumitomo Dainippon, and Takeda, as well as any affiliates of the foregoing companies. Mergers and acquisitions in the biotechnology and pharmaceutical industries may result in even more resources being concentrated among a smaller number of our competitors. Our commercial opportunity could be reduced or eliminated if our competitors develop and commercialize products that are safer, more effective, have fewer or less severe side effects, are more convenient or are less expensive than any products that we may develop. Our competitors also may obtain FDA or other regulatory approval for their products more rapidly than we may obtain approval for ours, which could result in our competitors establishing a strong market position before we are able to enter the market.

We may seek to establish collaborations, and, if we are not able to establish them on commercially reasonable terms, we may have to alter our development and commercialization plans.

Our drug development programs and the potential commercialization of our product candidates will require substantial additional cash to fund expenses. For some of our product candidates, we may decide to collaborate with pharmaceutical and biotechnology companies for the development and potential commercialization of those product candidates.

We face significant competition in seeking appropriate collaborators. Whether we reach a definitive agreement for collaboration will depend, among other things, upon our assessment of the collaborator's resources and expertise, the terms and conditions of the proposed collaboration and the proposed collaborator's evaluation of a number of factors.

Those factors may include the design or results of clinical trials, the likelihood of approval by the FDA or similar regulatory authorities outside the United States, the potential markets for the subject product candidate, the costs and complexities of manufacturing and delivering such product candidate to patients, the potential of competing products, the existence of uncertainty with respect to our ownership of technology, which can exist if there is a challenge to such ownership without regard to the merits of the challenge and industry and market conditions generally. The collaborator may also consider alternative product candidates or technologies for similar indications that may be available to collaborate on and whether such collaboration could be more attractive than the one with us for our product candidate. The terms of any collaboration or other arrangements that we may establish may not be favorable to us.

We may also be restricted under existing collaboration agreements from entering into future agreements on certain terms with potential collaborators. Collaborations are complex and time-consuming to negotiate and document. In addition, there have been a significant number of recent business combinations among large pharmaceutical companies that have resulted in a reduced number of potential future collaborators.

We may not be able to negotiate collaborations on a timely basis, on acceptable terms, or at all. If we are unable to do so, we may have to curtail the development of the product candidate for which we are seeking to collaborate, reduce or delay its development program or one or more of our other development programs, delay its potential commercialization or reduce the scope of any sales or marketing activities, or increase our expenditures and undertake development or commercialization activities at our own expense. If we elect to increase our expenditures to fund development or commercialization activities on our own, we may need to obtain additional capital, which may not be available to us on acceptable terms or at all. If we do not have sufficient funds, we may not be able to further develop our product candidates or bring them to market and generate product revenue.

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In addition, any future collaboration that we enter into may not be successful. The success of our collaboration arrangements will depend heavily on the efforts and activities of our collaborators. Collaborators generally have significant discretion in determining the efforts and resources that they will apply to these collaborations. Disagreements between parties to a collaboration arrangement regarding clinical development and commercialization matters can lead to delays in the development process or commercializing the applicable product candidate and, in some cases, termination of the collaboration arrangement. These disagreements can be difficult to resolve if neither of the parties has final decision-making authority. Collaborations with pharmaceutical or biotechnology companies and other third parties often are terminated or allowed to expire by the other party. Any such termination or expiration would adversely affect us financially and could harm our business reputation.

We may not be successful in our efforts to identify or discover additional product candidates or we may expend our limited resources to pursue a particular product candidate or indication and fail to capitalize on product candidates or indications that may be more profitable or for which there is a greater likelihood of success.

The success of our business depends primarily upon our ability to identify, develop and commercialize product candidates with commercial and therapeutic potential. Although AV-101 is in Phase 2 clinical development for treatment of depression, we may fail to pursue additional CNS-related Phase 2 development opportunities for AV-101, or identify additional product candidates for clinical development for a number of reasons. Our research methodology may be unsuccessful in identifying new product candidates or our product candidates may be shown to have harmful side effects or may have other characteristics that may make the products unmarketable or unlikely to receive marketing approval.

Because we currently have limited financial and management resources, we necessarily focus on a limited number of research and development programs and product candidates and are currently focused primarily on development of AV-101, with additional limited focus on NCE drug rescue and RM. As a result, we may forego or delay pursuit of opportunities with other product candidates or for other potential CNS-related indications for AV-101 that later prove to have greater commercial potential. Our resource allocation decisions may cause us to fail to capitalize on viable commercial drugs or profitable market opportunities. Our spending on current and future research and development programs and product candidates for specific indications may not yield any commercially viable drugs. If we do not accurately evaluate the commercial potential or target market for a particular product candidate, we may relinquish valuable rights to that product candidate through future collaboration, licensing or other royalty arrangements in cases in which it would have been more advantageous for us to retain sole development and commercialization rights to such product candidate.

If any of these events occur, we may be forced to abandon our development efforts for a program or programs, which would have a material adverse effect on our business and could potentially cause us to cease operations. Research and development programs to identify and advance new product candidates require substantial technical, financial and human resources. We may focus our efforts and resources on potential programs or product candidates that ultimately prove to be unsuccessful.

We are subject to healthcare laws and regulations, which could expose us to criminal sanctions, civil penalties, contractual damages, reputational harm and diminished profits and future earnings.

Although we do not currently have any products on the market, once we begin commercializing our products, we may be subject to additional healthcare statutory and regulatory requirements and enforcement by the federal government and the states and foreign governments in which we conduct our business. Healthcare providers, physicians and others will play a primary role in the recommendation and prescription of our product candidates, if approved. Our future arrangements with third-party payors will expose us to broadly applicable fraud and abuse and other healthcare laws

and regulations that may constrain the business or financial arrangements and relationships through which we market, sell and distribute our product candidates, if we obtain marketing approval. Restrictions under applicable federal and state healthcare laws and regulations include the following:

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

The federal False Claims Act imposes criminal and civil penalties, including those from civil whistleblower or qui tam actions, against individuals or entities for knowingly presenting, or causing to be presented, to the federal government, claims for payment that are false or fraudulent or making a false statement to avoid, decrease, or conceal an obligation to pay money to the federal government.

The federal Health Insurance Portability and Accountability Act of 1996, as amended by the Health Information Technology for Economic and Clinical Health Act, imposes criminal and civil liability for executing a scheme to defraud any healthcare benefit program and also imposes obligations, including mandatory contractual terms, with respect to safeguarding the privacy, security and transmission of individually identifiable health information.

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

The federal transparency requirements, sometimes referred to as the “Sunshine Act,” under the Patient Protection and Affordable Care Act, require manufacturers of drugs, devices, biologics and medical supplies that are reimbursable under Medicare, Medicaid, or the Children’s Health Insurance Program to report to the Department of Health and Human Services information related to physician payments and other transfers of value and physician ownership and investment interests.

Analogous state laws and regulations, such as state anti-kickback and false claims laws and transparency laws, may apply to sales or marketing arrangements and claims involving healthcare items or services reimbursed by non-governmental third-party payors, including private insurers, and some state laws require pharmaceutical companies to comply with the pharmaceutical industry’s voluntary compliance guidelines and the relevant compliance.

Guidance promulgated by the federal government in addition to requiring drug manufacturers to report information related to payments to physicians and other healthcare providers or marketing expenditures and drug pricing.

Ensuring that our future business arrangements with third parties comply with applicable healthcare laws and regulations could be costly. It is possible that governmental authorities will conclude that our business practices do not comply with current or future statutes, regulations or case law involving applicable fraud and abuse or other healthcare laws and regulations. If our operations, including anticipated activities to be conducted by our sales team, were 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 and exclusion from government funded healthcare programs, such as Medicare and Medicaid, any of which could substantially disrupt our operations. If any of the physicians or other providers or entities with whom we expect to do business is found not to be in compliance with applicable laws, they may be subject to criminal, civil or administrative sanctions, including exclusions from government funded healthcare programs.

The FDA and other regulatory agencies actively enforce the laws and regulations prohibiting the promotion of off-label uses. If we are found to have improperly promoted off-label uses, we may become subject to significant liability.

The FDA and other regulatory agencies strictly regulate the promotional claims that may be made about prescription products, such as AV-101, if approved. In particular, a product may not be promoted for uses that are not approved by the FDA or such other regulatory agencies as reflected in the product’s approved labeling. For example, if we receive marketing approval for AV-101 as an adjunctive treatment of MDD, physicians may nevertheless prescribe AV-101 to their patients in a manner that is inconsistent with the approved label. If we are found to have promoted such off-label uses, we may become subject to significant liability. The federal government has levied large civil and criminal fines against companies for alleged improper promotion and has enjoined several companies from engaging in off-label promotion. The FDA has also requested that companies enter into consent decrees or permanent injunctions under which specified promotional conduct is 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.

Even if approved, reimbursement policies could limit our ability to sell our product candidates.

Market acceptance and sales of our product candidates will depend heavily on reimbursement policies and may be affected by healthcare reform measures. Government authorities and third-party payors, such as private health insurers and health maintenance organizations, decide which medications they will pay for and establish reimbursement levels for those medications. Cost containment is a primary concern in the U.S. healthcare industry and elsewhere. Government authorities and these third-party payors have attempted to control costs by limiting coverage and the amount of reimbursement for particular medications. We cannot be sure that reimbursement will be available for our product candidates and, if reimbursement is available, the level of such reimbursement. Reimbursement may impact the demand for, or the price of, our product candidates. If reimbursement is not available or is available only at limited levels, we may not be able to successfully commercialize our product candidates.

In some foreign countries, particularly in Canada and European countries, the pricing of prescription pharmaceuticals is subject to strict governmental control. In these countries, pricing negotiations with governmental authorities can take six months or longer after the receipt of regulatory approval and product launch. To obtain favorable reimbursement for the indications sought or pricing approval in some countries, we may be required to conduct a clinical trial that compares the cost-effectiveness of our product candidates with other available therapies. If reimbursement for our product candidates is unavailable in any country in which we seek reimbursement, if it is limited in scope or amount, if it is conditioned upon our completion of additional clinical trials, or if pricing is set at unsatisfactory levels, our operating results could be materially adversely affected.

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We may seek FDA Orphan Drug designation for one or more of our product candidates, including AV-101. Even if we have obtained FDA Orphan Drug designation for AV-101 of other product candidates, there may be limits to the regulatory exclusivity afforded by such designation.

We may, in the future, choose to seek FDA Orphan Drug designation for one or more of our product candidates, including AV-101. Even if we obtain Orphan Drug designation from the FDA for AV-101 or any other product candidates, there are limitations to the exclusivity afforded by such designation. In the United States, the company that first obtains FDA approval for a designated orphan drug for the specified rare disease or condition receives orphan drug marketing exclusivity for that drug for a period of seven years. This orphan drug exclusivity prevents the FDA from approving another application, including a full NDA to market the same drug for the same orphan indication, except in very limited circumstances, including when the FDA concludes that the later drug is safer, more effective or makes a major contribution to patient care. For purposes of small molecule drugs, the FDA defines “same drug” as a drug that contains the same active moiety and is intended for the same use as the drug in question. To obtain Orphan Drug status for a drug that shares the same active moiety as an already approved drug, it must be demonstrated to the FDA that the drug is safer or more effective than the approved orphan designated drug, or that it makes a major contribution to patient care. In addition, a designated orphan drug may not receive orphan drug exclusivity if it is approved for a use that is broader than the indication for which it received orphan designation. In addition, orphan drug exclusive marketing rights in the United States may be lost if the FDA later determines that the request for designation was materially defective or if the manufacturer is unable to assure sufficient quantity of the drug to meet the needs of patients with the rare disease or condition or if another drug with the same active moiety is determined to be safer, more effective, or represents a major contribution to patient care.

Our future growth may depend, in part, on our ability to penetrate foreign markets, where we would be subject to additional regulatory burdens and other risks and uncertainties.

Our future profitability may depend, in part, on our ability to commercialize our product candidates in foreign markets for which we may rely on collaboration with third parties. If we commercialize our product candidates in foreign markets, we would be subject to additional risks and uncertainties, including:

our customers’ ability to obtain reimbursement for our product candidates in foreign markets;

our inability to directly control commercial activities because we are relying on third parties;

the burden of complying with complex and changing foreign regulatory, tax, accounting and legal requirements;

different medical practices and customs in foreign countries affecting acceptance in the marketplace;

import or export licensing requirements;

longer accounts receivable collection times;

longer lead times for shipping;

language barriers for technical training;

reduced protection of intellectual property rights in some foreign countries;

the existence of additional potentially relevant third party intellectual property rights;

foreign currency exchange rate fluctuations; and

the interpretation of contractual provisions governed by foreign laws in the event of a contract dispute.

Foreign sales of our product candidates could also be adversely affected by the imposition of governmental controls, political and economic instability, trade restrictions and changes in tariffs.

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We are a development stage biopharmaceutical company with no current revenues or approved products, and limited experience developing new drug, biological and/or regenerative medicine candidates, including conducting clinical trials and other areas required for the successful development and commercialization of therapeutic products, which makes it difficult to assess our future viability.

We are a development stage biopharmaceutical company. Although our lead drug candidate is in Phase 2 development, we currently have no approved products and currently generate no revenues, and we have not yet fully demonstrated an ability to overcome many of the fundamental risks and uncertainties frequently encountered by development stage companies in new and rapidly evolving fields of technology, particularly biotechnology. To execute our business plan successfully, we will need to accomplish the following fundamental objectives, either on our own or with strategic collaborators:

produce product candidates;

develop and obtain required regulatory approvals for commercialization of product candidates we produce;

maintain, leverage and expand our intellectual property portfolio;

establish and maintain sales, distribution and marketing capabilities, and/or enter into strategic partnering arrangements to access such capabilities;

gain market acceptance for our products; and

obtain adequate capital resources and manage our spending as costs and expenses increase due to research, production, development, regulatory approval and commercialization of product candidates.

Our future success is highly dependent upon our ability to successfully develop and commercialize AV-101 and discover, as well as produce, develop and commercialize proprietary drug rescue NCEs using our stem cell technology, and we cannot provide any assurance that we will successfully develop and commercialize AV-101 or drug rescue NCEs, or that, if produced, AV-101 or any drug rescue NCE will be successfully commercialized.

Research programs designed to identify and produce drug rescue NCEs require substantial technical, financial and human resources, whether or not any NCEs are ultimately identified and produced. In particular, our drug rescue programs may initially show promise in identifying potential NCEs, yet fail to yield a lead NCE suitable for preclinical, clinical development or commercialization for many reasons, including the following:

our drug rescue research and development methodology may not be successful in identifying and developing potential drug rescue NCEs;

competitors may develop alternatives that render our drug rescue NCEs obsolete;

a drug rescue NCE may, on further study, be shown to have harmful side effects or other characteristics that indicate it is unlikely to be effective or otherwise does not meet applicable regulatory criteria;

a drug rescue NCE may not be capable of being produced in commercial quantities at an acceptable cost, or at all; or

a drug rescue NCE may not be accepted as safe and effective by regulatory authorities, patients, the medical community or third-party payors.

In addition, we do not have a sales or marketing infrastructure, and we, including our executive officers, do not have any significant pharmaceutical sales, marketing or distribution experience. We may seek to collaborate with others to develop and commercialize AV-101, drug rescue NCEs and/or other product candidates if and when they are developed. If we enter into arrangements with third parties to perform sales, marketing and distribution services for our products, the resulting revenues or the profitability from these revenues to us are likely to be lower than if we had sold, marketed and distributed our products ourselves. In addition, we may not be successful in entering into arrangements with third parties to sell, market and distribute AV-101, any drug rescue NCEs or other product candidates or may be unable to do so on terms that are favorable to us. We likely will have little control over such third parties, and any of these third parties may fail to devote the necessary resources and attention to sell, market and distribute our products effectively. If we do not establish sales, marketing and distribution capabilities successfully, in collaboration with third parties, we will not be successful in commercializing our product candidates.

We have limited operating history with respect to drug development, including our anticipated focus on the identification and assessment of potential drug rescue NCEs and no operating history with respect to the production of drug rescue NCEs, and we may never be able to produce a drug rescue NCE.

If we are unable to develop and commercialize AV-101 or produce suitable drug rescue NCEs, we may not be able to generate sufficient revenues to execute our business plan, which likely would result in significant harm to our financial position and results of operations, which could adversely impact our stock price.

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There are a number of factors, in addition to the utility of CardioSafe 3D, that may impact our ability to identify and produce, develop or out-license and commercialize drug rescue NCEs, independently or with strategic partners, including:

our ability to identify potential drug rescue candidates in the public domain, obtain sufficient quantities of them, and assess them using our bioassay systems;

if we seek to rescue drug rescue candidates that are not available to us in the public domain, the extent to which third parties may be willing to out-license or sell certain drug rescue candidates to us on commercially reasonable terms;

our medicinal chemistry collaborator's ability to design and produce proprietary drug rescue NCEs based on the novel biology and structure-function insight we provide using CardioSafe 3D; and

financial resources available to us to develop and commercialize lead drug rescue NCEs internally, or, if we out-license them to strategic partners, the resources such partners choose to dedicate to development and commercialization of any drug rescue NCEs they license from us.

Even if we do produce proprietary drug rescue NCEs, we can give no assurance that we will be able to develop and commercialize them as a marketable drug, on our own or in collaboration with others. Before we generate any revenues from AV-101 and/or additional drug rescue NCEs we or our potential collaborators must complete preclinical and clinical developments, submit clinical and manufacturing data to the FDA, qualify a third party contract manufacturer, receive regulatory approval in one or more jurisdictions, satisfy the FDA that our contract manufacturer is capable of manufacturing the product in compliance with cGMP, build a commercial organization, make substantial investments and undertake significant marketing efforts ourselves or in partnership with others. We are not permitted to market or promote any of our product candidates before we receive regulatory approval from the FDA or comparable foreign regulatory authorities, and we may never receive such regulatory approval for any of our product candidates.

If CardioSafe 3D fails to predict accurately and efficiently the cardiac effects, both toxic and nontoxic, of drug rescue candidates and drug rescue NCEs, then our drug rescue programs will be adversely affected.

Our success is partly dependent on our ability to use CardioSafe 3D to identify and predict, accurately and efficiently, the potential toxic and nontoxic cardiac effects of drug rescue candidates and drug rescue NCEs. If CardioSafe 3D is not capable of providing physiologically relevant and clinically predictive information regarding human cardiac biology, our drug rescue business will be adversely affected.

CardioSafe 3D may not be meaningfully more predictive of the behavior of human cells than existing methods.

The success of our drug rescue programs is highly dependent upon CardioSafe 3D being more accurate, efficient and clinically predictive than long-established surrogate safety models, including animal cells and live animals, and immortalized, primary and transformed cells, currently used by pharmaceutical companies and others. We cannot give assurance that CardioSafe 3D will be more efficient or accurate at predicting the heart safety of new drug candidates than the testing models currently used. If CardioSafe 3D fails to provide a meaningful difference compared to existing or new models in predicting the behavior of human heart, respectively, their utility for drug rescue will be limited and

our drug rescue business will be adversely affected.

We may invest in producing drug rescue NCEs for which there proves to be no demand.

To generate revenue from our drug rescue activities, we must produce proprietary drug rescue NCEs for which there proves to be demand within the healthcare marketplace, and, if we intend to out-license a particular drug rescue NCE for development and commercialization prior to market approval, then also among pharmaceutical companies and other potential collaborators. However, we may produce drug rescue NCEs for which there proves to be no or limited demand in the healthcare market and/or among pharmaceutical companies and others. If we misinterpret market conditions, underestimate development costs and/or seek to rescue the wrong drug rescue candidates, we may fail to generate sufficient revenue or other value, on our own or in collaboration with others, to justify our investments, and our drug rescue business may be adversely affected.

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We may experience difficulty in producing human cells and our future stem cell technology research and development efforts may not be successful within the timeline anticipated, if at all.

Our human pluripotent stem cell technology is technically complex, and the time and resources necessary to develop various human cell types and customized bioassay systems are difficult to predict in advance. We might decide to devote significant personnel and financial resources to research and development activities designed to expand, in the case of drug rescue, and explore, in the case of drug discovery and regenerative medicine, potential applications of our stem cell technology platform. In particular, we may conduct exploratory non-clinical RM programs involving blood, bone, cartilage, and/or liver cells. Although we and our collaborators have developed proprietary protocols for the production of multiple differentiated cell types, we could encounter difficulties in differentiating and producing sufficient quantities of particular cell types, even when following these proprietary protocols. These difficulties could result in delays in production of certain cells, assessment of certain drug rescue candidates and drug rescue NCEs, design and development of certain human cellular assays and performance of certain exploratory non-clinical regenerative medicine studies. In the past, our stem cell research and development projects have been significantly delayed when we encountered unanticipated difficulties in differentiating human pluripotent stem cells into heart and liver cells. Although we have overcome such difficulties in the past, we may have similar delays in the future, and we may not be able to overcome them or obtain any benefits from our future stem cell technology research and development activities. Any delay or failure by us, for example, to produce functional, mature blood, bone, cartilage, and liver cells could have a substantial and material adverse effect on our potential drug discovery, drug rescue and regenerative medicine business opportunities and results of operations.

Restrictions on research and development involving human embryonic stem cells and religious and political pressure regarding such stem cell research and development could impair our ability to conduct or sponsor certain potential collaborative research and development programs and adversely affect our prospects, the market price of our common stock and our business model.

Some of our research and development programs may involve the use of human cells derived from our controlled differentiation of human embryonic stem cells (hESCs). Some believe the use of hESCs gives rise to ethical and social issues regarding the appropriate use of these cells. Our research related to differentiation of hESCs may become the subject of adverse commentary or publicity, which could significantly harm the market price of our common stock. Although now substantially less than in years past, certain political and religious groups in the United States and elsewhere voice opposition to hESC technology and practices. We may use hESCs derived from excess fertilized eggs that have been created for clinical use in in vitro fertilization (IVF) procedures and have been donated for research purposes with the informed consent of the donors after a successful IVF procedure because they are no longer desired or suitable for IVF. Certain academic research institutions have adopted policies regarding the ethical use of human embryonic tissue. These policies may have the effect of limiting the scope of future collaborative research opportunities with such institutions, thereby potentially impairing our ability to conduct certain research and development in this field that we believe is necessary to expand the drug rescue capabilities of our technology, which would have a material adverse effect on our business.

The use of embryonic or fetal tissue in research (including the derivation of hESCs) in other countries is regulated by the government, and varies widely from country to country. Government-imposed restrictions with respect to use of hESCs in research and development could have a material adverse effect on us by harming our ability to establish critical collaborations, delaying or preventing progress in our research and development, and causing a decrease in the market interest in our stock.

The foregoing potential ethical concerns do not apply to our use of induced pluripotent stem cells (iPSCs) because their derivation does not involve the use of embryonic tissues.

We have assumed that the biological capabilities of iPSCs and hESCs are likely to be comparable. If it is discovered that this assumption is incorrect, our exploratory research and development activities focused on potential regenerative medicine applications of our stem cell technology platform could be harmed.

We may use both hESCs and iPSCs to produce human cells for our customized in vitro assays for drug discovery and drug rescue purposes. However, we anticipate that our future exploratory research and development, if any, focused on potential regenerative medicine applications of our stem cell technology platform primarily will involve iPSCs. With respect to iPSCs, we believe scientists are still somewhat uncertain about the clinical utility, life span, and safety of such cells, and whether such cells differ in any clinically significant ways from hESCs. If we discover that iPSCs will not be useful for whatever reason for potential regenerative medicine programs, this would negatively affect our ability to explore expansion of our platform in that manner, including, in particular, where it would be preferable to use iPSCs to reproduce rather than approximate the effects of certain specific genetic variations.

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

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

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

In addition, we may incur substantial costs in order to comply with current or future environmental, health and safety laws and regulations. These current or future laws and regulations may impair our research, development or production efforts. Failure to comply with these laws and regulations also may result in substantial fines, penalties or other sanctions, which could have a material adverse effect on our operations.

To the extent our research and development activities involve using iPSCs, we will be subject to complex and evolving laws and regulations regarding privacy and informed consent. Many of these laws and regulations are subject to change and uncertain interpretation, and could result in claims, changes to our research and development programs and objectives, increased cost of operations or otherwise harm the Company.

To the extent that we pursue research and development activities involving iPSCs, we will be subject to a variety of laws and regulations in the United States and abroad that involve matters central to such research and development activities, including obligations to seek informed consent from donors for the use of their blood and other tissue to produce, or have produced for us, iPSCs, as well as state and federal laws that protect the privacy of such donors. United States federal and state and foreign laws and regulations are constantly evolving and can be subject to significant change. If we engage in iPSC-related research and development activities in countries other than the United States, we may become subject to foreign laws and regulations relating to human subjects research and other laws and regulations that are often more restrictive than those in the United States. In addition, both the application and interpretation of these laws and regulations are often uncertain, particularly in the rapidly evolving stem cell technology sector in which we operate. These laws and regulations can be costly to comply with and can delay or impede our research and development activities, result in negative publicity, increase our operating costs, require significant management time and attention and subject us to claims or other remedies, including fines or demands that we modify or cease existing business practices.

Legal, social and ethical concerns surrounding the use of iPSCs, biological materials and genetic information could impair our operations.

To the extent that our future stem cell research and development activities involve the use of iPSCs and the manipulation of human tissue and genetic information, the information we derive from such iPSC-related research and development activities could be used in a variety of applications, which may have underlying legal, social and ethical concerns, including the genetic engineering or modification of human cells, testing for genetic predisposition for

certain medical conditions and stem cell banking. Governmental authorities could, for safety, social or other purposes, call for limits on or impose regulations on the use of iPSCs and genetic testing or the manufacture or use of certain biological materials involved in our iPSC-related research and development programs. Such concerns or governmental restrictions could limit our future research and development activities, which could have a material adverse effect on our business, financial condition and results of operations.

Our human cellular bioassay systems and human cells we derive from human pluripotent stem cells, although not currently subject to regulation by the FDA or other regulatory agencies as biological products or drugs, could become subject to regulation in the future.

The human cells we produce from hPSCs and our customized bioassay systems using such cells, including CardioSafe 3D, are not currently sold, for research purposes or any other purpose, to biotechnology or pharmaceutical companies, government research institutions, academic and nonprofit research institutions, medical research organizations or stem cell banks, and they are not therapeutic procedures. As a result, they are not subject to regulation as biological products or drugs by the FDA or comparable agencies in other countries. However, if, in the future, we seek to include human cells we derive from hPSCs in therapeutic applications or product candidates, such applications and/or product candidates would be subject to the FDA's pre- and post-market regulations. For example, if we seek to develop and market human cells we produce for use in performing regenerative medicine applications, such as tissue engineering or organ replacement, we would first need to obtain FDA pre-market clearance or approval. Obtaining such clearance or approval from the FDA is expensive, time-consuming and uncertain, generally requiring many years to obtain, and requiring detailed and comprehensive scientific and clinical data. Notwithstanding the time and expense, these efforts may not result in FDA approval or clearance. Even if we were to obtain regulatory approval or clearance, it may not be for the uses that we believe are important or commercially attractive.

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Risks Related to Our Financial Position

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

We have incurred significant net losses in each fiscal year since our inception in 1998, including net losses of \$10.3 million and \$47.2 million, which includes \$26.7 million of non-cash expense related to the extinguishment of essentially all of our outstanding promissory notes and certain other indebtedness, during the fiscal years ended March 31, 2017 and 2016, respectively. We incurred a net loss of approximately \$7.3 million in the six months ended September 30, 2017 and, as of that date, we had an accumulated deficit of approximately \$149.3 million. We do not know whether or when we will become profitable. Substantially all of our operating losses have resulted from costs incurred in connection with our research and development programs and from general and administrative costs associated with our operations. We expect to incur increasing levels of operating losses over the next several years and for the foreseeable future. Our prior losses, combined with expected future losses, have had and will continue to have an adverse effect on our stockholders' equity (deficit) and working capital. We expect our research and development expenses to significantly increase in connection with non-clinical studies and clinical trials of our product candidates. In addition, if we obtain marketing approval for our product candidates, we may incur significant sales, marketing and outsourced-manufacturing expenses should we elect not to collaborate with one or more third parties for such services and capabilities. As a public company, we incur additional costs associated with operating as a public company. As a result, we expect to continue to incur significant and increasing operating losses for the foreseeable future. Because of the numerous risks and uncertainties associated with developing pharmaceutical products, we are unable to predict the extent of any future losses or when we will become profitable, if at all. Even if we do become profitable, we may not be able to sustain or increase our profitability on a quarterly or annual basis.

Our ability to become profitable depends upon our ability to generate revenues. To date, we have generated approximately \$17.7 million in revenues, including receipt of non-dilutive cash payments from collaborators, sublicense revenue, and research and development grant awards from the NIH, not including the fair market value of the ongoing NIMH AV-101 MDD Phase 2 Monotherapy Study under our NIMH CRADA. We have not yet commercialized any product or generated any revenues from product sales, and we do not know when, or if, we will generate any revenue from product sales. We do not expect to generate significant revenue unless and until we obtain marketing approval of, and begin to experience sales of, AV-101, or we enter into one or more development and commercialization agreements with respect to AV-101 or one or more other product candidates. Our ability to generate revenue depends on a number of factors, including, but not limited to, our ability to:

initiate and successfully complete non-clinical and clinical trials that meet their prescribed endpoints;

initiate and successfully complete all safety studies required to obtain U.S. and foreign marketing approval for our product candidates;

commercialize our product candidates, if approved, by developing a sales force or entering into collaborations with third parties; and

achieve market acceptance of our product candidates in the medical community and with third-party payors.

Unless we enter into a development and commercialization collaboration or partnership agreement, we expect to incur significant sales and marketing costs as we prepare to commercialize AV-101 or other product candidates. Even if we initiate and successfully complete pivotal clinical trials of AV-101 or other product candidates, and AV-101 or other product candidates are approved for commercial sale, and despite expending these costs, AV-101 or other product candidates may not be commercially successful. We may not achieve profitability soon after generating product sales, if ever. If we are unable to generate product revenue, we will not become profitable and may be unable to continue operations without continued funding.

We require additional financing to execute our business plan and continue to operate as a going concern.

Our audited consolidated financial statements for the year ended March 31, 2017 as well as the unaudited condensed consolidated financial statements for the period ended September 30, 2017 included elsewhere in this Report have been prepared assuming we will continue to operate as a going concern, although we and our auditors have indicated that our continuing losses and negative cash flows from operations raise substantial doubt about our ability to continue as such. Because we continue to experience net operating losses, our ability to continue as a going concern is subject to our ability to obtain necessary funding from outside sources, including obtaining additional funding from the sale of our securities or obtaining loans and grant awards from financial institutions and/or government agencies where possible. Our continued net operating losses increase the difficulty in completing such sales or securing alternative sources of funding, and there can be no assurances that we will be able to obtain such funding on favorable terms or at all. If we are unable to obtain sufficient financing from the sale of our securities or from alternative sources, we may be required to reduce, defer, or discontinue certain or all of our research and development activities or we may not be able to continue as a going concern.

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Since our inception, most of our resources have been dedicated to research and development of AV-101 and the drug rescue capabilities of our stem cell technology platform. In particular, we have expended substantial resources advancing AV-101 through preclinical development and Phase 1 clinical safety studies, and developing CardioSafe 3D and our cardiac stem cell technology for drug rescue and potential regenerative medicine applications, and we will continue to expend substantial resources for the foreseeable future developing and commercializing AV-101 for multiple CNS indications, and, potentially, developing drug rescue NCEs and RM therapies, on our own or in collaborations similar to the BlueRock Agreement. These expenditures will include costs associated with general and administrative costs, facilities costs, research and development, acquiring new technologies, manufacturing product candidates, conducting nonclinical experiments and clinical trials and obtaining regulatory approvals, as well as commercializing any products approved for sale.

At September 30, 2017, our existing cash and cash equivalents were not sufficient to fund our current operations for the next 12 months or to complete our proposed AV-101 MDD Phase 2 Adjunctive Treatment Study. Further, we have no current source of revenue to sustain our present activities, and we do not expect to generate revenue until, and unless, we (i) out-license or sell AV-101, a drug rescue NCE, and/or another drug candidate unrelated to AV-101 to third-parties, (ii) enter into license arrangements involving our stem cell technology, or (iii) obtain approval from the FDA or other regulatory authorities and successfully commercialize, on our own or through a future collaboration, one or more of our compounds.

As the outcome of our AV-101 and NCE drug rescue activities and future anticipated clinical trials is highly uncertain, we cannot reasonably estimate the actual amounts necessary to successfully complete the development and commercialization of our product candidates, on our own or in collaboration with others. In addition, other unanticipated costs may arise. As a result of these and other factors, we will need to seek additional capital in the near term to meet our future operating requirements, including capital necessary to develop, obtain regulatory approval for, and to commercialize our product candidates, and may seek additional capital in the event there exists favorable market conditions or strategic considerations even if we believe we have sufficient funds for our current or future operating plans. We are considering a range of potential sources of funding, including public or private equity or debt financings, government or other third-party funding, marketing and distribution arrangements and other collaborations, strategic alliances and licensing arrangements or a combination of these approaches, and we may complete additional financing arrangements in 2017 and beyond. Raising funds in the current economic environment may present additional challenges. Even if we believe we have sufficient funds for our current or future operating plans, we may seek additional capital if market conditions are favorable or if we have specific strategic considerations.

Our future capital requirements depend on many factors, including:

the number and characteristics of the product candidates we pursue, including AV-101 and drug rescue NCEs;

the scope, progress, results and costs of researching and developing our product candidates, and conducting preclinical and clinical studies;

the timing of, and the costs involved in, obtaining regulatory approvals for our product candidates;

the cost of commercialization activities if any of our product candidates are approved for sale, including marketing, sales and distribution costs;

the cost of manufacturing our product candidates and any products we successfully commercialize;

our ability to establish and maintain strategic partnerships, licensing or other arrangements and the financial terms of such agreements;

market acceptance of our products;

the effect of competing technological and market developments;

our ability to obtain government funding for our programs;

the costs involved in obtaining and enforcing patents to preserve our intellectual property;

the costs involved in defending against such claims that we infringe third-party patents or violate other intellectual property rights and the outcome of such litigation;

the timing, receipt and amount of potential future licensee fees, milestone payments, and sales of, or royalties on, our future products, if any; and

the extent to which we acquire or invest in businesses, products and technologies, although we currently have no commitments or agreements relating to any of these types of transactions.

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Any additional fundraising efforts will divert certain members of our management team from their day-to-day activities, which may adversely affect our ability to develop and commercialize our product candidates. In addition, we cannot guarantee that future financing will be available in sufficient amounts, in a timely manner, or on terms acceptable to us, if at all, and the terms of any financing may adversely affect the holdings or the rights of our stockholders and the issuance of additional securities, whether equity or debt, by us, or the possibility of such issuance, may cause the market price of our shares to decline. The sale of additional equity securities and the conversion or exchange of certain of our outstanding securities will dilute all of our stockholders. The incurrence of debt could result in increased fixed payment obligations and we could be required to agree to certain restrictive covenants, such as limitations on our ability to incur additional debt, limitations on our ability to acquire, sell or license intellectual property rights and other operating restrictions that could adversely impact our ability to conduct our business. We could also be required to seek funds through arrangements with collaborative partners or otherwise at an earlier stage than otherwise would be desirable and we may be required to relinquish rights to some of our technologies or product candidate or otherwise agree to terms unfavorable to us, any of which may have a material adverse effect on our business, operating results and prospects.

If we are unable to obtain additional funding on a timely basis and on acceptable terms, we may be required to significantly curtail, delay or discontinue one or more of our research or product development programs or the commercialization of any product candidate or be unable to continue or expand our operations or otherwise capitalize on our business opportunities, as desired, which could materially affect our business, financial condition and results of operations.

We have identified material weaknesses in our internal control over financial reporting, and our business and stock price may be adversely affected if we do not adequately address those weaknesses or if we have other material weaknesses or significant deficiencies in our internal control over financial reporting.

We have identified material weaknesses in our internal control over financial reporting. In particular, we concluded that (i) the size and capabilities of the Company's staff does not permit appropriate segregation of duties to prevent one individual from overriding the internal control system by initiating, authorizing and completing all transactions, and (ii) the Company utilizes accounting software that does not prevent erroneous or unauthorized changes to previous reporting periods and/or can be adjusted so as to not provide an adequate auditing trail of entries made in the accounting software.

The existence of one or more material weaknesses or significant deficiencies could result in errors in our financial statements, and substantial costs and resources may be required to rectify any internal control deficiencies. If we cannot produce reliable financial reports, investors could lose confidence in our reported financial information, we may be unable to obtain additional financing to operate and expand our business and our business and financial condition could be harmed.

Raising additional capital will cause substantial dilution to our existing stockholders, may restrict our operations or require us to relinquish rights, and may require us to seek stockholder approval to authorize additional shares of our common stock.

We intend to pursue private and public equity offerings, debt financings, strategic collaborations and licensing arrangements during 2017 and beyond. To the extent that we raise additional capital through the sale of common stock or securities convertible or exchangeable into common stock, or to the extent, for strategic purposes, we convert or exchange certain of our outstanding securities into common stock, our current stockholders' ownership interest in our company will be substantially diluted. In addition, the terms of any such securities may include liquidation or other preferences that materially adversely affect 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. 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.

Some of our programs have been partially supported by government grant awards, which may not be available to us in the future.

Since inception, we have received substantial funds under grant award programs funded by state and federal governmental agencies, such as the NIH, the NIH's National Institute of Neurological Disease and Stroke (NINDS) and the NIMH, and the California Institute for Regenerative Medicine (CIRM). To fund a portion of our future research and development programs, we may apply for additional grant funding from such or similar governmental organizations. However, funding by these governmental organizations may be significantly reduced or eliminated in the future for a number of reasons. For example, some programs are subject to a yearly appropriations process in Congress. In addition, we may not receive funds under future grants because of budgeting constraints of the agency administering the program. Therefore, we cannot assure you that we will receive any future grant funding from any government organization or otherwise. A restriction on the government funding available to us could reduce the resources that we would be able to devote to future research and development efforts. Such a reduction could delay the introduction of new products and hurt our competitive position.

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Our ability to use net operating losses to offset future taxable income is subject to certain limitations.

As of March 31, 2017, we had federal and state net operating loss carryforwards of \$77.1 million and \$67.6 million, respectively, which begin to expire in fiscal 2018. Under Section 382 of the Internal Revenue Code of 1986, as amended (the Code) changes in our ownership may limit the amount of our net operating loss carryforwards that could be utilized annually to offset our future taxable income, if any. This limitation would generally apply in the event of a cumulative change in ownership of our company of more than 50% within a three-year period. Any such limitation may significantly reduce our ability to utilize our net operating loss carryforwards and tax credit carryforwards before they expire. Any such limitation, whether as the result of future offerings, prior private placements, sales of our common stock by our existing stockholders or additional sales of our common stock by us in the future, could have a material adverse effect on our results of operations in future years. We have not completed a study to assess whether an ownership change for purposes of Section 382 has occurred, or whether there have been multiple ownership changes since our inception, due to the significant costs and complexities associated with such study.

General Company-Related Risks

If we fail to attract and retain senior management and key scientific personnel, we may be unable to successfully produce, develop and commercialize AV-101, drug rescue NCEs, other potential product candidates and other commercial applications of our stem cell technology.

Our success depends in part on our continued ability to attract, retain and motivate highly qualified management and scientific and technical personnel. We are highly dependent upon our Chief Executive Officer, President and Chief Scientific Officer, Chief Medical Officer and Chief Financial Officer, as well as other employees, consultants and scientific collaborators. As of the date of this Report, we have nine full-time employees, which may make us more reliant on our individual employees than companies with a greater number of employees. The loss of services of any of these individuals could delay or prevent the successful development of AV-101, drug rescue NCEs, other product candidates, and other applications of our stem cell technology, including our production and assessment of potential drug rescue NCEs or disrupt our administrative functions.

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

In addition, we rely on a diverse range of strategic consultants and advisors, including manufacturing, scientific and clinical development, and regulatory advisors, to assist us in designing and implementing our research and development and regulatory strategies and plans, including our AV-101 development and drug rescue strategies and plans. Our consultants and advisors may be employed by employers other than us and may have commitments under consulting or advisory contracts with other entities that may limit their availability to us.

As we seek to advance development of AV-101 for MDD and other CNS-related conditions, as well as stem cell technology-related drug rescue and RM programs, we will need to expand our research and development capabilities and/or contract with third parties to provide these capabilities for us. As our operations expand, we expect that we will need to manage additional relationships with various strategic partners and other third parties. Future growth will impose significant added responsibilities on members of management. Our future financial performance and our ability to develop and commercialize our product candidates and to compete effectively will depend, in part, on our ability to manage any future growth effectively. To that end, we must be able to manage our research and

development efforts effectively and hire, train and integrate additional management, administrative and technical personnel. The hiring, training and integration of new employees may be more difficult, costly and/or time-consuming for us because we have fewer resources than a larger organization. We may not be able to accomplish these tasks, and our failure to accomplish any of them could prevent us from successfully growing the company.

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

If we develop AV-101, drug rescue NCEs, other product candidates, or regenerative medicine product candidates, either on our own or in collaboration with others, we will face inherent risks of product liability as a result of the required clinical testing of such product candidates, and will face an even greater risk if we or our collaborators commercialize any such product candidates. For example, we may be sued if AV-101, any drug rescue NCE, other product candidate, or regenerative medicine product candidate we develop allegedly causes injury or is found to be otherwise unsuitable during product testing, manufacturing, marketing or sale. Any such product liability claims may include allegations of defects in manufacturing, defects in design, a failure to warn of dangers inherent in the product, negligence, strict liability, and a breach of warranties. Claims could also be asserted under state consumer protection acts. If we cannot successfully defend ourselves against product liability claims, we may incur substantial liabilities or be required to limit commercialization of our product candidates. Even successful defense would require significant financial and management resources. Regardless of the merits or eventual outcome, liability claims may result in:

decreased demand for products that we may develop;

injury to our reputation;

withdrawal of clinical trial participants;

costs to defend the related litigation;

a diversion of management's time and our resources;

substantial monetary awards to trial participants or patients; or

product recalls, withdrawals or labeling, marketing or promotional restrictions.

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

As a public company, we incur significant administrative workload and expenses to comply with U.S. regulations and requirements imposed by the NASDAQ Stock Market concerning corporate governance and public disclosure.

As a public company with common stock listed on the NASDAQ Capital Market, we must comply with various laws, regulations and requirements, including certain provisions of the Sarbanes-Oxley Act of 2002, as well as rules implemented by the SEC and the NASDAQ Stock Market. Complying with these statutes, regulations and requirements, including our public company reporting requirements, continues to occupy a significant amount of the time of management and involves significant accounting, legal and other expenses. Furthermore, these laws, regulations and requirements require us to observe greater corporate governance practices than we have employed in the past, including, but not limited to maintaining a sufficient number of independent directors, increased frequency of board meetings, and holding annual stockholder meetings. Our efforts to comply with these regulations are likely to result in increased general and administrative expenses and management time and attention directed to compliance activities.

Unfavorable global economic or political conditions could adversely affect our business, financial condition or results of operations.

Our results of operations could be adversely affected by global political conditions, as well as general conditions in the global economy and in the global financial and stock markets. Global financial and political crises cause extreme volatility and disruptions in the capital and credit markets. A severe or prolonged economic downturn, such as the recent global financial crisis, could result in a variety of risks to our business, including, weakened demand for our product candidates and our ability to raise additional capital when needed on acceptable terms, if at all. A weak or declining economy could also strain our suppliers, possibly resulting in supply disruption, or cause our customers to delay making payments for our services. Any of the foregoing could harm our business and we cannot anticipate all of the ways in which the current economic climate and financial market conditions could adversely impact our business.

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We or the third parties upon whom we depend may be adversely affected by natural disasters and our business continuity and disaster recovery plans may not adequately protect us from a serious disaster.

Natural disasters could severely disrupt our operations, and have a material adverse effect on our business, results of operations, financial condition and prospects. If a natural disaster, power outage or other event occurred that prevented us from using all or a significant portion of our headquarters, that damaged critical infrastructure, such as the manufacturing facilities of our third-party CMOs, or that otherwise disrupted operations, it may be difficult or, in certain cases, impossible for us to continue our business for a substantial period of time. The disaster recovery and business continuity plans we have in place may prove inadequate in the event of a serious disaster or similar event. We may incur substantial expenses as a result of the limited nature of our disaster recovery and business continuity plans, which could have a material adverse effect on our business.

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

Despite the implementation of security measures, our internal computer systems and those of our third-party CROs and other contractors and consultants are vulnerable to damage from computer viruses, unauthorized access, natural disasters, terrorism, war and telecommunication and electrical failures. While we have not experienced any such system failure, accident, or security breach to date, if such an event were to occur and cause interruptions in our operations, it could result in a material disruption of our programs. For example, the loss of clinical trial data for AV-101 or other product candidates could result in delays in our regulatory approval efforts and significantly increase our costs to recover or reproduce the data. To the extent that any disruption or security breach results in a loss of or damage to our data or applications or other data or applications relating to our technology or product candidates, or inappropriate disclosure of confidential or proprietary information, we could incur liabilities and the further development of our product candidates could be delayed.

We may acquire businesses or products, or form strategic alliances, in the future, and we may not realize the benefits of such acquisitions.

We may acquire additional businesses or products, form strategic alliances or create joint ventures with third parties that we believe will complement or augment our existing business. If we acquire businesses with promising markets or technologies, we may not be able to realize the benefit of acquiring such businesses if we are unable to successfully integrate them with our existing operations and company culture. We may encounter numerous difficulties in developing, manufacturing and marketing any new products resulting from a strategic alliance or acquisition that delay or prevent us from realizing their expected benefits or enhancing our business. We cannot assure you that, following any such acquisition, we will achieve the expected synergies to justify the transaction.

Risks Related to Our Intellectual Property Rights

If we are unable to adequately protect our proprietary technology, or obtain and maintain issued patents that are sufficient to protect our product candidates, others could compete against us more directly, which would have a material adverse impact on our business, results of operations, financial condition and prospects.

We strive to protect and enhance the proprietary technologies that we believe are important to our business, including seeking patents intended to cover our products and compositions, their methods of use and any other inventions we consider important to the development of our business. We also rely on trade secrets to protect aspects of our business that are not amenable to, or that we do not consider appropriate for, patent protection.

Our success will depend significantly on our ability to obtain and maintain patent and other proprietary protection for commercially important technology, inventions and know-how related to our business, to defend and enforce our patents, should they issue, to preserve the confidentiality of our trade secrets and to operate without infringing the valid and enforceable patents and proprietary rights of third parties. We also rely on know-how, continuing technological innovation and in-licensing opportunities to develop, strengthen and maintain the proprietary position of our product candidates. We own patent applications related to AV-101 and we own and have licensed patents and patent applications related to human pluripotent stem cell technology.

Although we have an issued patent relating to AV-101 in the U.S. and the European Union, we cannot yet provide any assurances that any of our other numerous pending U.S. and additional foreign patent applications relating to AV-101 will mature into issued patents and, if they do, that such patents will include claims with a scope sufficient to protect AV-101 or otherwise provide any competitive advantage. Moreover, other parties may have developed technologies that may be related or competitive to our approach, and may have filed or may file patent applications and may have received or may receive patents that may overlap or conflict with our patent applications, either by claiming the same methods or formulations or by claiming subject matter that could dominate our patent position. Such third-party patent positions may limit or even eliminate our ability to obtain patent protection.

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The patent positions of biotechnology and pharmaceutical companies, including our patent position, involve complex legal and factual questions, and, therefore, the issuance, scope, validity and enforceability of any additional patent claims that we may obtain cannot be predicted with certainty. Patents, if issued, may be challenged, deemed unenforceable, invalidated, or circumvented. U.S. patents and patent applications may also be subject to interference proceedings, ex parte reexamination, or inter partes review proceedings, supplemental examination and challenges in district court. Patents may be subjected to opposition, post-grant review, or comparable proceedings lodged in various foreign, both national and regional, patent offices. These proceedings could result in either loss of the patent or denial of the patent application or loss or reduction in the scope of one or more of the claims of the patent or patent application. In addition, such proceedings may be costly. Thus, any patents that we may own or exclusively license may not provide any protection against competitors. Furthermore, an adverse decision in an interference proceeding can result in a third party receiving the patent right sought by us, which in turn could affect our ability to develop, market or otherwise commercialize our product candidates.

Furthermore, though a patent is presumed valid and enforceable, its issuance is not conclusive as to its validity or its enforceability and it may not provide us with adequate proprietary protection or competitive advantages against competitors with similar products. Even if a patent issues and is held to be valid and enforceable, competitors may be able to design around our patents, such as using pre-existing or newly developed technology. Other parties may develop and obtain patent protection for more effective technologies, designs or methods. We may not be able to prevent the unauthorized disclosure or use of our technical knowledge or trade secrets by consultants, vendors, former employees and current employees. The laws of some foreign countries do not protect our proprietary rights to the same extent as the laws of the United States, and we may encounter significant problems in protecting our proprietary rights in these countries. If these developments were to occur, they could have a material adverse effect on our sales.

Our ability to enforce our patent rights depends on our ability to detect infringement. It is difficult to detect infringers who do not advertise the components that are used in their products. Moreover, it may be difficult or impossible to obtain evidence of infringement in a competitor's or potential competitor's product. Any litigation to enforce or defend our patent rights, even if we were to prevail, could be costly and time-consuming and would divert the attention of our management and key personnel from our business operations. We may not prevail in any lawsuits that we initiate and the damages or other remedies awarded if we were to prevail may not be commercially meaningful.

In addition, proceedings to enforce or defend our patents could put our patents at risk of being invalidated, held unenforceable, or interpreted narrowly. Such proceedings could also provoke third parties to assert claims against us, including that some or all of the claims in one or more of our patents are invalid or otherwise unenforceable. If any patents covering our product candidates are invalidated or found unenforceable, our financial position and results of operations would be materially and adversely impacted. In addition, if a court found that valid, enforceable patents held by third parties covered our product candidates, our financial position and results of operations would also be materially and adversely impacted.

The degree of future protection for our proprietary rights is uncertain, and we cannot ensure that:

any of our AV-101 or other pending patent applications, if issued, will include claims having a scope sufficient to protect AV-101 or any other products or product candidates, particularly considering that the compound patent to AV-101 has expired;

any of our pending patent applications will issue as patents at all;

we will be able to successfully commercialize our product candidates, if approved, before our relevant patents expire;

we were the first to make the inventions covered by each of our patents and pending patent applications;

we were the first to file patent applications for these inventions;

others will not develop similar or alternative technologies that do not infringe our patents;

others will not use pre-existing technology to effectively compete against us;

any of our patents, if issued, will be found to ultimately be valid and enforceable;

any patents issued to us will provide a basis for an exclusive market for our commercially viable products, will provide us with any competitive advantages or will not be challenged by third parties;

we will develop additional proprietary technologies or product candidates that are separately patentable; or

that our commercial activities or products will not infringe upon the patents or proprietary rights of others.

We also rely upon unpatented trade secrets, unpatented know-how and continuing technological innovation to develop and maintain our competitive position, which we seek to protect, in part, by confidentiality agreements with our employees and our collaborators and consultants. It is possible that technology relevant to our business will be independently developed by a person that is not a party to such an agreement. Furthermore, if the employees and consultants who are parties to these agreements breach or violate the terms of these agreements, we may not have adequate remedies for any such breach or violation, and we could lose our trade secrets through such breaches or violations. Further, our trade secrets could otherwise become known or be independently discovered by our competitors.

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We may infringe the intellectual property rights of others, which may prevent or delay our product development efforts and stop us from commercializing or increase the costs of commercializing our product candidates, if approved.

Our success will depend in part on our ability to operate without infringing the intellectual property and proprietary rights of third parties. We cannot assure you that our business, products and methods do not or will not infringe the patents or other intellectual property rights of third parties.

The pharmaceutical industry is characterized by extensive litigation regarding patents and other intellectual property rights. Other parties may allege that our product candidates or the use of our technologies infringes patent claims or other intellectual property rights held by them or that we are employing their proprietary technology without authorization. As we continue to develop and, if approved, commercialize our current product candidates and future product candidates, competitors may claim that our technology infringes their intellectual property rights as part of business strategies designed to impede our successful commercialization. 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 our product candidates. Because patent applications can take many years to issue, third parties may have currently pending patent applications that may later result in issued patents that our product candidates may infringe, or which such third parties claim are infringed by our technologies. The outcome of intellectual property litigation is subject to uncertainties that cannot be adequately quantified in advance. The coverage of patents is subject to interpretation by the courts, and the interpretation is not always uniform. If we are sued for patent infringement, we would need to demonstrate that our product candidates, products or methods either do not infringe the patent claims of the relevant patent or that the patent claims are invalid, and we may not be able to do this. Even if we are successful in these proceedings, we may incur substantial costs and the time and attention of our management and scientific personnel could be diverted in pursuing these proceedings, which could have a material adverse effect on us. In addition, we may not have sufficient resources to bring these actions to a successful conclusion.

Patent and other types of intellectual property litigation can involve complex factual and legal questions, and their outcome is uncertain. Any claim relating to intellectual property infringement that is successfully asserted against us may require us to pay substantial damages, including treble damages and attorney's fees if we are found to be willfully infringing another party's patents, for past use of the asserted intellectual property and royalties and other consideration going forward if we are forced to take a license. In addition, if any such claim was successfully asserted against us and we could not obtain such a license, we may be forced to stop or delay developing, manufacturing, selling or otherwise commercializing our product candidates.

Even if we are successful in these proceedings, we may incur substantial costs and divert management time and attention in pursuing these proceedings, which could have a material adverse effect on us. If we are unable to avoid infringing the patent rights of others, we may be required to seek a license, defend an infringement action or challenge the validity of the patents in court, or redesign our products. Patent litigation is costly and time-consuming. We may not have sufficient resources to bring these actions to a successful conclusion. In addition, intellectual property litigation or claims could force us to do one or more of the following:

cease developing, selling or otherwise commercializing our product candidates;

pay substantial damages for past use of the asserted intellectual property;

obtain a license from the holder of the asserted intellectual property, which license may not be available on reasonable terms, if at all; and

in the case of trademark claims, redesign, or rename, some or all of our product candidates to avoid infringing the intellectual property rights of third parties, which may not be possible and, even if possible, could be costly and time-consuming.

Any of these risks coming to fruition could have a material adverse effect on our business, results of operations, financial condition and prospects.

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

We enter into confidentiality and intellectual property assignment agreements with our employees, consultants, outside scientific collaborators, sponsored researchers and other advisors. These agreements generally provide that inventions conceived by the party in the course of rendering services to us will be our exclusive property. However, these agreements may not be honored and may not effectively assign intellectual property rights to us. For example, even if we have a consulting agreement in place with an academic advisor pursuant to which such academic advisor is required to assign any inventions developed in connection with providing services to us, such academic advisor may not have the right to assign such inventions to us, as it may conflict with his or her obligations to assign all such intellectual property to his or her employing institution.

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Litigation may be necessary to defend against these and other claims challenging inventorship or ownership. If we 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, valuable intellectual property. Such an outcome could have a material adverse effect on 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.

The U.S. Patent and Trademark Office (USPTO), European Patent Office (EPO) and various other foreign governmental patent agencies require compliance with a number of procedural, documentary, fee payment and other provisions during the patent process. There are situations in which noncompliance can result in abandonment or lapse of a patent or patent application, resulting in partial or complete loss of patent rights in the relevant jurisdiction. In such an event, competitors might be able to enter the market earlier than would otherwise have been the case.

Third parties may initiate legal proceedings against us alleging that we infringe their intellectual property rights or we may initiate legal proceedings against third parties to challenge the validity or scope of intellectual property rights controlled by third parties, the outcome of which would be uncertain and could have a material adverse effect on the success of our business. Any lawsuit we are engaged in to protect or enforce our patents or the patents of our licensors could be expensive, time-consuming and unsuccessful.

Even if the patent applications we own or license are issued, competitors may infringe these patents. To counter infringement or unauthorized use, we may be required to file infringement claims, which can be expensive and time-consuming. In addition, in an infringement proceeding, a court may decide that a patent of ours or our licensors is not valid, is unenforceable and/or is not infringed, or may refuse to stop the other party from using the technology at issue on the grounds that our patents do not cover the technology in question. An adverse result in any litigation or defense proceedings could put one or more of our patents at risk of being invalidated or interpreted narrowly and could put our patent applications at risk of not issuing.

Further, third parties may initiate legal proceedings against us or our licensors or collaborators alleging that we or our licensors or collaborators infringe their intellectual property rights or we or our licensors or collaborators may initiate legal proceedings against third parties to challenge the validity or scope of intellectual property rights controlled by third parties, including in oppositions, interferences, reexaminations, inter partes reviews or derivation proceedings before the United States or other jurisdictions. These proceedings can be expensive and time-consuming and many of our or our licensors' or collaborators' adversaries in these proceedings may have the ability to dedicate substantially greater resources to prosecuting these legal actions than we or our licensors or collaborators can. Our defense of litigation or interference proceedings may fail and, even if successful, may result in substantial costs and distract our management and other employees. We may not be able to prevent, alone or with our licensors, misappropriation of our intellectual property rights, particularly in countries where the laws may not protect those rights as fully as in the United States or European Union.

An unfavorable outcome could require us or our licensors or collaborators to cease using the related technology or developing or commercializing our product candidates, 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 or our licensors or collaborators a license on commercially reasonable terms or at all. Even if we or our licensors or collaborators obtain a license, it may be non-exclusive, thereby giving our competitors access to the same technologies licensed to us or our licensors or collaborators. In addition, we could be found liable for monetary damages, including treble damages and attorneys'

fees, if we are found to have willfully infringed a patent. A finding of infringement could prevent us from commercializing our product candidates or force us to cease some of our business operations, which could materially harm our business.

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.

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

If we or one of our licensing partners initiated legal proceedings against a third party to enforce a patent, if and when issued, covering one of our product candidates, 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 include alleged failures to meet any of several statutory requirements, including lack of novelty, obviousness or non-enablement. Grounds for unenforceability assertions include allegations that someone connected with prosecution of the patent withheld relevant information from the USPTO or EPO, 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 of our patents in such a way that they no longer cover our product candidates or competitive products. The outcome following legal assertions of invalidity and unenforceability is unpredictable. With respect to validity, 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.

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We will not seek to protect our intellectual property rights in all jurisdictions throughout the world and we may not be able to adequately enforce our intellectual property rights even in the jurisdictions where we seek protection.

Filing, prosecuting and defending patents on product candidates in all countries and jurisdictions throughout the world is prohibitively expensive, and our intellectual property rights in some countries outside the United States could be less extensive than those in the United States, assuming that rights are obtained 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. 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. The statutory deadlines for pursuing patent protection in individual foreign jurisdictions are based on the priority date of each of our patent applications. For the patent applications relating to AV-101, as well as for many of the patent families that we own or license, the relevant statutory deadlines have not yet expired. Thus, for each of the patent families that we believe provide coverage for our lead product candidates or technologies, we will need to decide whether and where to pursue protection outside the United States.

Competitors may use our technologies in jurisdictions where we do not pursue and obtain patent protection to develop their own products and further, may export otherwise 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. Even if we pursue and obtain issued patents in particular jurisdictions, our patent claims or other intellectual property rights may not be effective or sufficient to prevent third parties from so competing.

The laws of some foreign countries do not protect intellectual property rights to the same extent as the laws of the United States. Many companies have encountered significant problems in protecting and defending intellectual property rights in certain foreign jurisdictions. The legal systems of some countries, particularly developing countries, do not favor the enforcement of patents and other intellectual property protection, especially those relating to biotechnology. This could make it difficult for us to stop the infringement of our patents, if obtained, or the misappropriation of our other intellectual property rights. For example, many foreign countries have compulsory licensing laws under which a patent owner must grant licenses to third parties. In addition, many countries limit the enforceability of patents against third parties, including government agencies or government contractors. In these countries, patents may provide limited or no benefit. Patent protection must ultimately be sought on a country-by-country basis, which is an expensive and time-consuming process with uncertain outcomes. Accordingly, we may choose not to seek patent protection in certain countries, and we will not have the benefit of patent protection in such countries.

An unfavorable outcome could require us or our licensors or collaborators to cease using the related technology or developing or commercializing our product candidates, 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 or our licensors or collaborators a license on commercially reasonable terms or at all. Even if we or our licensors or collaborators obtain a license, it may be non-exclusive, thereby giving our competitors access to the same technologies licensed to us or our licensors or collaborators. In addition, we could be found liable for monetary damages, including treble damages and attorneys' fees, if we are found to have willfully infringed a patent. A finding of infringement could prevent us from commercializing our product candidates or force us to cease some of our business operations, which could materially harm our business.

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.

Furthermore, proceedings to enforce our patent rights in foreign jurisdictions 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, could put 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.

We are dependent, in part, on licensed intellectual property. If we were to lose our rights to licensed intellectual property, we may not be able to continue developing or commercializing our product candidates, if approved. If we breach any of the agreements under which we license the use, development and commercialization rights to our product candidates or technology from third parties or, in certain cases, we fail to meet certain development or payment deadlines, we could lose license rights that are important to our business.

We are a party to a number of license agreements under which we are granted rights to intellectual property that are or could become important to our business, and we expect that we may need to enter into additional license agreements in the future. Our existing license agreements impose, and we expect that future license agreements will impose on us, various development, regulatory and/or commercial diligence obligations, payment of fees, milestones and/or royalties and other obligations. If we fail to comply with our obligations under these agreements, or we are subject to a bankruptcy, the licensor may have the right to terminate the license, in which event we would not be able to develop or market products, which could be covered by the license. Our business could suffer, for example, if any current or future licenses terminate, if the licensors fail to abide by the terms of the license, if the licensed patents or other rights are found to be invalid or unenforceable, or if we are unable to enter into necessary licenses on acceptable terms. See “Business —Intellectual Property” herein for a description of our license agreements, which includes a description of the termination provisions of these agreements.

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As we have done previously, we may need to obtain licenses from third parties to advance our research or allow commercialization of our product candidates, and we cannot provide any assurances that third-party patents do not exist that might be enforced against our current product candidates or future products in the absence of such a license. We may fail to obtain any of these licenses on commercially reasonable terms, if at all. Even if we are able to obtain a license, it may be non-exclusive, thereby giving our competitors access to the same technologies licensed to us. In that event, we may be required to expend significant time and resources to develop or license replacement technology. If we are unable to do so, we may be unable to develop or commercialize the affected product candidates, which could materially harm our business and the third parties owning such intellectual property rights could seek either an injunction prohibiting our sales, or, with respect to our sales, an obligation on our part to pay royalties and/or other forms of compensation.

Licensing of intellectual property is of critical importance to our business and involves complex legal, business and scientific issues. Disputes may arise between us and our licensors regarding intellectual property subject to a license agreement, including:

the scope of rights granted under the license agreement and other interpretation-related issues;

whether and the extent to which our technology and processes infringe on intellectual property of the licensor that is not subject to the licensing agreement;

our right to sublicense patent and other rights to third parties under collaborative development relationships;

our diligence obligations with respect to the use of the licensed technology in relation to our development and commercialization of our product candidates, and what activities satisfy those diligence obligations; and

the ownership of inventions and know-how resulting from the joint creation or use of intellectual property by our licensors and us and our partners.

If disputes over intellectual property that we have licensed prevent or impair our ability to maintain our current licensing arrangements on acceptable terms, we may be unable to successfully develop and commercialize the affected product candidates.

We have entered into several licenses to support our various stem cell technology-related programs. We may enter into additional license(s) to third-party intellectual property that are necessary or useful to our business. Our current licenses and any future licenses that we may enter into impose various royalty payments, milestone, and other obligations on us. For example, the licensor may retain control over patent prosecution and maintenance under a license agreement, in which case, we may not be able to adequately influence patent prosecution or prevent inadvertent lapses of coverage due to failure to pay maintenance fees. If we fail to comply with any of our obligations under a current or future license agreement, our licensor(s) may allege that we have breached our license agreement and may accordingly seek to terminate our license with them. In addition, future licensor(s) may decide to terminate our license at will. Termination of any of our current or future licenses could result in our loss of the right to use the licensed intellectual property, which could materially adversely affect our ability to develop and commercialize a product candidate or product, if approved, as well as harm our competitive business position and our business

prospects.

In addition, if our licensors fail to abide by the terms of the license, if the licensors fail to prevent infringement by third parties, if the licensed patents or other rights are found to be invalid or unenforceable, or if we are unable to enter into necessary licenses on acceptable terms our business could suffer.

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Some intellectual property which we have licensed may have been discovered through government funded programs and thus may be subject to federal regulations such as “march-in” rights, certain reporting requirements, and a preference for U.S. industry. Compliance with such regulations may limit our exclusive rights, subject us to expenditure of resources with respect to reporting requirements, and limit our ability to contract with non-U.S. manufacturers.

Some of the intellectual property rights we have licensed or license in the future may have been generated through the use of U.S. government funding and may therefore be subject to certain federal regulations. As a result, the U.S. government may have certain rights to intellectual property embodied in our current or future product candidates pursuant to the Bayh-Dole Act of 1980 (Bayh-Dole Act). These U.S. government rights in certain inventions developed under a government-funded program include a non-exclusive, non-transferable, irrevocable worldwide license to use inventions for any governmental purpose. In addition, the U.S. government has the right to require us to grant exclusive, partially exclusive, or non-exclusive licenses to any of these inventions to a third party if it determines that: (i) adequate steps have not been taken to commercialize the invention; (ii) government action is necessary to meet public health or safety needs; or (iii) government action is necessary to meet requirements for public use under federal regulations (also referred to as “march-in rights”). The U.S. government also has the right to take title to these inventions if we fail, or the applicable licensor fails, to disclose the invention to the government and fail to file an application to register the intellectual property within specified time limits. In addition, the U.S. government may acquire title to these inventions in any country in which a patent application is not filed within specified time limits. Intellectual property generated under a government funded program is also subject to certain reporting requirements, compliance with which may require us, or the applicable licensor, to expend substantial resources. In addition, the U.S. government requires that any products embodying the subject invention or produced through the use of the subject invention be manufactured substantially in the U.S. The manufacturing preference requirement can be waived if the owner of the intellectual property can show that reasonable but unsuccessful efforts have been made to grant licenses on similar terms to potential licensees that would be likely to manufacture substantially in the U.S. or that under the circumstances domestic manufacture is not commercially feasible. This preference for U.S. manufacturers may limit our ability to contract with non-U.S. product manufacturers for products covered by such intellectual property.

In the event we apply for additional U.S. government funding, and we discover compounds or drug candidates as a result of such funding, intellectual property rights to such discoveries may be subject to the applicable provisions of the Bayh-Dole Act.

If we do not obtain additional protection under the Hatch-Waxman Amendments and similar foreign legislation by extending the patent terms and obtaining data exclusivity for our product candidates, our business may be materially harmed.

Depending upon the timing, duration and specifics of FDA marketing approval of our product candidates, one or more of the U.S. patents we own or license may be eligible for limited patent term restoration under the Drug Price Competition and Patent Term Restoration Act of 1984, referred to as the Hatch-Waxman Amendments. The Hatch-Waxman Amendments permit a patent restoration term of up to five years as compensation for patent term lost during product development and the FDA regulatory review process. However, we may not be granted an extension because of, for example, failing to apply within applicable deadlines, failing to apply prior to expiration of relevant patents or otherwise failing to satisfy applicable requirements. For example, we may not be granted an extension if the active ingredient of AV-101 is used in another drug company’s product candidate and that product candidate is the first to obtain FDA approval. Moreover, the applicable time period or the scope of patent protection afforded 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, our competitors may obtain approval of competing products following our patent expiration, and

our ability to generate revenues could be materially adversely affected.

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

As is the case with other biotechnology companies, our success is heavily dependent on intellectual property, particularly patents. Obtaining and enforcing patents in the biotechnology industry involve both technological and legal complexity, and is therefore costly, time-consuming and inherently uncertain. In addition, the United States has recently enacted and is currently implementing wide-ranging patent reform legislation: the Leahy-Smith America Invents Act, referred to as the America Invents Act. The America Invents Act includes a number of significant changes to U.S. patent law. These include provisions that affect the way patent applications will be prosecuted and may also affect patent litigation. It is not yet clear what, if any, impact the America Invents Act will have on the operation of our business. However, the America Invents Act and its implementation could increase the uncertainties and costs surrounding the prosecution of our patent applications and the enforcement or defense of any patents that may issue from our patent applications, all of which could have a material adverse effect on our business and financial condition.

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In addition, 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. The full impact of these decisions is not yet known. For example, on March 20, 2012 in *Mayo Collaborative Services, DBA Mayo Medical Laboratories, et al. v. Prometheus Laboratories, Inc.*, the Court held that several claims drawn to measuring drug metabolite levels from patient samples and correlating them to drug doses were not patentable subject matter. The decision appears to impact diagnostics patents that merely apply a law of nature via a series of routine steps and it has created uncertainty around the ability to obtain patent protection for certain inventions. Additionally, on June 13, 2013 in *Association for Molecular Pathology v. Myriad Genetics, Inc.*, the Court held that claims to isolated genomic DNA are not patentable, but claims to complementary DNA molecules are patent eligible because they are not a natural product. The effect of the decision on patents for other isolated natural products is uncertain. Additionally, on March 4, 2014, the USPTO issued a memorandum to patent examiners providing guidance for examining claims that recite laws of nature, natural phenomena or natural products under the *Myriad* and *Prometheus* decisions. This guidance did not limit the application of *Myriad* to DNA but, rather, applied the decision to other natural products. Further, in 2015, in *Ariosa Diagnostics, Inc. v. Sequenom, Inc.*, the Court of Appeals for the Federal Circuit held that methods for detecting fetal genetic defects were not patent eligible subject matter.

In addition to increasing uncertainty with regard to our ability to obtain future patents, this combination of events has created uncertainty with respect to the value of patents, once obtained. Depending on these and other decisions by the U.S. Congress, the federal courts and the USPTO, the laws and regulations governing patents could change in unpredictable ways that would weaken our ability to obtain new patents or to enforce any patents that may issue in the future.

We may be subject to damages resulting from claims that we or our employees have wrongfully used or disclosed alleged trade secrets of their former employers.

Certain of our current employees have been, and certain of our future employees may have been, previously employed at other biotechnology or pharmaceutical companies, including our competitors or potential competitors. We also engage advisors and consultants who are concurrently employed at universities or who perform services for other entities.

Although we are not aware of any claims currently pending or threatened against us, we may be subject to claims that we or our employees, advisors or consultants have inadvertently or otherwise used or disclosed intellectual property, including trade secrets or other proprietary information, of a former employer or other third party. We have and may in the future also be subject to claims that an employee, advisor or consultant performed work for us that conflicts with that person's obligations to a third party, such as an employer, and thus, that the third party has an ownership interest in the intellectual property arising out of work performed for us. Litigation may be necessary to defend against these claims. Even if we are successful in defending against these claims, litigation could result in substantial costs and be a distraction to management. If we fail in defending such claims, in addition to paying monetary claims, we may lose valuable intellectual property rights or personnel. A loss of key personnel or their work product could hamper or prevent our ability to commercialize our product candidates, which would materially adversely affect our commercial development efforts.

Numerous factors may limit any potential competitive advantage provided by our intellectual property rights.

The degree of future protection afforded by our intellectual property rights is uncertain because intellectual property rights have limitations, and may not adequately protect our business, provide a barrier to entry against our competitors or potential competitors, or permit us to maintain our competitive advantage. Moreover, if a third party has intellectual property rights that cover the practice of our technology, we may not be able to fully exercise or extract value from

our intellectual property rights. The following examples are illustrative:

others may be able to develop and/or practice technology that is similar to our technology or aspects of our technology but that is not covered by the claims of patents, should such patents issue from our patent applications;

we might not have been the first to make the inventions covered by a pending patent application that we own;

we might not have been the first to file patent applications covering an invention;

others may independently develop similar or alternative technologies without infringing our intellectual property rights;

pending patent applications that we own or license may not lead to issued patents;

patents, if issued, that we own or license may not provide us with any competitive advantages, or may be held invalid or unenforceable, as a result of legal challenges by our competitors;

third parties may compete with us in jurisdictions where we do not pursue and obtain patent protection;

we may not be able to obtain and/or maintain necessary or useful licenses on reasonable terms or at all; and

the patents of others may have an adverse effect on our business.

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Should any of these events occur, they could significantly harm our business and results of operations.

If, instead of identifying drug rescue candidates based on information available to us in the public domain, we seek to in-license drug rescue candidates from biotechnology, medicinal chemistry and pharmaceutical companies, academic, governmental and nonprofit research institutions, including the NIH, or other third parties, there can be no assurances that we will obtain material ownership or economic participation rights over intellectual property we may derive from such licenses or similar rights to the drug rescue NCEs we may produce and develop. If we are unable to obtain ownership or substantial economic participation rights over intellectual property related to drug rescue NCEs we produce and develop, our business may be adversely affected.

Risks Related to our Securities

The limited public market for our securities may adversely affect an investor's ability to liquidate an investment in the Company.

Our common stock is currently quoted on the NASDAQ Capital Market, however, there is presently limited trading activity. We can give no assurance that an active market will develop, or if developed, that it will be sustained. If an investor acquires shares of our common stock, the investor may not be able to liquidate the shares should there be a need or desire to do so.

Market volatility may affect our stock price and the value of your investment.

The market price for our common stock, similar to other biopharmaceutical companies, is likely to be highly volatile. The market price of our common stock may fluctuate significantly in response to a number of factors, most of which we cannot control, including, among others:

plans for, progress of or results from non-clinical and clinical development activities related to our product candidates;

the failure of the FDA to approve our product candidates;

announcements of new products, technologies, commercial relationships, acquisitions or other events by us or our competitors;

the success or failure of other CNS therapies;

regulatory or legal developments in the United States and other countries;

failure of our product candidates, if approved, to achieve commercial success;

fluctuations in stock market prices and trading volumes of similar companies;

general market conditions and overall fluctuations in U.S. equity markets;

variations in our quarterly operating results;

changes in our financial guidance or securities analysts' estimates of our financial performance;

changes in accounting principles;

our ability to raise additional capital and the terms on which we can raise it;

sales of large blocks of our common stock, including sales by our executive officers, directors and significant stockholders;

additions or departures of key personnel;

discussion of us or our stock price by the press and by online investor communities; and

other risks and uncertainties described in these risk factors.

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Future sales and issuances of our common stock may cause our stock price to decline.

Sales or issuances of a substantial number of shares of our common stock in the public market, or the perception that such sales or issuances are occurring or might occur, could significantly reduce the market price of our common stock and impair our ability to raise adequate capital through the sale of additional equity securities.

The stock market in general, and small biopharmaceutical companies like ours in particular, have frequently experienced significant volatility in the market prices for securities that often has been unrelated to the operating performance of the underlying companies. These broad market and industry fluctuations may adversely affect the market price of our common stock, regardless of our actual operating performance. In certain recent situations in which the market price of a stock has been volatile, holders of that stock have instituted securities class action litigation against such company that issued the stock. If any of our stockholders were to bring a lawsuit against us, the defense and disposition of the lawsuit could be costly and divert the time and attention of our management and harm our operating results. Additionally, if the trading volume of our common stock remains low and limited there will be an increased level of volatility and you may not be able to generate a return on your investment.

A significant portion of our total outstanding shares are restricted from immediate resale but may be sold into the market in the near future. Future sales of shares by existing stockholders could cause our stock price to decline, even if our business is doing well.

Sales of a substantial number of shares of our common stock in the public market could occur at any time. These sales, or the perception in the market that the holders of a large number of shares intend to sell shares, could reduce the market price of our common stock. Historically, there has been a limited public market for shares of our common stock. Future sales and issuances of a substantial number of shares of our common stock in the public market, including shares issued upon the conversion of our Series A Preferred, Series B Preferred or Series C Preferred, and the exercise of outstanding options and warrants for common stock which are issuable upon exercise, in the public market, or the perception that these sales and issuances are occurring or might occur, could significantly reduce the market price for our common stock and impair our ability to raise adequate capital through the sale of equity securities.

Our principal institutional stockholders may continue to have substantial control over us and could limit your ability to influence the outcome of key transactions, including changes in control.

Certain of our current institutional stockholders own a substantial portion of our outstanding capital stock, including our common stock, Series A Preferred, Series B Preferred, and Series C Preferred, all of which preferred stock is convertible into a substantial number of shares of common stock. Accordingly, institutional stockholders may exert significant influence over us and over the outcome of any corporate actions requiring approval of holders of our common stock, including the election of directors and amendments to our organizational documents, such as increases in our authorized shares of common stock, any merger, consolidation or sale of all or substantially all of our assets or any other significant corporate transactions. These stockholders may also delay or prevent a change of control of us, even if such a change of control would benefit our other stockholders. The significant concentration of stock ownership may adversely affect the trading price of our common stock due to investors' perception that conflicts of interest may exist or arise. Furthermore, the interests of our principal institutional stockholders may not always coincide with your interests or the interests of other stockholders 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, which might affect the prevailing market price for our common stock.

If equity research analysts do not publish research or reports about our business or if they issue unfavorable commentary or downgrade our common stock, the price of our common stock could decline.

The trading market for our common stock relies in part on the research and reports that equity research analysts publish about us and our business. We do not control these analysts. The price of our common stock could decline if one or more equity research analysts downgrade our common stock or if such analysts issue other unfavorable commentary or cease publishing reports about us or our business.

There may be additional issuances of shares of preferred stock in the future.

Our Restated Articles of Incorporation (the Articles) permit us to issue up to 10.0 million shares of preferred stock. Our Board has authorized the issuance of (i) 500,000 shares of Series A Preferred, all of which shares are issued and outstanding at September 30, 2017; (ii) 4.0 million shares of Series B 10% Convertible Preferred stock, of which approximately 1.2 million shares remain issued and outstanding at September 30, 2017; and (iii) 3.0 million shares of Series C Convertible Preferred Stock, of which approximately 2.3 million shares are issued and outstanding at September 30, 2017. Our Board could authorize the issuance of additional series of preferred stock in the future and such preferred stock could grant holders preferred rights to our assets upon liquidation, the right to receive dividends before dividends would be declared to holders of our common stock, and the right to the redemption of such shares, possibly together with a premium, prior to the redemption of the common stock. In the event and to the extent that we do issue additional preferred stock in the future, the rights of holders of our common stock could be impaired thereby, including without limitation, with respect to liquidation.

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We do not intend to pay dividends on our common stock and, consequently, our stockholders' ability to achieve a return on their investment will depend on appreciation in the price of our common stock.

We have never declared or paid any cash dividend on our common stock and do not currently intend to do so in the foreseeable future. We currently anticipate that we will retain future earnings for the development, operation and expansion of our business and do not anticipate declaring or paying any cash dividends in the foreseeable future. Therefore, the success of an investment in shares of our common stock will depend upon any future appreciation in their value. There is no guarantee that shares of our common stock will appreciate in value or even maintain the price at which our stockholders purchased them.

We incur significant costs to ensure compliance with corporate governance, federal securities law and accounting requirements.

Since becoming a public company by means of a reverse merger in 2011, we have been subject to the reporting requirements of the Securities Exchange Act of 1934, as amended (Exchange Act), which requires that we file annual, quarterly and current reports with respect to our business and financial condition, and the rules and regulations implemented by the SEC, the Sarbanes-Oxley Act of 2002, the Dodd-Frank Act, and the Public Company Accounting Oversight Board, each of which imposes additional reporting and other obligations on public companies. We have incurred and will continue to incur significant costs to comply with these public company reporting requirements, including accounting and related audit costs, legal costs to comply with corporate governance requirements and other costs of operating as a public company. These legal and financial compliance costs will continue to require us to divert a significant amount of money that we could otherwise use to achieve our research and development and other strategic objectives.

The filing and internal control reporting requirements imposed by federal securities laws, rules and regulations on companies that are not "smaller reporting companies" under federal securities laws are rigorous and, once we are no longer a smaller reporting company, we may not be able to meet them, resulting in a possible decline in the price of our common stock and our inability to obtain future financing. Certain of these requirements may require us to carry out activities we have not done previously and complying with such requirements may divert management's attention from other business concerns, which could have a material adverse effect on our business, results of operations, financial condition and cash flows. Any failure to adequately comply with applicable federal securities laws, rules or regulations could subject us to fines or regulatory actions, which may materially adversely affect our business, results of operations and financial condition.

In addition, changing laws, regulations and standards relating to corporate governance and public disclosure are creating uncertainty for public companies, increasing legal and financial compliance costs and making some activities more time consuming. These 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. This could result in continuing uncertainty regarding compliance matters and higher costs necessitated by ongoing revisions to disclosure and governance practices. We will continue to invest resources to comply with evolving laws, regulations and standards, however this investment may result in increased general and administrative expenses and a diversion of management's time and attention from revenue-generating activities to compliance activities. If our efforts to comply with new laws, regulations and standards differ from the activities intended by regulatory or governing bodies due to ambiguities related to their application and practice, regulatory authorities may initiate legal proceedings against us and our business may be adversely affected.

Risks Related to this Offering

Our management team may invest or spend the proceeds of this offering in ways with which you may not agree or in ways which may not yield a significant return.

Our management will have broad discretion over the use of proceeds from this offering. We currently intend to use the net proceeds from the sale of securities offered by this prospectus for general corporate purposes, including research and development, working capital and capital expenditures. We may use a portion of the net proceeds to fund production of, and nonclinical and clinical studies related to Phase 2 and Phase 3 development of, AV-101 and other drug candidates. We may also use the net proceeds from the sale of the securities under this prospectus to in-license, acquire or invest in complementary businesses, technologies, products or assets. However, our management will have broad discretion in the application of the net proceeds from this offering and could spend the proceeds in ways that do not improve our results of operations or enhance the value of our common stock. The failure by management to apply these funds effectively could result in financial losses that could have a material adverse effect on our business, cause the price of our common stock to decline and delay the development of our product candidates.

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Purchasers in this offering will experience immediate and substantial dilution in the book value of their investment.

Because the combined public offering price per share and related warrant is substantially higher than the book value per share of our common stock, you will incur immediate and substantial dilution in the net tangible book value of the common stock you purchase in this offering. After giving effect to the assumed sale by us of 8,333,333 shares of our common stock and warrants to purchase up to 8,333,333 shares of common stock at an assumed combined public offering price of \$1.80 per share (the last reported sale price of our common stock on the NASDAQ Capital Market on December 6, 2017), and after deducting the estimated underwriting discount and estimated offering expenses payable by us, you will suffer immediate and substantial dilution of \$1.20 per share in the pro forma net tangible book value of the common stock you purchase in this offering, assuming no value is attributed to the warrants, and the warrants are accounted for and classified as equity. To the extent outstanding options, warrants or other derivative securities are ultimately exercised or converted, or if we issue restricted stock to our employees under the 2016 Plan, there will be further dilution to investors who purchase shares in this offering. In addition, if we issue additional equity securities or derivative securities, investors purchasing shares in this offering will experience additional dilution. For a further description of the dilution that you will experience immediately after this offering, see “Dilution” on page 43.

Sales of a substantial number of shares of our common stock, or the perception that such sales may occur, may adversely impact the price of our common stock.

Sales of a substantial number of shares of our common stock in the public market could occur at any time. These sales, or the perception that such sales may occur, may adversely impact the price of our common stock, even if there is no relationship between such sales and the performance of our business. As of December 6, 2017, we had 11,848,974 shares of common stock outstanding, as well as outstanding options to purchase an aggregate of 3,279,871 shares of our common stock at a weighted average exercise price of \$3.23 per share, up to 4,228,252 shares of common stock issuable upon conversion of outstanding shares of our preferred stock, and outstanding warrants to purchase up to an aggregate of 7,107,157 shares of our common stock at a weighted average exercise price of \$4.70 per share. The exercise of such outstanding derivative securities may result in further dilution of your investment.

There is no public market for the warrants being offered by this prospectus, and we do not anticipate such a market ever developing in the future.

There is no established public trading market for the warrants being offered by this prospectus and we do not intend to have the warrants listed on a national securities exchange or any other recognized trading system in the future. Without an active market, the liquidity of any warrants sold by means of this prospectus will be limited.

The warrants being offered by means of this prospectus may not have any value.

The warrants offered by us in this offering do not confer any rights of ownership of shares of common stock on its holders, such as voting rights or the right to receive dividends, but only represent the right to acquire shares of common stock at a fixed price for a limited period of time. In the event that the market price of our common stock does not exceed the exercise price of the warrants sold in this offering during the period when the warrants are exercisable, the warrants may not have any value.

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CAUTIONARY NOTES REGARDING FORWARD-LOOKING STATEMENTS

This prospectus contains forward-looking statements that involve substantial risks and uncertainties. All statements contained in this prospectus, other than statements of historical facts, are forward-looking statements including statements regarding our strategy, future operations, future financial position, future revenue, projected costs, prospects, plans, objectives of management and expected market growth. These statements involve known and unknown risks, uncertainties and other important factors that may cause our actual results, performance or achievements to be materially different from any future results, performance or achievements expressed or implied by the forward-looking statements.

The words “anticipate,” “believe,” “estimate,” “expect,” “intend,” “may,” “plan,” “predict,” “project,” “target,” “potential,” “w,” “should,” “continue,” and similar expressions are intended to identify forward-looking statements, although not all forward-looking statements contain these identifying words. These forward-looking statements include, among other things, statements about:

the availability of capital to satisfy our working capital requirements;

the accuracy of our estimates regarding expenses, future revenues and capital requirements;

our plans to develop and commercialize our lead product candidate, AV-101, initially as an adjunctive treatment for MDD in patients with an inadequate response to standard, FDA-approved antidepressants, and subsequently as a treatment for additional CNS diseases and disorders;

our ability to initiate and complete our clinical trials and to advance our product candidates into additional clinical trials, including pivotal clinical trials, and successfully complete such clinical trials;

regulatory developments in the U.S. and foreign countries;

the performance of the U.S. National Institute of Mental Health, our third-party contractors involved with the manufacturer and production of our drug candidates for nonclinical and clinical development activities, contract research organizations and other third-party nonclinical and clinical development collaborators and regulatory service providers;

our ability to obtain and maintain intellectual property protection for our core assets;

the size of the potential markets for our product candidates and our ability to serve those markets;

the rate and degree of market acceptance of our product candidates for any indication once approved;

the success of competing products and product candidates in development by others that are or become available for the indications that we are pursuing;

the loss of key scientific, clinical and nonclinical development, and/or management personnel, internally or from one of our third-party collaborators; and

other risks and uncertainties, including those described under “Risk Factors.”

These forward-looking statements are only predictions and we may not actually achieve the plans, intentions or expectations disclosed in our forward-looking statements, so you should not place undue reliance on our forward-looking statements. Actual results or events could differ materially from the plans, intentions and expectations disclosed in the forward-looking statements we make. We have based these forward-looking statements largely on our current expectations and projections about future events and trends that we believe may affect our business, financial condition and operating results. We have included important factors in the cautionary statements included in this prospectus that could cause actual future results or events to differ materially from the forward-looking statements that we make. Our forward-looking statements do not reflect the potential impact of any future acquisitions, mergers, dispositions, joint ventures or investments we may make.

You should read this prospectus with the understanding that our actual future results may be materially different from what we expect. We do not assume any obligation to update any forward-looking statements whether as a result of new information, future events or otherwise, except as required by applicable law.

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USE OF PROCEEDS

We estimate that the net proceeds to us from this offering will be approximately \$13.6 million, assuming the sale of 8,333,333 shares of common stock and warrants to purchase up to 8,333,333 shares of our common stock at an assumed combined public offering price of \$1.80 per share and related warrant (the last reported sale price of our common stock on the NASDAQ Capital Market on December 6, 2017) after deducting the estimated underwriting discount and estimated offering expenses payable by us.

Each \$0.20 increase (decrease) in the assumed combined public offering price of \$1.80 per share and related warrant would increase (decrease) the net proceeds to us from this offering by approximately \$1.6 million, assuming the number of shares of common stock and related warrants offered by us, as set forth on the cover page of this prospectus, remains the same and after deducting the estimated underwriting discount and estimated offering expenses payable by us. We may also increase or decrease the number of shares of common stock and related warrants we are offering. Each increase (decrease) of 1,000,000 shares in the number of shares of common stock and related warrants we are offering would increase (decrease) the net proceeds to us from this offering by approximately \$1.7 million, assuming that the assumed combined public offering price remains the same, and after deducting the estimated underwriting discount and estimated offering expenses payable by us. This information is illustrative only, and we will adjust this information based on the actual combined public offering price and other terms of this offering determined at pricing. We do not expect that a change in the combined public offering price or the number of shares of common stock and related warrants by these amounts would have a material effect on our intended uses of the net proceeds from this offering, although it may impact the amount of time prior to which we may need to seek additional capital.

We expect to use the net proceeds from this offering for research and development, primarily related to the AV-101 MDD Phase 2 Adjunctive Treatment Study, ordinary course working capital needs and other general corporate purposes. We may also use a portion of the net proceeds to (i) produce additional supplies of AV-101 for future nonclinical and clinical studies, (ii) conduct certain standard nonclinical studies necessary to enable longer term oral administration (initially at least 90 days) of AV-101 for MDD and multiple other potential CNS indications and to facilitate a smooth transition to Phase 3 development of AV-101 for adjunctive treatment of MDD should the results of the AV-101 MDD Phase 2 Adjunctive Treatment Study be positive and warrant further clinical development of AV-101 for MDD, and (iii) conduct a small, single dose, food effects clinical study of AV-101 in healthy volunteers (not MDD patients) to enable Phase 3 development of AV-101. We may also use a portion of the net proceeds from the sale of the securities under this prospectus to in-license, acquire or invest in complementary businesses, technologies, products or assets. The table below reflects our current planned use of the net proceeds from this offering. Each of these amounts is an estimate only, and is subject to change at any time before or after closing of the offering.

Assumed gross proceeds	\$
Underwriting discount and other offering expenses	\$
Net proceeds	\$
Research and development	\$
Working capital and other general and administrative purposes	\$

We expect net proceeds from this offering to provide sufficient funding to launch and complete our AV-101 MDD Phase 2 Adjunctive Treatment Study in 2018.

Pending other uses, we intend to invest the net proceeds from this offering in short-term investments or hold them as cash. We cannot predict whether the proceeds invested will yield a favorable return. Our management will have broad

discretion in the use of the net proceeds from this offering, and investors will be relying on the judgment of our management regarding the application of the net proceeds.

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MARKET FOR OUR COMMON STOCK

Market Information

Our common stock is listed on the NASDAQ Capital Market under the symbol “VTGN.”

Shown below is the range of high and low sales prices for our common stock for the periods indicated as reported by the NASDAQ Capital Market.

	High	Low
Fiscal Year Ending March 31, 2018		
First quarter ending June 30, 2017	\$2.40	\$1.72
Second quarter ending September 30, 2017	\$2.05	\$1.53
Third quarter ending December 31, 2017 (through December 6, 2017)	\$2.65	\$0.69
Fiscal Year Ending March 31, 2017		
First quarter ending June 30, 2016	\$9.00	\$3.00
Second quarter ending September 30, 2016	\$4.69	\$2.81
Third quarter ending December 31, 2016	\$4.50	\$3.11
Fourth quarter ending March 31, 2017	\$3.90	\$1.74
Fiscal Year Ending March 31, 2016		
First quarter ending June 30, 2015	\$16.50	\$8.00
Second quarter ending September 30, 2015	\$14.90	\$6.50
Third quarter ending December 31, 2015	\$10.25	\$4.00
Fourth quarter ending March 31, 2016	\$9.97	\$6.50

On December 6, 2017, the closing price of our common stock on the NASDAQ Capital Market was \$1.80 per share. As of December 6, 2017, there were approximately 1,300 holders of our common stock.

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DIVIDEND POLICY

We have never declared or paid cash dividends on our capital stock. We currently intend to retain all available funds for use in the operation and expansion of our business, and do not anticipate paying any cash dividends in the foreseeable future. Our Series B Preferred accrues dividends at a rate of 10% per annum, which dividends are payable solely in unregistered shares of our common stock at the time the Series B Preferred is converted into common stock. Any future determination related to dividend policy will be made at the discretion of our Board of Directors, subject to applicable laws, and will depend on a number of factors, including our financial condition, results of operations, capital requirements, contractual restrictions, general business conditions and other factors that our Board of Directors may deem relevant.

Table of Contents**DILUTION**

If you purchase shares of our common stock and related warrants in this offering, you will experience dilution to the extent of the difference between the combined public offering price per share and related warrant in this offering and our pro forma as adjusted net tangible book value per share immediately after this offering assuming no value is attributed to the warrants, and the warrants are accounted for and classified as equity. As of September 30, 2017, our net tangible book value (deficit) was approximately \$(1,671,600), or approximately \$(0.14) per share. Net tangible book value (deficit) is equal to our total assets minus the sum of liabilities and intangible assets. Net tangible book value (deficit) per share is net tangible book value (deficit) divided by the total number of shares of common stock outstanding.

After giving effect to the assumed sale by us of 8,333,333 shares of our common stock and warrants to purchase up to 8,333,333 shares of our common stock in this offering at an assumed combined public offering price of \$1.80 per share and related warrant (the last reported sale price of our common stock on the NASDAQ Capital Market on December 6, 2017), and after deducting the estimated underwriting discount and estimated offering expenses payable by us, our as-adjusted net tangible book value as of September 30, 2017 would have been approximately \$11.9 million, or approximately \$0.60 per share. This amount represents an immediate increase in net tangible book value of \$0.74 per share to existing stockholders and an immediate dilution in net tangible book value of \$1.20 per share to purchasers of our common stock and related warrants in this offering.

The following table illustrates the dilution in net tangible book value per share to new investors:

Assumed combined public offering price per share and related warrant:	\$1.80
Net tangible book value (deficit) per share as of September 30, 2017	\$(0.14)
Increase in pro forma net tangible book value per share after this offering	\$0.74
Pro forma net tangible book value per share after this offering	\$0.60
Dilution per share to new investors	\$1.20

Each \$0.20 increase (decrease) in the assumed combined public offering price of \$1.80 per share and related warrant would increase (decrease) our pro forma net tangible book value by approximately \$0.08 per share of common stock and the dilution to new investors by approximately \$0.12 per share, assuming that the number of shares and related warrants offered by us, as set forth on the cover page of this prospectus, remains the same, after deducting the estimated underwriting discount and estimated offering expenses payable by us.

We may also increase or decrease the number of shares and related warrants we are offering. An increase of 1,000,000 shares in the number of shares of common stock and warrants offered by us, as set forth on the cover page of this prospectus, would increase our pro forma net tangible book value by approximately \$1.7 million, or approximately \$0.05 per share, and decrease the dilution per share to new investors in this offering by approximately \$0.05 share, assuming that the assumed combined public offering price per share and related warrant remains the same, and after deducting the estimated underwriting discount and estimated offering expenses payable by us. Similarly, a decrease of 1,000,000 shares in the number of shares of common stock and related warrants offered by us, as set forth on the cover page of this prospectus, would decrease our pro forma net tangible book value by approximately \$1.7 million, or approximately \$0.05 per share, and increase the dilution per share to new investors in this offering by approximately \$0.05 per share, assuming that the assumed combined public offering price per share and related warrant remains the same, and after deducting the estimated underwriting discount and estimated offering expenses payable by us. The

information discussed above is illustrative only and will change based on the actual combined public offering price and other terms of this offering determined at pricing.

The foregoing discussion and table do not take into account further dilution to new investors that could occur upon the exercise of outstanding options or warrants having a per share exercise price less than the combined public offering price in this offering. To the extent that we raise additional capital through the sale of equity or convertible debt securities, the issuance of those securities could result in further dilution to our stockholders.

The table and discussion above are based on 11,628,974 shares of our common stock outstanding as of September 30, 2017, and excludes as of that date the following:

3,279,871 shares of common stock issuable upon exercise of outstanding stock options with a weighted average exercise price of \$3.23 per share;

6,965,151 shares of common stock issuable upon exercise of outstanding warrants with a weighted average exercise price of \$4.77 per share;

750,000 shares of common stock issuable upon conversion of all outstanding shares of our Series A Preferred;

1,160,240 shares of common stock issuable upon conversion of all outstanding shares of our Series B Preferred and 663,460 shares of common stock reserved for issuance as payment of accrued dividends on outstanding shares of Series B Preferred;

2,318,012 shares of common stock issuable upon conversion of all outstanding shares of our Series C Preferred;

6,082,162 shares of common stock reserved for future issuance in connection with future grants under our Amended and Restated 2016 Stock Incentive Plan; and

shares of common stock issuable upon the exercise of the warrants offered hereby.

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CAPITALIZATION

The following table sets forth our cash and cash equivalents and capitalization as of September 30, 2017:

on an actual basis; and

on a pro forma basis giving effect to the assumed sale and issuance by us of 8,333,333 shares of common stock warrants to purchase up to 8,333,333 shares of our common stock in this offering, at an assumed combined public offering price of \$1.80 per share and related warrant (the last reported sale price of our common stock on the NASDAQ Capital Market on December 6, 2017), and after deducting the estimated underwriting discount and estimated offering expenses payable by us assuming no value is attributed to the warrants, and the warrants are accounted for and classified as equity.

As of September 30, 2017 (amounts in dollars and in thousands, except share and per share amounts)	Actual	Pro forma
Cash and cash equivalents	\$ 1,764	\$ 15,374
Long-term debt	\$—	\$—
Stockholders' equity (deficit):		
Preferred stock, \$0.001 par value, 10,000,000 shares authorized:		
Series A Preferred, 500,000 shares authorized and outstanding, actual and pro forma	1	1
Series B Preferred, 4,000,000 shares authorized and 1,160,240 shares outstanding, actual and pro forma	1	1
Series C Preferred, 3,000,000 shares authorized and 2,318,012 shares outstanding, actual and pro forma	2	2
Common stock, \$0.001 par value, 100,000,000 shares authorized; 11,764,639 shares issued, actual; 20,097,972 shares issued, pro forma	12	20
Additional paid-in capital	151,541	165,143
Treasury stock, at cost, 135,665 shares, actual and pro forma	(3,968)	(3,968)
Accumulated deficit	(149,261)	(149,261)
Total stockholders' equity (deficit)	(1,672)	11,938
Total capitalization	\$ 1,672	\$11,938

Common stock outstanding in the table above excludes the following shares as of September 30, 2017:

3,279,871 shares of common stock issuable upon exercise of outstanding stock options with a weighted average exercise price of \$3.23 per share;

6,965,151 shares of common stock issuable upon exercise of outstanding warrants with a weighted average exercise price of \$4.77 per share;

750,000 shares of common stock issuable upon conversion of all outstanding shares of our Series A Preferred;

1,160,240 shares of common stock issuable upon conversion of all outstanding shares of our Series B Preferred and 663,460 shares of common stock reserved for issuance as payment of accrued dividends on outstanding shares of Series B Preferred;

2,318,012 shares of common stock issuable upon conversion of all outstanding shares of our Series C Preferred;

6,082,162 shares of common stock reserved for future issuance in connection with future grants under our Amended and Restated 2016 Stock Incentive Plan ; and

shares of common stock issuable upon the exercise of the warrants offered hereby.

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SELECTED CONSOLIDATED FINANCIAL DATA

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The following selected consolidated financial data of VistaGen Therapeutics, Inc. should be read in conjunction with, and are qualified by reference to, the section titled “Management’s Discussion and Analysis of Financial Condition and Results of Operations” and the consolidated financial statements and notes thereto included elsewhere in this prospectus. The consolidated statement of operations data for the years ended March 31, 2017 and 2016, and the consolidated balance sheet data as of March 31, 2017 and 2016 are derived from, and qualified by reference to, our audited consolidated financial statements included elsewhere in this prospectus and should be read in conjunction with those consolidated financial statements and notes thereto. The consolidated statement of operations data for the six-month periods ended September 30, 2017 and 2016 and the consolidated balance sheet data as of September 30, 2017 are derived from our unaudited consolidated financial statements included elsewhere in this prospectus which, in our opinion, have been prepared on the same basis as the audited consolidated financial statements and reflect only adjustments, consisting only of normal recurring adjustments, necessary for a fair presentation of our results of operations and financial position. Our results for the six months ended September 30, 2017 are not necessarily indicative of results to be expected for the full year or any other period.

	Fiscal Year Ended March 31,		Six-Months Ended September 30,	
	2017	2016	2017	2016
Consolidated Statement of Operations Data:	(in thousands, except share and per share amounts)			
Revenues	\$ 1,250	\$ –	\$ –	\$ –
Operating expenses:				
Research and development	5,204	3,932	3,523	2,432
General and administrative	6,295	13,919	3,731	2,631
Total operating expenses	11,499	17,851	7,254	5,063
Loss from operations	(10,249)	(17,851)	(7,254)	(5,063)
Other expenses, net:				
Interest expense, net	(5)	(771)	(6)	(3)
Change in warrant liabilities	–	(1,895)	–	–
Loss on early extinguishment of debt	–	(26,700)	–	–
Other expense	–	(2)	–	–
Loss before income taxes	(10,254)	(47,219)	(7,260)	(5,063)
Income taxes	(2)	(2)	(2)	(2)
Net loss and comprehensive loss	(10,256)	(47,221)	(7,262)	(5,068)
Accrued dividend on Series B Preferred Stock	(1,257)	(2,140)	(504)	(781)
Deemed dividend on Series B Preferred Units	(111)	(2,058)	–	(111)
Net loss attributable to common stockholders	\$ (11,624)	\$ (51,419)	\$ (7,766)	\$ (5,960)

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Basic and diluted net loss attributable to common stockholders per common share	\$ (1.54)	\$ (29.08)	\$ (0.82)	\$ (0.91)
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Weighted average shares used in computing basic and diluted net loss attributable to common stockholders per common share	7,531,642	1,767,957	9,465,459	6,577,769
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	As of March 31,		As of September 30, 2017	
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	2017	2016	2017	Pro Forma (1)
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Consolidated Balance Sheet Data:

Cash and cash equivalents	\$ 2,921	\$ 429	\$ 1,764	\$ 15,374
Total assets	\$ 3,712	\$ 990	\$ 2,618	\$ 16,228
Current portion of notes payable and accrued interest	\$ 55	\$ 44	\$ 105	\$ 105
Working capital	\$ 2,010	\$ (939)	\$ 443	\$ 14,053
Common stock, preferred stock, treasury stock and additional paid-in capital	\$ 142,615	\$ 132,734	\$ 147,589	\$ 161,199
Total stockholders' equity (deficit)	\$ 616	\$ (2,977)	\$ (1,672)	\$ 11,938

(1)

The pro forma column gives effect to the assumed sale and issuance by us of 8,333,333 shares of our common stock and warrants to purchase up to 8,333,333 shares of our common stock in this offering, assuming a combined public offering price of \$1.80 per share and related warrant (the last reported sale price of our common stock on the NASDAQ Capital Market on December 6, 2017), after deducting the estimated underwriting discount and estimated offering expenses payable by us assuming no value is attributed to the warrants, and the warrants are accounted for and classified as equity.

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MANAGEMENT'S DISCUSSION AND ANALYSIS OF FINANCIAL CONDITION AND RESULTS OF OPERATIONS

You should read the following discussion of our financial condition and results of operations in conjunction with the financial statements and the notes thereto included elsewhere in this prospectus. The following discussion contains forward-looking statements that reflect our plans, estimates and beliefs. Our actual results could differ materially from those discussed in the forward-looking statements. Factors that could cause or contribute to these differences include those discussed below and elsewhere in this prospectus, particularly in “Risk Factors.”

Overview

We are a clinical-stage biopharmaceutical company focused on developing new generation medicines for depression and other central nervous system (CNS) disorders.

AV-101 is our oral CNS product candidate in Phase 2 clinical development in the United States, initially as a new generation adjunctive treatment for Major Depressive Disorder (MDD) in patients with an inadequate response to standard antidepressants approved by the U.S. Food and Drug Administration (FDA). AV-101's mechanism of action (MOA) involves both NMDA (N-methyl-D-aspartate) and AMPA (alpha-amino-3-hydroxy-5-methyl-4-isoxazolepropionic acid) receptors in the brain responsible for fast excitatory synaptic activity throughout the CNS. AV-101's MOA is fundamentally differentiated from all FDA-approved antidepressants, as well as all atypical antipsychotics such as aripiprazole often used adjunctively to augment them. We believe AV-101 also has potential as a non-opioid treatment alternative for neuropathic pain, as well as several additional CNS indications where modulation of the NMDA receptors, activation of AMPA pathways and/or key active metabolites of AV-101 may achieve therapeutic benefit, including Parkinson's disease levodopa-induced dyskinesia (PD LID), epilepsy and Huntington's disease.

Clinical studies conducted at the U.S. National Institute of Mental Health (NIMH), part of the U.S. National Institutes of Health (NIH), by Dr. Carlos Zarate, Jr., Chief of the NIMH's Experimental Therapeutics & Pathophysiology Branch and its Section on Neurobiology and Treatment of Mood and Anxiety Disorders, have focused on the antidepressant effects of low dose intravenous (IV) administration of ketamine hydrochloride (ketamine), an NMDAR antagonist, in patients with treatment-resistant MDD. These NIMH studies, as well as clinical research at Yale University and other academic institutions, have demonstrated robust antidepressant effects in treatment-resistant MDD patients within twenty-four hours of a single IV dose of ketamine.

As published in the October 2015 issue of the peer-reviewed, Journal of Pharmacology and Experimental Therapeutics, in an article entitled, The prodrug 4-chlorokynurenine causes ketamine-like antidepressant effects, but not side effects, by NMDA/glycineB-site inhibition, using well-established preclinical models of depression, AV-101 was shown to induce fast-acting, dose-dependent, persistent and statistically significant antidepressant-like responses following a single treatment. These responses were equivalent to those seen with a single sub-anesthetic control dose of ketamine. In addition, these studies confirmed that the fast-acting antidepressant effects of AV-101 were mediated through the GlyB site and also involved the activation of another key neurological pathway, the alpha-amino-3-hydroxy-5-methyl-4-isoxazolepropionic acid (AMPA) receptor pathway.

Pursuant to our Cooperative Research and Development Agreement (CRADA) with the NIMH, the NIMH is currently funding, and Dr. Zarate, as Principal Investigator, and his team are currently conducting, a small Phase 2 clinical study of AV-101 monotherapy in subjects with treatment-resistant MDD (the NIMH AV-101 MDD Phase 2 Monotherapy Study). In October 2017, we received FDA authorization to launch our 180-patient Phase 2 multi-center, multi-dose, double blind, placebo-controlled efficacy and safety study of AV-101 as a new generation adjunctive treatment of

MDD in adult patients with an inadequate therapeutic response to standard, FDA-approved antidepressants (the AV-101 MDD Phase 2 Adjunctive Treatment Study). Subject to completion of this offering, we intend to launch the AV-101 MDD Phase 2 Adjunctive Treatment Study in the first quarter of 2018 with Dr. Maurizio Fava, Professor of Psychiatry at Harvard Medical School and Director, Division of Clinical Research, Massachusetts General Hospital (MGH) Research Institute, as the Principal Investigator. Dr. Fava was the co-Principal Investigator with Dr. A. John Rush of the STAR*D study, the largest clinical trial conducted in depression to date, whose findings were published in journals such as the New England Journal of Medicine (NEJM) and the Journal of the American Medical Association (JAMA).

VistaStem Therapeutics (VistaStem) is our wholly owned subsidiary focused on applying human pluripotent stem cell (hPSC) technology, internally and with third-party collaborators, to discover, rescue, develop and commercialize (i) proprietary new chemical entities (NCEs), including small molecule NCEs with regenerative potential, for CNS and other diseases and (ii) cellular therapies involving stem cell-derived blood, cartilage, heart and liver cells. Our internal drug rescue programs are designed to utilize CardioSafe 3D, our customized cardiac bioassay system, to develop small molecule NCEs for our pipeline. In December 2016, we exclusively sublicensed to BlueRock Therapeutics LP, a next generation regenerative medicine company established by Bayer AG and Versant Ventures (BlueRock Therapeutics), rights to certain proprietary technologies relating to the production of cardiac stem cells for the treatment of heart disease (the BlueRock Agreement). Through VistaStem, we may also pursue additional potential regenerative medicine (RM) applications, including using blood, cartilage, and/or liver cells derived from hPSCs for (A) cell-based therapy, (B) cell repair therapy, and/or (C) tissue engineering. In a manner similar to our exclusive sublicense agreement with BlueRock Therapeutics, we may pursue these additional RM applications in collaboration with third-parties.

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Financial Operations Overview

Net Loss.

Despite generating \$1.25 million of sublicense revenue from the BlueRock Agreement in December 2016, we have not yet achieved recurring revenue-generating status from any of our product candidates or technologies. Since our inception in May 1998, we have devoted substantially all of our time and efforts to developing our lead CNS product candidate, AV-101, from early nonclinical studies to our ongoing Phase 2 clinical development program in MDD, as well as stem cell technology research and development, bioassay development, small molecule drug development, and creating, protecting and patenting intellectual property related to our product candidates and technologies, with the corollary initiatives of recruiting and retaining personnel and raising working capital. As of September 30, 2017, we had an accumulated deficit of approximately \$149.3 million. Our net loss for the six months ended September 30, 2017 and 2016 was approximately \$7.3 million and \$5.1 million, respectively. We expect losses to continue for the foreseeable future, primarily related to our further development of AV-101 for the adjunctive treatment of MDD, as well as other CNS indications.

Summary of Our Fiscal Year Ended March 31, 2017 and Six Months Ended September 30, 2017.

During Fiscal 2017 and the first two quarters of Fiscal 2018, we have continued to (i) advance nonclinical, including manufacturing, and clinical development of AV-101 as a potential new generation antidepressant and as a potential new therapeutic alternative for several other CNS indications with significant unmet medical need, (ii) expand the regulatory and intellectual property foundation to support broad clinical development and, ultimately, commercialization of AV-101 in the U.S. and foreign markets, and (iii) on a limited basis, advance the predictive toxicology capabilities of CardioSafe 3D for small molecule new chemical entity drug rescue and development applications and collaborative regenerative medicine opportunities related to our cardiac stem cell technology platform .

Pursuant to our Cooperative Research and Development Agreement (CRADA) with the NIH, the NIH continues to fund, and Dr. Carlos Zarate Jr. of the NIMH continues to conduct, the NIMH AV-101 MDD Phase 2 Monotherapy Study, at no cost to us other than supplying clinical trial material. Although we do not direct or control the progress of this study, we currently anticipate that the NIMH will complete the NIMH AV-101 MDD Phase 2 Monotherapy Study in the first half of 2018.

In May 2016, we consummated an underwritten public offering of our securities pursuant to which we received net proceeds of approximately \$9.54 million and issued to institutional investors an aggregate of 2,570,040 registered shares of our common stock and five-year warrants exercisable at \$5.30 per share to purchase an aggregate of 2,705,883 shares of our common stock (May 2016 Public Offering). In connection with the May 2016 Public Offering, our common stock was approved for listing on The NASDAQ Capital Market, where it has traded under the symbol "VTGN" since May 11, 2016. Please see the section titled "Liquidity and Capital Resources" below, for a summary of our capital raising activity subsequent to March 31, 2017 and a discussion of our expected future capital requirements.

In addition to bolstering our Clinical and Regulatory Advisory Board with the appointment of Dr. Maurizio Fava (Harvard University) as Chairman and the addition of members Dr. Sanjay Matthew (Baylor University) and Dr. Thomas Laughren (former director, FDA's Division of Psychiatry), all pre-eminent opinion leaders in the field of depression, and the addition of veteran healthcare executive Jerry Gin, Ph.D., MBA to our Board of Directors, we enhanced our management team with the addition of Mark A. Smith, MD, Ph.D., as our Chief Medical Officer in June 2016. Dr. Smith has over 20 years of pharmaceutical industry and CNS drug development experience. He has been a

successful project leader in both drug discovery and development on projects resulting in approximately 20 investigational new drugs (INDs). Dr. Smith has directed clinical trials examining depression, bipolar disorder, anxiety, schizophrenia, Alzheimer's disease, ADHD and agitation in Phase 1 through Phase 2b. In addition, Dr. Smith has vast knowledge and expertise in translational neuroscience, clinical trial design and regulatory interactions. In September 2016, we appointed Mark A. McPartland as our Vice President of Corporate Development. Mr. McPartland has over 20 years of experience in corporate development, capital markets, corporate communications and management consulting for companies at varying stage of their corporate evolution, including early- and mid-stage biopharmaceutical companies. Mr. McPartland is primarily concentrating his efforts in expanding awareness of VistaGen across a range of investors, researchers, patients, clinicians and potential partners. In July 2017, we appointed Mark Wallace, M.D., Distinguished Professor of Clinical Anesthesiology at the University of California, San Diego, to our Clinical and Regulatory Advisory Board to assist us in advancing development of AV-101 as a potential non-opioid treatment alternative for neuropathic pain. Dr. Wallace is an internationally recognized leader in the field of multi-modal pain management, with over 30 years of professional experience, board certifications, licensures, honors/awards, grants, articles and abstracts.

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In December 2016, we entered into the BlueRock Agreement with BlueRock Therapeutics, LP, a next generation regenerative medicine company recently established by Bayer AG and Versant Ventures (BlueRock), pursuant to which BlueRock received exclusive rights to utilize certain technologies exclusively licensed by us from University Health Network (UHN) for the production of cardiac stem cells for the treatment of heart disease. We retained rights to technology licensed from UHN related to small molecule, protein and antibody drug discovery, drug rescue and drug development, including small molecules with cardiac regenerative potential, as well as small molecule, protein and antibody testing involving cardiac cells. In January 2017, we received an upfront cash payment of \$1.25 million under the BlueRock Agreement and we may potentially receive additional cash milestones and royalty payments in the future upon BlueRock's achievement of certain development objectives and commercial sales.

Between late-March 2017 and June 2017, we entered into self-placed private placement transactions with individual accredited investors, pursuant to which we sold units consisting of an aggregate of 495,001 shares of our unregistered common stock and warrants which are not exercisable until six months and one day following issuance and expire on April 30, 2021, to purchase an aggregate of 247,500 shares of our common stock at a weighted average fixed exercise price of \$3.99 per share, subject to adjustment only for customary stock dividends, reclassifications, splits and similar transactions. We received cash proceeds of approximately \$1 million in this self-placed private placement transaction. In September 2017, we reduced the exercise price of each of the warrants issued in the Spring 2017 Private Placement to \$2.00 per share and issued additional warrants, also exercisable six months and one day following issuance at an exercise price of \$2.00 per share, to purchase an aggregate of 247,501 unregistered shares of our common stock. In August 2017, in a self-placed private placement transaction, we sold units consisting of 28,572 shares of our unregistered common stock and warrants exercisable six months and one day following the date of issuance through April 30, 2021 to purchase 28,572 unregistered shares of our common stock at an exercise price of \$4.00 per share. We received cash proceeds of \$50,000 from this private placement transaction.

In September 2017, we completed a firm commitment underwritten public offering, pursuant to which we sold 1,371,430 shares of our common stock and Series A1 Warrants to purchase up to 1,388,931 shares of common stock and Series A2 Warrants to purchase up to 503,641 of common stock (collectively, the Warrants), each exercisable for \$1.82 per share warrants to two of our existing institutional investors, resulting in net proceeds of approximately \$2.0 million (the September 2017 Public Offering). The Series A1 Warrants will be exercisable for a five-year period commencing on March 7, 2018, and the Series A2 Warrants are exercisable at any time and expire on September 6, 2022. The common stock and the shares of common stock underlying the Warrants issued in the September 2017 Public Offering were sold pursuant to our effective Registration Statement on Form S-3 (Registration No. 333-215671) to cover this and potential future sales of our equity securities in one or more public offerings from time to time.

In July 2017, we appointed Mark Wallace, M.D., Distinguished Professor of Clinical Anesthesiology at the University of California, San Diego, to our Clinical and Regulatory Advisory Board to assist us in advancing development of AV-101 as a potential non-opioid treatment alternative for neuropathic pain. Dr. Wallace is an internationally recognized leader in the field of multi-modal pain management, with over 30 years of professional experience, board certifications, licensures, honors/awards, grants, articles and abstracts.

We continue to prepare for the launch of our AV-101 MDD Phase 2 Adjunctive Treatment Study with initiatives that have significantly improved the efficiency of our AV-101 manufacturing processes and production of sufficient quantities of AV-101 to enable a more comprehensive initiation of the study. We currently anticipate the launch of the AV-101 MDD Phase 2 Adjunctive Treatment Study, with Dr. Maurizio Fava of Harvard Medical School serving as Principal Investigator, in the first quarter of 2018.

Additionally, we are pursuing initiatives to secure a broad spectrum of intellectual property protection for AV-101 covering multiple CNS indications. We have filed and are pursuing several patent applications in Europe, the U.S. and other selected regions. Several of these patent applications have already been granted or allowed, on both (i) certain novel therapeutic methods of use of AV-101, including depression, and (ii) certain novel methods of producing AV-101. In Europe, the European Patent Office (EPO) recently granted our patent related to methods of treating depression with AV-101 and certain other neurological indications. In the U.S. and other selected regional markets, we are currently pursuing a counterpart AV-101 patent application similar to the patent granted by the EPO. Although the U.S. Patent and Trademark Office (USPTO) has not yet allowed the counterpart application filed in the U.S., we believe that our counterpart patent applications under review by the USPTO and other countries ultimately will be granted.

In Europe, the U.S. and other selected regional markets, we are also prosecuting patent applications related to novel methods of producing AV-101. We recently received a Notice of Allowance for manufacturing patent applications regarding our manufacturing process from the USPTO and Chinese patent office. We have submitted a counterpart application to the EPO, which has not yet been approved. However, given that our application at the USPTO was sufficient to obtain an allowed patent in the U.S., we believe our counterpart patent application at the EPO ultimately will be granted.

As a matter of course, we continue to minimize, to the greatest extent possible cash commitments and expenditures for both internal and external research and development and general and administrative services. To further advance the nonclinical and clinical development of AV-101 and our stem cell technology platform, as well as support our operating activities, we continue to carefully manage our routine operating costs, including our internal employee related expenses, as well as external costs relating to regulatory consulting, contract research and development, investor relations and corporate development, legal, acquisition and protection of intellectual property, public company compliance and other professional services and internal costs.

Results of Operations

Comparison of Fiscal Years Ended March 31, 2017 and 2016

The following table summarizes the results of our operations for the fiscal years ended March 31, 2017 and 2016 (amounts in thousands).

	Fiscal Years Ended March 31,	
	2017	2016
Sublicense revenue	\$1,250	\$-
Operating expenses:		
Research and development	5,204	3,932
General and administrative	6,295	13,919
Total operating expenses	11,499	17,851
Loss from operations	(10,249)	(17,851)
Interest expense (net)	(5)	(771)
Change in warrant liabilities	-	(1,895)
Loss on extinguishment of debt	-	(26,700)
Other expense	-	(2)
Loss before income taxes	(10,254)	(47,219)
Income taxes	(2)	(2)
Net loss	(10,256)	(47,221)
Accrued dividend on Series B Preferred Stock	(1,257)	(2,140)
Deemed dividend on Series B Preferred Stock	(111)	(2,058)
Net loss attributable to common stockholders	\$(11,624)	\$(51,419)

Revenue

We recognized \$1.25 million in sublicense revenue pursuant to the BlueRock Agreement in the quarter ended December 31, 2016. While we may potentially receive additional payments and royalties under the BlueRock Agreement in the future, in the event certain performance-based milestones and commercial sales are achieved by BlueRock, the agreement might not provide recurring revenue to us in the near term. We reported no other revenue for the fiscal years ended March 31, 2017 or 2016 and we presently have no revenue-generating arrangements with respect to AV-101 or other potential product candidates. However, as indicated previously, under our CRADA with the NIH, the NIH is fully funding and conducting the NIMH AV-101 MDD Phase 2 Monotherapy Study at no cost to us.

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Research and Development Expense

Research and development expense totaled \$5.2 million for the fiscal year ended March 31, 2017 (Fiscal 2017), an increase of approximately 33% compared with the \$3.9 million incurred for the fiscal year ended March 31, 2016 (Fiscal 2016), demonstrating our increased and continuing focus on manufacturing and nonclinical and clinical development of AV-101 in preparation for our AV-101 MDD Phase 2 Adjunctive Treatment Study, which we currently anticipate to begin in the first quarter of 2018. Of the amounts reported, non-cash expenses, related primarily to grants or modifications of our equity securities, totaled approximately \$534,000 in Fiscal 2017 and \$1.7 million in Fiscal 2016. The following table indicates the primary components of research and development expense for each of the periods (amounts in thousands):

	Fiscal Years Ended March 31,	
	2017	2016
Salaries and benefits	\$1,331	\$818
Stock-based compensation	375	1,093
Consulting and other professional services	(75)	112
Technology licenses and royalties, including UHN	746	1,010
Project-related research, development and supplies:		
AV-101	2,292	406
Stem cell and all other	185	100
	2,477	506
Rent	310	219
Depreciation	37	37
Warrant modification expense	-	135
All other	3	2
Total Research and Development Expense	\$5,204	\$3,932

The increase in salaries and benefits reflects the impact of the hiring of our Chief Medical Officer (CMO) in June 2016, as well as salary increases and bonus payments granted to our President and Chief Scientific Officer (CSO) and to the four non-officer members of our scientific staff.

The decrease in stock based compensation expense is primarily attributable to the \$852,200 fair value, determined using the Black-Scholes Option Pricing Model and the assumptions indicated in Note 13, Stock Option Plans and 401(k) Plan, to the accompanying Consolidated Financial Statements in Part 8 of this Report, of the September 2015 grant of immediately vested and expensed warrants to purchase 150,000 shares of our common stock granted to our CSO. Stock compensation expense in Fiscal 2017 reflects the ratable amortization of option grants made to our CSO and CMO, scientific staff and consultants, in November 2016, June 2016 (CSO and CMO only) and September 2015. Our stock options are generally amortized over a two-year to four-year vesting period. A substantial number of the

option grants made in or prior to our fiscal year ended March 31, 2014 became fully-vested and were fully-expensed by March 31, 2017.

Consulting services reflects fees paid or accrued for scientific, nonclinical and clinical development and regulatory advisory and consulting services rendered to us by third-parties, primarily by members of our scientific and CNS clinical and regulatory advisory boards. The reduction in expense for Fiscal 2017 primarily reflects the rationalization of our stem cell-related scientific advisory board and related accruals, including as a result of the BlueRock Agreement.

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Technology license expense reflects both recurring annual fees as well as legal counsel and other costs related to patent prosecution and protection pursuant to certain of our stem cell technology license agreements or for other potential commercial purposes. We recognize these costs as they are invoiced to us by the licensors and they do not occur ratably throughout the year or between years. Additionally, in both periods, this expense includes legal counsel and other costs we have incurred to advance in the U.S. and numerous foreign countries several pending patent applications with respect to AV-101 and our stem cell technology platform. Technology license-related legal expense for Fiscal 2017 also includes \$55,000 representing the fair value of a warrant granted to intellectual property counsel as partial compensation for services. Fiscal 2017 expense further includes a net of \$158,000 related to the sublicense consideration paid to University Health Network (UHN) related to the BlueRock Agreement plus additional fees and expenses related to two newly acquired licenses from UHN related to cardiac stem cell technology, net of amounts previously accrued in connection with our prior sponsored research collaboration with UHN. Technology license expense for Fiscal 2016 included (i) approximately \$153,000 of fees and expenses incurred for additional stem cell technology related licenses acquired in connection with our agreement with UHN; (ii) \$120,000 of noncash expense resulting from the grants to two intellectual property legal service providers in July 2015 of an aggregate of 10,000 shares of our Series B Preferred, and (iii) \$254,000 of noncash expense resulting from the March 2016 grant of immediately-vested warrants to purchase an aggregate of 50,000 shares of our common stock to two intellectual property legal service providers.

AV-101 expenses for Fiscal 2017 include continuing costs incurred to develop more efficient and cost-effective proprietary production methods for AV-101 and for certain pre-production and nonclinical trial analyses and procedures to facilitate Phase 2 clinical development of AV-101, including our AV-101 MDD Phase 2 Adjunctive Treatment Study. We expect these expenses to increase significantly during Fiscal 2018 and Fiscal 2019 as we continue preparations for, initiate and conduct our AV-101 MDD Phase 2 Adjunctive Treatment Study. Additionally, AV-101 expense in both periods reflects nominal costs associated with standard monitoring for and responding to potential feedback related to our AV-101 Phase 1 clinical safety program and addressing any other matters that may be required under the terms of our prior NIH grant awards, primarily facilitated by our CRO for our Phase 1 safety studies, Cato Research Ltd. The increase in stem cell and other project related expenses in Fiscal 2017 primarily reflects in-house costs associated with our participation in the FDA's CiPA project focused on using next generation cardiac stem-cell technology-based bioassay systems for in vitro cardiac predictive toxicology screening.

The increase in rent expense in Fiscal 2017 reflects both the impact of the scheduled rent increase for our South San Francisco headquarters and laboratory facilities effective August 2016, as well as the impact of accounting for the November 2016 lease amendment extending our lease of those facilities by five years from July 31, 2017 to July 31, 2022.

Warrant modification expense in Fiscal 2016 reflects the increase in fair value resulting from the November 2015 modification of outstanding warrants to purchase an aggregate of 315,000 shares of our common stock held by our CSO and a key scientific advisor to reduce the exercise prices thereof from a range of \$9.25 to \$12.80 per share to \$7.00 per share. No similar modifications occurred in Fiscal 2017.

General and Administrative Expense

General and administrative expense decreased to \$6.3 million in Fiscal 2017 from \$13.9 million in Fiscal 2016 primarily as a result of the decrease in non-cash stock compensation expense attributable to option and warrant grants to employees, officers and independent Board members in Fiscal 2016, partially offset by an increase in non-cash expense related to grants of equity securities in payment of certain professional services during Fiscal 2017. Of the amounts reported, non-cash expenses, related primarily to grants or modifications of our equity securities, totaled approximately \$3.1 million in Fiscal 2017 and \$11.9 million in Fiscal 2016. The following table indicates the primary

components of general and administrative expenses for each of the periods (amounts in thousands):

	Fiscal Years Ended March 31,	
	2017	2016
Salaries and benefits	\$1,206	\$694
Stock-based compensation	476	2,949
Board fees	140	98
Legal, accounting and other professional fees	2,093	3,405
Investor relations	1,219	172
Insurance	165	140
Travel and entertainment	179	96
Rent and utilities	220	157
Warrant modification expense	427	6,083
All other expenses	170	125
Total General and Administrative Expense	\$6,295	\$13,919

The increase in salaries and benefits reflects the impact of salary increases and bonus payments granted to our Chief Executive Officer (CEO), Chief Financial Officer (CFO), and a member of our administrative staff and the change in that employee's status from part-time to full-time, as well as the hiring of our VP, Corporate Development in September 2016.

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The decrease in stock based compensation expense is primarily attributable to the \$2.8 million fair value, determined using the Black-Scholes Option Pricing Model and the assumptions indicated in Note 13, Stock Option Plans and 401(k) Plan, to the accompanying Consolidated Financial Statements in Item 8 of this Report, of the September 2015 grant of immediately vested and expensed warrants to purchase 500,000 shares of our common stock granted to our CEO, CFO, independent members of our Board of Directors and certain consultants. Stock compensation expense in Fiscal 2017 reflects the ratable amortization of option grants made to our CEO, CFO, independent members of our Board of Directors and administrative staff and consultants, in November 2016, June 2016 (CEO, CFO and independent Board members only) and September 2015, as well as to our VP-Corporate Development upon the commencement of his employment in September 2016. Our stock options are generally amortized over a two-year to four-year vesting period. A substantial number of the option grants made in or prior to our fiscal year ended March 31, 2014 became fully-vested and were fully-expensed by March 31, 2017.

Board fees includes fees recognized for the services of independent members of our Board of Directors. We added an additional independent director, Dr. Jerry Gin, to our Board in March 2016.

Legal, accounting and other professional fees in Fiscal 2017 and Fiscal 2016 includes \$337,500 and \$1.0 million, respectively, of non-cash expense recognized pursuant to the June 2015 grant of an aggregate of 90,000 shares of our Series B Preferred having an aggregate fair value at the time of issuance of \$1.4 million as compensation for financial advisory and corporate development service contracts with two independent service providers for services performed between July 2015 and June 2016. During Fiscal 2017, in addition to the expense noted above attributable to the June 2015 Series B Preferred grant, we granted an aggregate of 25,000 unregistered shares of our common stock having a fair value at the date of issuance of \$108,500 to a legal services provider as partial compensation for services and an aggregate of 320,000 unregistered shares of our common stock having a fair value at the date of issuance of \$1.1 million as partial compensation for financial advisory, investment banking and business development services. During Fiscal 2016, in addition to the expense noted above attributable to the June 2015 Series B Preferred grant, we also granted (i) an aggregate of 50,000 shares of our common stock having an aggregate fair value of \$500,000 pursuant to two corporate development contracts initiated during the first quarter of Fiscal 2016; (ii) 25,000 shares of our Series B Preferred having a fair value of \$250,000 to legal counsel as compensation for services in connection with our debt restructuring and other corporate finance matters, and (iii) 15,750 shares of our unregistered common stock and a five-year warrant to purchase 7,500 unregistered shares of our common stock having an aggregate fair value of \$138,000 in connection with investment banking services. In both years, professional services expense also includes cash payments for routine legal fees and expenses and the expense related to the annual audit of the prior year financial statements, preparation of the prior year income tax returns, and quarterly reviews of current year financial statements.

Investor relations expense includes the fees of our external service providers for a significantly expanded broad spectrum of institutional investor relations and public market awareness and support functions and, particularly during Fiscal 2017, initiatives that included numerous meetings in multiple U.S. markets and other communication activities focused on expanding public market awareness of the Company, including among investment professionals and investment advisors, and individual and institutional investors. During Fiscal 2017, in addition to cash fees and expenses we incurred, we granted an aggregate of 160,000 unregistered shares of our common stock to six investor relations and market awareness service providers as full or partial compensation for their services and recognized non-cash expense of \$472,800, representing the fair value of the stock at the time of issuance. We also granted three-year, immediately exercisable warrants to purchase an aggregate of 75,000 shares of our unregistered common stock at exercise prices ranging from \$4.50 per share to \$6.00 per share to three investor relations service providers and recognized non-cash expense of \$172,300 representing the fair value of the warrants at the time of issuance.

In both periods, travel expense reflects costs associated with presentations to and meetings in numerous U.S. markets with existing and potential investors and investment professionals and advisors, media and securities analysts, as well as various investor relations, market awareness and business development initiatives, in Fiscal 2017 by our CEO, CMO and VP, Corporate Development.

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Between January 2016 and December 2016, we entered into various warrant exchange agreements with certain warrant holders pursuant to which those holders exchanged outstanding warrants to purchase shares of our common stock for a lesser number of unregistered shares of our common stock. In both periods, we accounted for these transactions as warrant modifications. Between April 2016 and December 2016, certain warrant holders agreed to exchange an aggregate of 224,513 shares of our common stock for an aggregate of 156,246 shares of our unregistered common stock, resulting in our recognition of an aggregate of \$350,700 in noncash expense attributable to the increase in fair value related to Fiscal 2017 warrant exchanges. Further, in December 2016, we modified an outstanding warrant to reduce the exercise price from \$8.00 per share to \$3.51 per share and increase the number of shares exercisable under the warrant from 25,000 shares to 50,000 shares, recognizing \$76,900 in expense as the incremental fair value attributable to the modification. Noncash warrant modification expense in Fiscal 2016 includes (i) \$122,000 representing the increase in the fair value attributable to the June 2015 modification of outstanding warrants to purchase an aggregate of 54,576 shares of our common stock to reduce the exercise prices thereof, generally from \$30.00 per share to \$10.00 per share; (ii) \$358,000 representing the increase in the fair value attributable to the November 2015 modification of outstanding warrants to purchase an aggregate of 808,553 shares of our common stock previously granted to our CEO, CFO, and independent members of our Board of Directors to reduce the exercise prices thereof from a range of \$9.25 to \$12.80 per share to \$7.00 per share; and (iii) \$5.6 million representing the aggregate increase in the fair value of certain warrant exchange transactions conducted during the fourth quarter of Fiscal 2016. In January 2016, we entered into an Exchange Agreement with PLTG pursuant to which PLTG exchanged warrants, including all outstanding PLTG Warrants and the shares issuable pursuant to the Series A Preferred Exchange Warrant, to purchase an aggregate of 2,824,016 shares of our common stock for 2,118,012 unregistered shares of our Series C Convertible Preferred Stock (Series C Preferred) at the ratio of 0.75 share of Series C Preferred for each warrant share cancelled. We recognized related noncash warrant modification expense of \$3.2 million. In February and March 2016, we entered into similar agreements with certain other warrant holders pursuant to which such warrant holders exchanged outstanding warrants to purchase an aggregate of 1,086,611 shares of our common stock for an aggregate of 814,989 shares of our unregistered common stock. We recognized an additional \$2.4 million in non-cash warrant modification expense. In February 2016, we also extended the term of certain outstanding warrants to purchase an aggregate of 91,230 shares of our common stock and recognized \$46,000 of non-cash expense as a result of such modifications.

Interest and Other Expenses, Net

Interest expense, net, totaled \$4,600 for Fiscal 2017, a significant decrease compared to the \$770,800 reported for Fiscal 2016, resulting from the extinguishment of substantially all of our promissory notes, as well as other indebtedness, having an aggregate carrying value at the time of extinguishment of approximately \$15.9 million, between May 2015 and August 2015 by conversion into our shares of our Series B Preferred at a conversion price of \$7.00 per share or cash repayment and the related elimination of note interest and discount amortization. The following table summarizes the primary components of interest expense for each of the periods (amounts in thousands):

Fiscal Years
Ended
March 31,

2017 2016

Interest expense on promissory notes	\$1	\$209
Amortization of discount on promissory notes	-	565
Other interest expense, including on capital leases and premium financing	4	3
Total interest expense	5	777
Effect of foreign currency fluctuations on notes payable	-	(6)
Interest income	-	-
Interest expense, net	\$5	\$771

Interest expense on promissory notes in Fiscal 2017 represents only the interest accrued on our promissory note to Progressive Medical Research prior to its repayment in June 2016. The substantial overall decrease in interest expense on promissory notes and the related amortization of discounts on such notes between the periods reflects the cessation of interest accrual and discount amortization upon the extinguishment and conversion of all outstanding Senior Secured Convertible Notes, certain 10% convertible notes (2014 Unit Notes) and other outstanding promissory notes into shares of our Series B Preferred between May 2015 and August 2015.

Under the terms of our October 2012 Note Exchange and Purchase Agreement with PLTG, we issued certain Senior Secured Convertible Promissory Notes and a related Exchange Warrant and Investment Warrants between October 2012 and July 2013. Upon PLTG's exchange of the shares of our Series A Preferred Stock held by PLTG into shares of our common stock, we were also required to issue a Series A Exchange Warrant to PLTG. We determined that the various warrants included certain exercise price resets and other adjustment features requiring us to treat the warrants as liabilities. Accordingly, we recorded a noncash warrant liability at its estimated fair value as of the date of warrant issuance or contract execution. In May 2015, we entered into an agreement with PLTG pursuant to which we amended the various warrants and fixed the exercise price thereof and eliminated the anti-dilution reset features that had previously required the warrants to be treated as liabilities and carried at fair value. Accordingly, during the first quarter of Fiscal 2016, we adjusted these warrants to their fair value, reflecting an increase in the fair value in the amount of \$1.9 million since March 31, 2015, resulting primarily from the increase in the market price of our common stock in relation to the exercise price of the warrants, and then subsequently eliminated the entire warrant liability with respect to these warrants. In January 2016, the PLTG warrants were cancelled and exchanged for shares of our Series C Preferred stock.

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Between May 2015 and August 2015 we extinguished outstanding promissory notes and other indebtedness having a carrying value of approximately \$15.9 million, including our Senior Secured Convertible Notes, our 2014 Unit Notes and other debt and certain adjustments thereto that were either already due and payable or would have otherwise matured prior to March 31, 2016 by converting such balances into shares of our Series B Preferred at a conversion price (the stated value of the Series B Preferred issued) of \$7.00 per share. We treated the conversion of the indebtedness into Series B Preferred as extinguishments of debt for accounting purposes. Since the fair value of the Series B Preferred we negotiated in settlement of the promissory notes and other indebtedness exceeded the carrying value of the debts, we incurred non-recurring noncash losses on each of the extinguishments. Additionally, under the terms of our May 2015 agreement with PLTG in which PLTG agreed to, among other things, convert the Senior Secured Notes and certain other of our convertible promissory notes into Series B Preferred, we issued to PLTG 400,000 shares of Series B Preferred having an aggregate fair value of \$4.0 million and Series B Warrants to purchase 1,200,000 shares of our common stock having an aggregate of fair value of \$8,270,900. We recognized this aggregate fair value as a further non-recurring noncash component of loss on extinguishment of debt. Many of the 2014 Unit Notes that were converted into Series B Preferred contained a beneficial conversion feature at the time they were originally issued. We accounted for the repurchase of the beneficial conversion feature at the time the 2014 Unit Notes were extinguished and converted, an aggregate of \$2.2 million, as a reduction to the loss on extinguishment of debt. We recorded an aggregate net non-recurring non-cash loss of approximately \$26.7 million attributable to the extinguishment of substantially all of our indebtedness as a result of the conversion of such indebtedness into shares of our Series B Preferred at a conversion price (stated value) of \$7.00 per share.

We allocated the proceeds from self-placed private placement sales of Series B Preferred Units to the Series B Preferred and the Series B Warrants based on their relative fair values on the dates of the sales. The difference between the relative fair value per share of the Series B Preferred, approximately \$4.20 per share and \$4.13 per share for Fiscal 2017 and Fiscal 2016, respectively, and its conversion price (or stated value) of \$7.00 per share represented a deemed dividend to the purchasers of the Series B Preferred Units. Accordingly, we recognized a deemed dividend in the aggregate amount of \$111,100 and \$2,058,000 in arriving at net loss attributable to common stockholders for Fiscal 2017 and Fiscal 2016 in the accompanying Consolidated Statement of Operations and Comprehensive Loss included in Item 8 of this Annual Report. Further, we recognized \$1.3 million and \$2.1 million for Fiscal 2017 and Fiscal 2016, respectively, representing the 10% cumulative dividend payable on our Series B Preferred as an additional deduction in arriving at net loss attributable to common stockholders in the accompanying Consolidated Statement of Operations and Comprehensive Loss, included in this Annual Report. The reduction in the dividend accrual results from the automatic conversion of an aggregate of 2,403,051 shares of Series B Preferred upon our completion of the May 2016 Public Offering and a subsequent voluntary conversion of 87,500 shares of our Series B Preferred in August 2016, as disclosed in Note 9, Capital Stock, to the accompanying Consolidated Financial Statements in Item 8 of this Annual Report.

Comparison of Three Months Ended September 30, 2017 and 2016

The following table summarizes the results of our operations, including both cash and noncash components, for the three months ended September 30, 2017 and 2016 (amounts in thousands).

Three Months
Ended September
30,

2017 2016

Operating expenses:

Research and development	\$2,427	\$1,606
General and administrative	2,567	1,494
Total operating expenses	4,994	3,100
Loss from operations	(4,994)	(3,100)
Interest expense, net	(3)	(1)
Loss before income taxes	(4,997)	(3,101)
Income taxes	-	-
Net loss	(4,997)	(3,101)
Accrued dividend on Series B Preferred Stock	(256)	(241)
Deemed dividend on Series B Preferred Stock	-	-
Net loss attributable to common stockholders	\$(5,253)	\$(3,342)

Revenue

We reported no revenue for the quarters ended September 30, 2017 or 2016 and we presently have no recurring revenue generating arrangements with respect to AV-101 or other potential product candidates. While we may potentially receive future milestone payments and royalties under the BlueRock Agreement upon achievement of certain performance-based milestones and commercial sales. However, there can be no assurance that the BlueRock Agreement will provide additional revenue to us in the near term or at all.

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Research and Development Expense

Research and development expense, including both cash and noncash components, totaled \$2.4 million for the quarter ended September 30, 2017, an increase of approximately 51% compared to the \$1.6 million reported for the quarter ended September 30, 2016. Noncash expenses, including stock compensation, depreciation and a portion of rent expense in both periods and a portion of AV-101 project expenses in the quarter ended September 30, 2017, totaled approximately \$592,000 and \$83,000 in the quarters ended September 30, 2017 and 2016, respectively. The increase in research and development expense in 2017 reflects our continued focus on nonclinical and clinical development of AV-101, particularly our preparations for the launch of the AV-101 MDD Phase 2 Adjunctive Treatment Study, which is currently anticipated in the first quarter of 2018, subject to securing additional financing. The following table indicates the primary components of research and development expense for each of the periods (amounts in thousands):

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Three Months
Ended September
30,

2017 2016

Salaries and benefits	\$566	\$461
Stock-based compensation	137	82
Consulting and other professional services	6	31
Technology licenses and royalties	97	94
Project-related research and supplies:		
AV-101	1,476	817
Stem cell and all other	27	51
	1,503	868
Rent	99	62
Depreciation	19	8
Total Research and Development Expense	\$2,427	\$1,606

The increase in salaries and benefits expense reflects the impact of salary increases granted to our Chief Medical Officer (CMO) and Chief Scientific Officer (CSO) in July 2017 and to the non-officer members of our scientific staff in June 2017. Additionally, our then-newly-hired CMO did not receive a bonus payment in July 2016; however, both our CMO and CSO were granted a bonus in September 2017. The full amount of each officer's 2017 bonus was accrued at the time it was granted, but only 25% of the total amount was paid by September 30, 2017, with the remaining balance to be paid ratably through March 31, 2018.

Noncash stock based compensation expense increased in the current period primarily from the routine amortization of option grants made to our CSO, CMO and scientific staff in September 2017, April 2017 and November 2016, which grants, in the aggregate, account for approximately \$105,000 of 2017 expense. Expense attributable to these grants is being amortized over two-year to four-year vesting periods, based on the terms of the respective grants. Substantially all option grants made prior to September 2015 were fully-vested and fully-expensed prior to June 30, 2017.

Consulting services reflects fees paid or accrued for scientific, nonclinical and clinical development and regulatory advisory services rendered to us by third-parties, primarily by members of our scientific and CNS clinical and regulatory advisory boards. The reduction in expense in the current period primarily reflects the change in terms of consulting agreements with our stem cell-related scientific advisory board members.

Technology license expense reflects both recurring annual fees as well as legal counsel and other costs related to patent prosecution and protection pursuant to our stem cell technology license agreements or that we have elected to pursue for commercial purposes. We recognize these costs as they are invoiced to us by the licensors or counsel and they do not occur ratably throughout the year or between years. In both periods, this expense includes legal counsel and other costs we have incurred to advance pending patent applications in the U.S. and numerous foreign countries with respect to AV-101 and our stem cell technology platform.

AV-101 project expense for the quarter ended September 30, 2017 includes continuing costs incurred to develop more efficient and cost-effective proprietary manufacturing methods for AV-101, and to produce clinical trial material for the AV-101 MDD Phase 2 Adjunctive Treatment Study, as well as costs incurred for certain other nonclinical studies to facilitate further clinical development of AV-101 in MDD and potentially for other indications, and to comply with

applicable FDA regulations. Stem cell and other project related expenses reflects costs associated with our in-house stem cell technology-related initiatives, and, in 2016, our participation in the FDA's Comprehensive In Vitro Proarrhythmia Assay (CiPA) project.

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The increase in rent expense for the quarter ended September 30, 2017 reflects higher commercial property rents prevalent in the South San Francisco real estate market and recognized in our November 2016 lease amendment extending the lease of our headquarters facilities in South San Francisco by five years from July 31, 2017 to July 31, 2022 and the related accounting for the extension amendment.

General and Administrative Expense

General and administrative expense, including both cash and noncash components, increased by 72% to approximately \$2.6 million from \$1.5 million for the quarters ended September 30, 2017 and 2016, respectively. Noncash expense aggregated approximately \$1,494,000 and \$579,000 for the quarters ended September 30, 2017 and 2016, respectively, including, in both periods, stock compensation expense, a portion of investor relations and investment banking expenses, warrant modification expense, and a portion of rent expense. The overall increase in general and administrative expenses resulted primarily from increased professional services expenses, including noncash expense attributable to the grant of common stock for services, noncash warrant modification expense and, to a lesser extent, salary and benefits and noncash stock compensation expenses. The following table indicates the primary components of general and administrative expenses, including the cash and noncash components, for each of the periods (amounts in thousands):

	Three Months Ended September 30,	
	2017	2016
Salaries and benefits	\$650	\$485
Stock-based compensation	194	117
Board fees	39	36
Legal, accounting and other professional fees	388	207
Investor relations	831	471
Insurance	60	38
Travel expenses	22	23
Rent and utilities	67	45
Warrant modification expense	280	17
All other expenses	36	55
	\$2,567	\$1,494

The increase in salaries and benefits primarily reflects the impact of the hiring of our Vice President of Corporate Development (VP-Corporate Development) in September 2016 and salary increases granted in July 2017 to our Chief Executive Officer (CEO), Chief Financial Officer (CFO), and VP-Corporate Development and, in June 2017, to a non-officer member of our administrative staff. Additionally, each of our officers was granted a bonus in September 2017, but our VP-Corporate Development had not yet joined the Company in July 2016, when our CEO and CFO received a bonus payment. The full amount of each officer's 2017 bonus was accrued at the time it was granted, but only 25% of the total amount was paid by September 30, 2017, with the remaining balance to be paid ratably through March 31, 2018.

Stock based compensation expense increased in the current period primarily as a result of the routine amortization of option grants to independent members of our Board and our CEO, CFO and administrative staff in September 2017, April 2017 and November 2016, plus the new-hire grant made to our VP-Corporate Development in September 2016, which grants account for approximately \$117,000 of 2017 expense. Expense attributable to these grants is being amortized over a two-year to four-year vesting period, based on the terms of the respective grants. Substantially all option grants made prior to September 2015 were fully-vested and fully-expensed prior to June 30, 2017.

Board fees includes fees recognized for the services of independent members of our Board. The Board modified committee assignments effective in April 2017, resulting in the slight increase in expense.

Legal, accounting and other professional fees for the quarters ended September 30, 2017 and 2016 includes cash expense related to routine legal services as well as accounting expense related to the review of the financial statements for the second quarter of each fiscal year. In the quarter ended September 30, 2017, additionally, we granted an aggregate of 20,000 unregistered shares of our common stock having an aggregate fair value of \$30,800 to legal services providers as compensation for services and an aggregate of 150,000 unregistered shares of our common stock having an aggregate fair value of \$234,000 to two investment banking firms pursuant to financial advisory agreements. In the quarter ended September 30, 2016, we granted an aggregate of 25,000 unregistered shares of our common stock having a fair value of \$108,500 to a legal services provider as compensation for services.

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Investor relations expense includes the fees of our various external service providers for a broad spectrum of investor relations, market awareness and strategic advisory and support functions, as well as initiatives that included numerous meetings in multiple U.S. markets and other communication activities focused on expanding market awareness of the Company, including among registered investment professionals and investment advisors, and individual and institutional investors. In the quarter ended September 30, 2017, in addition to cash fees and expenses we incurred, we granted an aggregate of 457,000 unregistered shares of our common stock to various corporate development, investor relations, market awareness and strategic business advisory service providers for their services and recognized noncash expense of \$713,300, representing the fair value of the stock at the time of issuance. In the quarter ended September 30, 2016, we granted an aggregate of 25,000 unregistered shares of our common stock having a fair value at the time of issuance of \$108,500 to an investor relations service provider as compensation for services.

In both periods, travel expense reflects costs associated with management presentations to and meetings in multiple U.S. markets with existing and potential individual and institutional investors, investment professionals and advisors, media, and securities analysts, as well as various investor relations, market awareness and corporate development initiatives.

The increase in rent expense for the quarter ended September 30, 2017 reflects higher commercial property rents prevalent in the local real estate market and recognized in our November 2016 lease amendment extending the lease of

our headquarters facilities by five years from July 31, 2017 to July 31, 2022 and the related accounting for the extension amendment.

In September 2017, we reduced the exercise price of 247,500 warrants issued in the Spring 2017 Private Placement from a weighted average exercise price of \$3.99 per share to \$2.00 per share. We also issued to each of the Spring 2017 Private Placement investors additional warrants to purchase an aggregate total of 247,501 shares of common stock, with an exercise price of \$2.00 per share. We recognized noncash expense of \$279,700 in the quarter ended September 30, 2017 representing the increase in fair value of the warrants granted initially before and after the modification and the fair value of the additional warrants granted. In August 2016, we entered into warrant exchange agreements with certain warrant holders pursuant to which the warrant holders exchanged outstanding warrants to purchase an aggregate of 20,000 shares of our common stock for an aggregate of 15,000 shares of our unregistered common stock. As we had with similar prior transactions, we accounted for these transactions as warrant modifications, resulting in our recognition of \$17,100 in noncash expense in the quarter ended September 30, 2016.

Interest and Other Expenses

Interest expense totaled \$3,300 for the quarter ended September 30, 2017 compared to \$1,400 reported for the quarter ended September 30, 2016. Interest expense in both periods relates to interest paid on insurance premium financing and on a capital lease of office equipment.

We recognized \$256,300 and \$241,000 for the quarters ended September 30, 2017 and 2016, respectively, representing the 10% cumulative dividend payable on our Series B Preferred as an additional deduction in arriving at net loss attributable to common stockholders in the accompanying Condensed Consolidated Statement of Operations and Comprehensive Loss included in Part I of this Report. There have been no conversions of outstanding shares of our Series B Preferred stock into shares of our common stock since August 2016.

Comparison of Six Months Ended September 30, 2017 and 2016

The following table summarizes the results of our operations, including both cash and noncash components, for the six months ended September 30, 2017 and 2016 (amounts in thousands).

	Six Months Ended September 30,	
	2017	2016
Operating expenses:		
Research and development	\$3,523	\$2,432
General and administrative	3,731	2,631
Total operating expenses	7,254	5,063
Loss from operations	(7,254)	(5,063)
Interest expense (net)	(6)	(3)

Loss before income taxes	(7,260)	(5,066)
Income taxes	(2)	(2)
Net loss	(7,262)	(5,068)
Accrued dividend on Series B Preferred Stock	(504)	(781)
Deemed dividend on Series B Preferred Stock	-	(111)
Net loss attributable to common stockholders	\$(7,766)	\$(5,960)

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Revenue

We reported no revenue for the six-month periods ended September 30, 2017 or 2016 and we presently have no recurring revenue generating arrangements with respect to AV-101 or other potential product candidates. While we may potentially receive future milestone payments and royalties under the BlueRock Agreement upon the achievement of certain performance-based milestones and commercial sales, there can be no assurance that the BlueRock Agreement will provide additional revenue to us in the near term or at all.

Research and Development Expense

Research and development expense, including both cash and noncash components, totaled \$3.5 million for the six months ended September 30, 2017, an increase of approximately 45% compared to the \$2.4 million reported for the six months ended September 30, 2016. Noncash expenses totaled approximately \$843,000 and \$131,000 in the six months ended September 30, 2017 and 2016, respectively, including stock compensation, depreciation and a portion of rent expense in both periods, and a portion of AV-101 project expenses in the six months ended September 30, 2017. The increase in research and development expense in 2017 reflects our continued focus on nonclinical and clinical development of AV-101, particularly our preparations for the launch of the AV-101 MDD Phase 2 Adjunctive Treatment Study, which is currently anticipated in the first quarter of 2018, subject to securing additional financing. The following table indicates the primary cash and noncash components of research and development expense for each of the periods (amounts in thousands):

	Six Months Ended September 30,	
	2017	2016
Salaries and benefits	\$884	\$711
Stock-based compensation	328	126
Consulting and other professional services	16	58
Technology licenses and royalties	157	254
Project-related research and supplies:		

AV-101	1,800	1,069
Stem cell and all other	93	79
	1,893	1,148
Rent	204	118
Depreciation	38	17
All other	3	-
Total Research and Development Expense	\$3,523	\$2,432

The increase in salaries and benefits reflects the hiring of our Chief Medical Officer (CMO) in June 2016, and salary increases granted to our CMO in July 2017, to our Chief Scientific Officer (CSO) and to the non-officer members of our scientific staff in June 2016 and June 2017. Additionally, our then-newly-hired CMO did not receive a bonus payment in July 2016; however, both our CMO and CSO were granted a bonus in September 2017. The full amount of each officer's September 2017 bonus was accrued at the time it was granted, but only 25% of the total amount was paid by September 30, 2017, with the remaining balance to be paid ratably through March 31, 2018.

Stock based compensation expense increased in the current period primarily as a result of the routine amortization of option grants made to our CSO, CMO and scientific staff in September 2017, April 2017 and November 2016, plus the new-hire grant made to our CMO in June 2016, which grants account for approximately \$193,000 of 2017 expense. The expense attributable to these grants is being amortized over a two-year to four-year vesting period, based on the terms of the respective grants. Substantially all option grants made prior to September 2015 were fully-vested and fully-expensed prior to June 30, 2017.

Consulting services reflects fees paid or accrued for scientific, nonclinical and clinical development and regulatory advisory services rendered to us by third-parties, primarily by members of our scientific and CNS clinical and regulatory advisory boards. The reduction in expense in 2017 primarily reflects the change in terms of consulting agreements with our stem cell-related scientific advisory board members.

Technology license expense reflects both recurring annual fees as well as legal counsel and other costs related to patent prosecution and protection pursuant to our stem cell technology license agreements or that we have elected to pursue for commercial purposes. We recognize these costs as they are invoiced to us by the licensors or counsel and they do not occur ratably throughout the year or between years. In both periods, but to a greater extent in 2016, this expense includes legal counsel and other costs we have incurred to advance numerous pending patent applications in the U.S. and various foreign countries with respect to AV-101 and our stem cell technology platform.

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AV-101 project expense for both periods includes continuing costs incurred to develop more efficient and cost-effective proprietary manufacturing methods for AV-101, and to produce clinical trial material enabling the AV-101 MDD Phase 2 Adjunctive Treatment Study, and, primarily in 2017, costs incurred for certain other nonclinical studies to facilitate further clinical development of AV-101 in MDD and potentially for other indications and to comply with applicable FDA regulations. Stem cell and other project related expenses reflects costs associated with our in-house stem cell technology-related initiatives, and, in 2016, our participation in the FDA's Comprehensive In Vitro Proarrhythmia Assay (CiPA) project.

The increase in rent expense for 2017 reflects higher commercial property rents prevalent in the South San Francisco real estate market and recognized in our November 2016 lease amendment extending the lease of our headquarters facilities in South San Francisco by five years, from July 31, 2017 to July 31, 2022, and the related accounting for the extension amendment.

General and Administrative Expense.

General and administrative expense, including both cash and noncash components, increased approximately 42% to \$3.7 million for the six months ended September 30, 2017 from \$2.6 million for the six months ended September 30, 2016. Noncash expense aggregated approximately \$1.7 million and \$1.0 million for the six months ended September 30, 2017 and 2016, respectively, including, in both periods, stock compensation expense, a portion of investor relations and investment banking expenses, warrant modification expense, and a portion of rent expense. The overall increase in general and administrative expenses was primarily attributable to increased salary and benefits expense and noncash stock compensation, professional services and warrant modification expenses. The following table indicates the primary components of general and administrative expenses, including noncash components, for each of the periods (amounts in thousands):

	Six Months Ended September 30,	
	2017	2016
Salaries and benefits	\$921	\$675
Stock-based compensation	370	181
Board fees	78	69
Legal, accounting and other professional fees	695	749
Investor relations	997	579
Insurance	121	78
Travel expenses	62	72
Rent and utilities	140	85
Warrant modification expense	280	57
All other expenses	67	86
	\$3,731	\$2,631

The increase in salaries and benefits reflects the impact of the hiring of our Vice President of Corporate Development (VP-Corporate Development) in September 2016 and salary increases granted in June 2016 and July 2017 to our Chief Executive Officer (CEO) and Chief Financial Officer (CFO), and in June 2016 and June 2017 to a non-officer member of our administrative staff. Additionally, each of our officers was granted a bonus in September 2017, but our VP-Corporate Development had not yet joined the Company in July 2016, when our CEO and CFO received a bonus payment. The full amount of each officer's 2017 bonus was accrued at the time it was granted, but only 25% of the total amount was paid by September 30, 2017, with the remaining balance to be paid ratably through March 31, 2018.

Stock based compensation expense increased in the current period primarily as a result of the routine amortization of option grants to independent members of our Board of Directors and our CEO, CFO and administrative staff in September 2017, April 2017 and November 2016, plus the new-hire grant made to our VP-Corporate Development in September 2016 in September 2016, which grants account for approximately \$232,000 of 2017 expense. The expense attributable to these grants is being amortized over a two-year to four-year vesting period based on the terms of the respective grants. Substantially all option grants made prior to September 2015 were fully-vested and fully-expensed prior to June 30, 2017.

Board fees includes fees recognized for the services of independent members of our Board. The Board modified committee assignments effective in April 2017, resulting in the slight increase in expense.

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Legal, accounting and other professional fees for the six months ended September 30, 2017 and 2016 includes expense related to routine legal services as well as the accounting expense related to the annual audit of the prior year's financial statements and the review of the financial statements for the first and second quarters of the current fiscal year. In addition to cash fees incurred, during the six months ended September 30, 2017, we granted an aggregate of 20,000 unregistered shares of our common stock having an aggregate fair value of \$30,800 to legal services providers as compensation for services and an aggregate of 150,000 unregistered shares of our common stock having an aggregate fair value of \$234,000 to two investment banking firms pursuant to financial advisory agreements. Noncash expense for the six months ended September 30, 2016 included approximately \$338,000 recognized in the quarter ended June 30, 2016 pursuant to the June 30, 2015 grant of an aggregate of 90,000 shares of our Series B 10% convertible preferred stock having an aggregate fair value of \$1,350,000 as compensation for financial advisory and corporate development service contracts with two independent providers for services to be performed through June 30, 2016 and \$108,500 recognized in the quarter ended September 30, 2016 representing the fair value of 25,000 unregistered shares of our common stock granted to a legal services provider as compensation for services.

Investor relations expense includes the fees of our various external service providers for a broad spectrum of investor relations, market awareness and strategic advisory and support functions, as well as initiatives that included numerous meetings in multiple U.S. markets and other communication activities focused on expanding market awareness of the Company, including among registered investment professionals and investment advisors, and individual and institutional investors. In the six months ended September 30, 2017, in addition to cash fees and expenses we incurred, in the quarter ended June 30, 2017, we granted 25,000 unregistered shares of our common stock to an investor relations and awareness service provider as partial compensation for its services and recognized noncash expense of approximately \$50,000 representing the fair value of the stock at the time of issuance; additionally, in the quarter ended September 30, 2017, we granted an aggregate of 457,000 unregistered shares of our common stock to various corporate development, investor relations, market awareness and business advisory service providers as compensation for their services and recognized noncash expense of \$713,300, representing the fair value of the stock at the time of issuance. In the six months ended September 30, 2016, we granted an aggregate of 25,000 unregistered shares of our common stock having a fair value at the time of issuance of \$108,500 to an investor relations service provider as compensation for its services.

In both periods, travel expense reflects costs associated with management presentations to and meetings in multiple U.S. markets with existing and potential individual and institutional investors, investment professionals and advisors, media, and securities analysts, as well as various investor relations, market awareness and corporate development initiatives.

The increase in rent expense for 2017 reflects higher commercial property rents prevalent in the South San Francisco real estate market and recognized in our November 2016 lease amendment extending the lease of our headquarters facilities in South San Francisco by five years, from July 31, 2017 to July 31, 2022, and the related accounting for the extension amendment.

In September 2017, we reduced the exercise price of 247,500 warrants issued in the Spring 2017 Private Placement from a weighted average exercise price of \$3.99 per share to \$2.00 per share. We also issued to each of the Spring 2017 Private Placement investors additional warrants to purchase an aggregate total of 247,501 shares of common

stock, with an exercise price of \$2.00 per share. We recognized noncash expense of \$279,700 in the six months ended September 30, 2017, representing the increase in fair value of the warrants granted initially before and after the modification and the fair value of the additional warrants granted. Between April 2016 and August 2016, we entered into warrant exchange agreements with certain warrant holders pursuant to which the warrant holders exchanged outstanding warrants to purchase an aggregate of 61,649 shares of our common stock for an aggregate of 46,238 shares of our unregistered common stock. As with similar prior transactions, we accounted for these transactions as warrant modifications, resulting in our recognition of an aggregate of \$57,400 in noncash expense during the six-month period ended September 30, 2016.

Interest and Other Expenses.

Interest expense totaled \$5,700 for the six months ended September 30, 2017 compared to \$2,800 reported for the six months ended September 30, 2016. Interest expense in both periods relates to interest paid on insurance premium financing and on a capital lease of office equipment.

We recognized \$503,600 and \$780,800 for the six months ended September 30, 2017 and 2016, respectively, representing the 10% cumulative noncash dividend payable on our Series B Preferred as an additional deduction in arriving at net loss attributable to common stockholders in the accompanying Condensed Consolidated Statement of Operations and Comprehensive Loss included in Part I of this Report. The reduction in the dividend accrual results from the automatic conversion of an aggregate of 2,403,051 shares of Series B Preferred into an equal number of shares of our common stock upon our completion of our May 2016 public offering of shares of our common stock and warrants, and a subsequent voluntary conversion of 87,500 shares of our Series B Preferred in August 2016. There have been no conversions of outstanding shares of Series B Preferred into common shares since August 2016.

During the quarter ended June 30, 2016, we allocated the proceeds from our self-placed private placement sales of Series B Preferred Units to the Series B Preferred stock and the Series B Warrants based on their relative fair values on the dates of the sales. The difference between the relative fair value per share of the Series B Preferred, approximately \$4.20 per share, and its Conversion Price (or stated value) of \$7.00 per share represented a deemed dividend to the purchasers of the Series B Preferred Units. Accordingly, we recognized a deemed dividend in the aggregate amount of \$111,100 in arriving at net loss attributable to common stockholders for the six months ended September 30, 2016 in the accompanying Condensed Consolidated Statement of Operations and Comprehensive Loss included in Part I of this Report.

Critical Accounting Policies and Estimates

We consider certain accounting policies related to revenue recognition, impairment of long-lived assets, research and development, stock-based compensation, warrant liability and income taxes to be critical accounting policies that require the use of significant judgments and estimates relating to matters that are inherently uncertain and may result in materially different results under different assumptions and conditions. The preparation of financial statements in conformity with United States generally accepted accounting principles (GAAP) requires us to make estimates and assumptions that affect the amounts reported in the financial statements and accompanying notes to the consolidated financial statements. These estimates include useful lives for property and equipment and related depreciation calculations, and assumptions for valuing options, warrants and other stock-based compensation. Our actual results could differ from these estimates.

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Revenue Recognition.

We have historically generated revenue principally from collaborative research and development arrangements, licensing and technology access fees and government grants. We recognize revenue under the provisions of the SEC issued Staff Accounting Bulletin 104, Topic 13, Revenue Recognition Revised and Updated (SAB 104) and Accounting Standards Codification (ASC) 605-25, Revenue Arrangements-Multiple Element Arrangements (ASC 605-25). Revenue for arrangements not having multiple deliverables, as outlined in ASC 605-25, is recognized once costs are incurred and collectability is reasonably assured.

Revenue arrangements with multiple components are divided into separate units of accounting if certain criteria are met, including whether the delivered component has stand-alone value to the customer. Consideration received is allocated among the separate units of accounting based on their respective selling prices. The selling price for each unit is based on vendor-specific objective evidence, or VSOE, if available, third party evidence if VSOE is not available, or estimated selling price if neither VSOE nor third party evidence is available. The applicable revenue recognition criteria are then applied to each of the units.

We recognize revenue when the four basic criteria of revenue recognition are met: (i) a contractual agreement exists; (ii) the transfer of technology has been completed or services have been rendered; (iii) the fee is fixed or determinable; and (iv) collectability is reasonably assured. For each source of revenue, we comply with the above revenue recognition criteria in the following manner:

Collaborative arrangements typically consist of non-refundable and/or exclusive technology access fees, cost reimbursements for specific research and development spending, and various future product development milestone and royalty payments. If the delivered technology does not have stand-alone value, the amount of revenue allocable to the delivered technology is deferred. Non-refundable upfront fees with stand-alone value that are not dependent on future performance under these agreements are recognized as revenue when received, and are deferred if we have continuing performance obligations and have no objective and reliable evidence of the fair value of those obligations. We recognize non-refundable upfront technology access fees under agreements in which we have a continuing performance obligation ratably, on a straight-line basis, over the period in which we are obligated to provide services. Cost reimbursements for research and development spending are recognized when the related costs are incurred and when collectability is reasonably assured. Payments received related to substantive, performance-based “at-risk” milestones are recognized as revenue upon achievement of the milestone event specified in the underlying contracts, which represent the culmination of the earnings process. Amounts received in advance are recorded as deferred revenue until the technology is transferred, costs are incurred, or a milestone is reached.

Technology license agreements typically consist of non-refundable upfront license fees, annual minimum access fees and/or royalty payments. Non-refundable upfront license fees and annual minimum payments received with separable stand-alone values are recognized when the technology is transferred or accessed, provided that the technology transferred or accessed is not dependent on the outcome of the continuing research and development efforts. Otherwise, revenue is recognized over the period of our continuing involvement.

Government grant awards, which support our research efforts on specific projects, generally provide for reimbursement of approved costs as defined in the terms of grant awards. We recognize grant revenue when associated project costs are incurred.

The Financial Accounting Standards Board (the FASB) has recently issued new guidance regarding revenue recognition. This new guidance will be effective for our fiscal year beginning April 1, 2018, with earlier adoption permitted. We have completed our initial assessment of the new guidance and will be developing an implementation plan to evaluate the accounting and disclosure requirements under the new guidance. Based on our assessment to date,

we do not believe that adoption of the new guidance will have a material impact on our consolidated financial statements. We have not yet finalized our transition method for adoption of the new guidance.

Impairment of Long-Lived Assets.

In accordance with ASC 360-10, Property, Plant & Equipment—Overall, we review for impairment whenever events or changes in circumstances indicate that the carrying amount of property and equipment may not be recoverable. Determination of recoverability is based on an estimate of undiscounted future cash flows resulting from the use of the asset and its eventual disposition. In the event that such cash flows are not expected to be sufficient to recover the carrying amount of the assets, we write down the assets to their estimated fair values and recognize the loss in the Consolidated Statements of Operations and Comprehensive Loss.

Research and Development Expenses.

Research and development expenses are composed of both internal and external costs. Internal costs include salaries and employment-related expenses of scientific personnel and direct project costs. External research and development expenses consist primarily of costs associated with manufacturing and nonclinical and clinical development of AV-101, our oral CNS prodrug candidate in Phase 2 clinical development for MDD, and, from-time-to-time, sponsored stem cell research and development, as well as costs related to the application and prosecution of patents related to AV-101 and our stem cell technology platform. All such costs are charged to expense as incurred.

Stock-Based Compensation.

We recognize non-cash compensation expense for all stock-based awards to employees based on the grant date fair value of the award. We record this expense over the period during which the employee is required to perform services in exchange for the award, which generally represents the scheduled vesting period. We have granted no restricted stock awards nor do we have any awards with market or performance conditions. For equity awards to non-employees, we re-measure the fair value of the awards as they vest and the resulting value is recognized as an expense during the period over which the services are performed.

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We use the Black-Scholes option pricing model to estimate the fair value of stock-based awards as of the grant date. The Black-Scholes model is complex and dependent upon key data input estimates. The primary data inputs with the greatest degree of judgment are the expected term of the stock options and the estimated volatility of our stock price. The Black-Scholes model is highly sensitive to changes in these two inputs. The expected term of the options represents the period of time that options granted are expected to be outstanding. We use the simplified method to estimate the expected term as an input into the Black-Scholes option pricing model. We determine expected volatility using the historical method, which, because of the limited period during which our stock has been publicly traded and its historically limited trading volume, is based on the historical daily trading data of the common stock of a peer group of public companies over the expected term of the option.

Warrant Liability.

Although we did not have a warrant liability at March 31, 2017 or 2016, in conjunction with certain Senior Secured Convertible Promissory Notes that we issued to Platinum Long Term Growth VII, LLC (PLTG) between October 2012 and July 2013 and the related warrants, and the contingently issuable Series A Exchange Warrant (collectively, the PLTG Warrants), we determined that the PLTG Warrants included certain exercise price and other adjustment features requiring them to be treated as non-cash liabilities. Accordingly, the PLTG Warrants were recorded at their issuance-date estimated fair values and marked to market at each subsequent reporting period, recording the change in the fair value as non-cash expense or non-cash income. The key component in determining the fair value of the PLTG Warrants and the related liability was the market price of our common stock, which is subject to significant fluctuation and is not under our control. The resulting change in the fair value of the warrant liability on our net income or loss was therefore also subject to significant fluctuation and would have continued to be so until all of the PLTG Warrants were issued and exercised, amended, cancelled or expired. Assuming all other fair value inputs remained generally constant, we recorded an increase in the warrant liability and non-cash losses when our stock price increased and a decrease in the warrant liability and non-cash income when our stock price decreased.

Notwithstanding the foregoing, on May 12, 2015, we entered into an agreement with PLTG pursuant to which we (i) fixed the exercise price of the PLTG Warrants at \$7.00 per share, (ii) eliminated the exercise price reset features and (iii) fixed the number of shares of our common stock issuable thereunder. This agreement and the related amendments to the PLTG Warrants resulted in the elimination of the warrant liability with respect to the PLTG Warrants during the quarter ending June 30, 2015. As further described in Note 9, Capital Stock, to the Consolidated Financial Statements for the years ended March 31, 2017 and 2016 elsewhere in this Prospectus, the PLTG Warrants, including the right to receive the Series A Exchange Warrant, were cancelled in exchange for our issuance of shares of our Series C Preferred stock to PLTG in January 2016.

Income Taxes.

We account for income taxes using the asset and liability approach for financial reporting purposes. We recognize deferred tax assets and liabilities for the future tax consequences attributable to differences between the financial statement carrying amounts of existing assets and liabilities and their respective tax bases and operating loss and tax credit carryforwards. Deferred tax assets and liabilities are measured using enacted tax rates expected to apply to taxable income in the years in which those temporary differences are expected to be recovered or settled. The effect on deferred tax assets and liabilities of a change in tax rates is recognized in income in the period that includes the enactment date. Valuation allowances are established, when necessary, to reduce the deferred tax assets to an amount expected to be realized.

Recent Accounting Pronouncements.

We believe the following recent accounting pronouncements or changes in accounting pronouncements are of significance or potential significance to the Company.

In May 2014, the Financial Accounting Standards Board (the FASB) issued ASU No. 2014-09, Revenue from Contracts with Customers (Topic 606), which supersedes all existing revenue recognition requirements, including most industry-specific guidance. The new standard requires a company to recognize revenue when it transfers goods or services to customers in an amount that reflects the consideration that the company expects to receive for those goods or services. The standard creates a five-step model that requires entities to exercise judgment when considering the recognition of revenue, including (1) identifying the contract(s) with the customer, (2) identifying the separate performance obligations in the contract, (3) determining the transaction price, (4) allocating the transaction price to the separate performance obligations, and (5) recognizing revenue as each performance obligation is satisfied. The standard also requires additional disclosure about the nature, amount, timing and uncertainty of revenue and cash flows arising from customer contracts, including qualitative and quantitative information about contracts with customers, significant judgments and changes in judgments and assets recognized with respect to costs incurred to obtain or fulfill a contract. The FASB has continued to issue accounting standards updates to clarify and provide implementation guidance related to Revenue from Contracts with Customers, including ASU 2016-08, Revenue from Contract with Customers: Principal versus Agent Considerations, ASU 2016-10, Revenue from Contracts with Customers: Identifying Performance Obligations and Licensing, and ASU 2016-12, Revenue from Contracts with Customers: Narrow-Scope Improvements and Practical Expedients. These amendments address a number of areas, including a company's identification of its performance obligations in a contract, collectability, non-cash consideration, presentation of sales tax and a company's evaluation of the nature of its promise to grant a license of intellectual property and whether or not that revenue is recognized over time or at a point in time. These new standards will be effective for our fiscal year beginning April 1, 2018, with earlier adoption permitted. We have completed our initial assessment of the new guidance and will be developing an implementation plan to evaluate the accounting and disclosure requirements under the new standards. Based on our assessment to date, we do not believe that adoption of Topic 606 and the related standards will have a material impact on our consolidated financial statements. We have not yet finalized our transition method for adoption of the new standards.

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In August 2014, the FASB issued ASU No. 2014-15, Presentation of Financial Statements—Going Concern (Subtopic 205-40): Disclosure of Uncertainties about an Entity’s Ability to Continue as a Going Concern (ASU 2014-15). The ASU sets forth a requirement for management to evaluate whether there are conditions or events that raise substantial doubt about an entity’s ability to continue as a going concern by incorporating and expanding upon certain principles that are currently in U.S. auditing standards. Specifically, the amendments (1) provide a definition of the term substantial doubt; (2) require an evaluation every reporting period, including interim periods; (3) provide principles for considering the mitigating effect of management’s plans; (4) require certain disclosures when substantial doubt is alleviated as a result of consideration of management’s plans; (5) require an express statement or other disclosures when substantial doubt is not alleviated; and (6) require an assessment for a period of one year after the date the financial statements are issued or available to be issued. Substantial doubt about an entity’s ability to continue as a going concern exists when relevant conditions and events, considered in the aggregate, indicate that it is probable (as defined under ASC 450, Contingencies) that the entity will be unable to meet its obligations as they become due within one year after the date that the financial statements are issued or are available to be issued. If substantial doubt exists, the extent of the required disclosures depends on an evaluation of management’s plans (if any) to mitigate the going concern uncertainty. This evaluation should include consideration of conditions and events that are either known or are reasonably knowable at the date the financial statements are issued or are available to be issued, as well as whether it is probable that management’s plans to address the substantial doubt will be implemented and, if so, whether it is probable that the plans will alleviate the substantial doubt. We adopted ASU 2014-15 for our fiscal year ended March 31, 2017 and Note 2, Basis of Presentation and Going Concern, includes our disclosures regarding substantial doubt about our ability to continue as a going concern and the steps we have planned to alleviate such doubt for the twelve months following the date of the issuance of these Consolidated Financial Statements.

In April 2015, the FASB issued ASU No. 2015-03, Interest - Imputation of Interest (Subtopic 835-30): Simplifying the Presentation of Debt Issuance Costs. The amendments in this ASU require that debt issuance costs related to a recognized debt liability be presented in the balance sheet as a direct deduction from the carrying amount of that debt liability, consistent with debt discounts. The amendments in this update are effective for financial statements issued for fiscal years ending after December 31, 2015, and interim periods within those fiscal years. We have adopted this ASU effective with our fiscal year beginning April 1, 2016, but have incurred no debt issuance costs since that date.

In November 2015, the FASB issued ASU No. 2015-17, Balance Sheet Classification of Deferred Taxes, which amends existing guidance on income taxes to require the classification of all deferred tax assets and liabilities as non-current on the balance sheet. We have adopted this ASU effective with our fiscal year beginning April 1, 2017 on a prospective basis. We do not expect this ASU to have a material impact on our consolidated financial statements.

In January 2016, the FASB issued ASU No. 2016-01, Financial Instruments - Overall: Recognition and Measurement of Financial Assets and Financial Liabilities. The updated guidance enhances the reporting model for financial instruments, which includes amendments to address aspects of recognition, measurement, presentation and disclosure. The amendment to the standard is effective for financial statements issued for our fiscal year beginning April 1, 2018. We do not believe that this ASU will have a material impact on our consolidated financial statements.

In February 2016, the FASB issued ASU 2016-02, Leases (ASC 842), which will replace the existing guidance in ASC 840, Leases, and which sets out the principles for the recognition, measurement, presentation and disclosure of leases for both parties to a contract (i.e. lessees and lessors). The new standard requires lessees to apply a dual approach, classifying leases as either finance or operating leases based on the principle of whether or not the lease is effectively a financed purchase by the lessee. This classification will determine whether lease expense is recognized based on an effective interest method or on a straight-line basis over the term of the lease, respectively. A lessee is also required to record a right-of-use asset and a lease liability for all leases with a term of greater than 12 months regardless of their classification. Leases with a term of 12 months or less will be accounted for similar to the current

guidance for operating leases. The standard is effective for financial statements issued for fiscal years beginning after December 15, 2018, and interim periods within those fiscal years, with early adoption permitted. We are in the process of evaluating the impact that this new guidance will have on our consolidated financial statements.

In March 2016, the FASB issued ASU No. 2016-09, Compensation—Stock Compensation (Topic 718): Improvements to Employee Share-Based Payment Accounting which includes multiple provisions intended to simplify several aspects of accounting for share-based payment transactions, including income tax consequences, classification of awards as either equity or liabilities, an option to recognize gross stock compensation expense with actual forfeitures recognized as they occur, as well as certain classifications on the statement of cash flows. The standard is effective for our fiscal year beginning April 1, 2017. We are evaluating the impact of this ASU on our consolidated financial statements.

Liquidity and Capital Resources

From our inception in May 1998 through September 30, 2017, we have financed our operations and technology acquisitions primarily through the issuance and sale of our equity and debt securities, including convertible promissory notes and short-term promissory notes, for cash proceeds of approximately \$47.6 million, as well as from an aggregate of approximately \$17.6 million of government research grant awards, strategic collaboration payments, intellectual property sublicensing and other revenues. We have also issued equity securities with an approximate aggregate value at issuance of \$32.3 million in non-cash settlements of certain liabilities, including liabilities for professional services rendered to us or as compensation for such services. Additionally, pursuant to our CRADA with the NIH, the NIMH continues to conduct Phase 2 clinical development activities relating to AV-101 as a potential new generation antidepressant. The NIMH AV-101 MDD Phase 2 Monotherapy Study is being sponsored in full, at no cost to us other than supplying clinical trial material, by the NIMH under the direction of Dr. Carlos Zarate Jr. as Principal Investigator.

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Between late-March 2017 and June 30, 2017, we sold to accredited investors, in a self-placed private placement, units consisting of an aggregate of 495,001 unregistered shares of our common stock and warrants to purchase an aggregate of 247,500 unregistered shares of our common stock pursuant to which we received proceeds of approximately \$1.0 million (the Spring 2017 Private Placement), resulting in our cash and cash equivalents balance of \$1.6 million at June 30, 2017. In August 2017, in a self-placed private placement transaction, we sold to an accredited investor units consisting of 28,572 shares of our unregistered common stock and warrants to purchase 28,572 unregistered shares of our common stock at an exercise price of \$4.00 per share. We received cash proceeds of \$50,000 from this sale of our securities. In September, we completed an underwritten, public offering of shares of our common stock and warrants, pursuant to which we received net proceeds of approximately \$2.0 million. Our cash balance at June 30, 2017 plus the proceeds from subsequent sales of our securities was not sufficient to enable us to fund our planned operations, including expected cash expenditures of approximately \$12 million for the next twelve months, including expenditures required to further prepare for, launch and satisfy a significant portion of the projected expenses associated with our proposed AV-101 MDD Phase 2 Adjunctive Treatment Study.

In September 2017, we completed a firm commitment underwritten public offering, pursuant to which we offered and sold an aggregate of 1,371,430 shares of our common stock and warrants to purchase an aggregate of 1,892,572 shares of our common stock to two of our existing institutional investors, resulting in gross proceeds of approximately \$2.4 million under our Registration Statement on Form S-3 (Registration No. 333-215671) (the S-3 Registration Statement) that had been previously declared effective by the Securities and Exchange Commission to cover potential future sales of our equity securities in one or more public offerings from time to time. While we may make additional sales of our equity securities under the S-3 Registration Statement, we do not have an obligation to do so.

Additionally, in September 2017, to facilitate potential future issuances and sales of our equity securities for ordinary corporate finance and general corporate purposes and as recommended by our Board, our stockholders approved an amendment to our Restated and Amended Articles of Incorporation to increase the number of shares of common stock available for issuance from 30 million shares to 100 million shares, an amount our Board determined is customary and appropriate for a NASDAQ-listed, clinical-stage biopharmaceutical company. Following that approval, in October 2017, we filed a Registration Statement on Form S-1 (Registration No. 333-221009) (the S-1 Registration Statement) pursuant to which we anticipate completing an offering of our equity securities with proceeds sufficient to enable the launch and completion of the AV-101 MDD Phase 2 Adjunctive Treatment Study and fund our internal operations for at least the next twelve months. There can be no assurance, however, that we will achieve effectiveness of the S-1 Registration Statement or successfully complete an offering thereunder.

Although our current financial resources are not yet sufficient to fully fund completion of the AV-101 MDD Phase 2 Adjunctive Treatment Study, we anticipate, as we have numerous times in the past, raising sufficient additional capital as and when necessary and advisable to sustain our operations and achieve our key corporate objectives through at least the next twelve months, including initiating and conducting the AV-101 MDD Phase 2 Adjunctive Treatment Study in an ordinary course manner. We expect to secure additional capital primarily through the sale of our equity securities in one or more private placements to accredited investors or public offerings. We have filed a Registration Statement on Form S-3 (Registration No. 333-215671) (the S-3 Registration Statement) that has been declared effective by the Securities and Exchange Commission (the Commission) to cover our potential future sale of our equity securities in one or more public offerings from time to time. As of the date of this prospectus, we have sold approximately \$6.28 million of securities under the S-3 Registration Statement.

We may also seek research and development collaborations that could generate revenue, funding for development of AV-101 and additional product candidates, as well as additional government grant awards and agreements similar to our current CRADA with the NIMH, which provides for the NIMH to fully fund the NIMH's ongoing NIMH AV-101 MDD Phase 2 Monotherapy Study. Such strategic collaborations may provide non-dilutive resources to advance our

strategic initiatives while reducing a portion of our future cash outlays and working capital requirements. In a manner similar to the BlueRock Agreement, we may also pursue similar arrangements with third-parties covering other of our intellectual property. Although we may seek additional collaborations that could generate revenue and/or non-dilutive funding for development of AV-101 and other product candidates, as well as new government grant awards and/or agreements similar to our CRADA with NIMH, no assurance can be provided that any such collaborations, awards or agreements will occur in the future.

Our future working capital requirements will depend on many factors, including, without limitation, the scope and nature of opportunities related to our success and the success of certain other companies in clinical trials, including our development and commercialization of AV-101 as an adjunctive treatment for MDD and other potential CNS conditions, and various applications of our stem cell technology platform, the availability of, and our ability to obtain, government grant awards and agreements, and our ability to enter into collaborations on terms acceptable to us. To further advance the clinical development of AV-101 and our stem cell technology platform, as well as support our operating activities, we plan to continue to carefully manage our routine operating costs, including our employee headcount and related expenses, as well as the timing of and projected costs relating to key research and development projects, including our expenses associated with our proposed AV-101 MDD Phase 2 Adjunctive Treatment Study, regulatory consulting, CRO services, investor relations and corporate development, legal, acquisition and protection of intellectual property, public company compliance and other professional services and working capital costs.

Notwithstanding the foregoing, substantial additional financing may not be available to us on a timely basis, on acceptable terms, or at all. If we are unable to obtain substantial additional financing on a timely basis when needed in 2017 and beyond, our business, financial condition, and results of operations may be harmed, the price of our stock may decline, we may be required to reduce, defer, or discontinue certain of our research and development activities and we may not be able to continue as a going concern.

Cash and Cash Equivalents.

The following table summarizes changes in cash and cash equivalents for the periods stated (in thousands):

	Six Months Ended September 30,		Fiscal Years Ended March 31,	
	2017	2016	2017	2016
Net cash used in operating activities	\$(4,010)	\$(3,873)	\$(7,263)	\$(4,808)
Net cash used in investing activities	(2)	(8)	(239)	(26)
Net cash provided by financing activities	2,855	9,709	9,994	5,193
Net increase in cash and cash equivalents	(1,157)	5,828	2,492	359
Cash and cash equivalents at beginning of period	2,921	429	429	70
Cash and cash equivalents at end of period	\$1,764	\$6,257	\$2,921	\$429

Off-Balance Sheet Arrangements

We have no off-balance sheet arrangements.

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BUSINESS

Business Overview

We are a clinical-stage biopharmaceutical company focused on developing new generation medicines for depression and other central nervous system (CNS) disorders. Unless the context otherwise requires, the words “VistaGen Therapeutics, Inc.” “VistaGen,” “we,” “the Company,” “us” and “our” refer to VistaGen Therapeutics, Inc., a Nevada corporation. All references to future quarters and years in this prospectus supplement refer to calendar quarters and calendar years, unless reference is made otherwise.

AV-101 is our oral CNS product candidate in Phase 2 clinical development in the United States, initially as a new generation adjunctive treatment for Major Depressive Disorder (MDD) in patients with an inadequate response to standard antidepressants approved by the U.S. Food and Drug Administration (FDA). During the years ending March 31, 2017 and 2016, we spent approximately \$5.2 million and \$3.9 million, respectively, on research and development, including development of AV-101. AV-101’s mechanism of action (MOA) involves both NMDA (N-methyl-D-aspartate) and AMPA (alpha-amino-3-hydroxy-5-methyl-4-isoxazolepropionic acid) receptors in the brain responsible for fast excitatory synaptic activity throughout the CNS. AV-101’s MOA is fundamentally differentiated from all FDA-approved antidepressants, as well as all atypical antipsychotics such as aripiprazole often used adjunctively to augment them. We believe AV-101 also has potential as a non-opioid treatment alternative for neuropathic pain, as well as several additional CNS indications where modulation of the NMDA receptors, activation of AMPA pathways and/or key active metabolites of AV-101 may achieve therapeutic benefit, including Parkinson’s disease levodopa -induced dyskinesia (PD LID), epilepsy and Huntington’s disease.

Clinical studies conducted at the U.S. National Institute of Mental Health (NIMH), part of the U.S. National Institutes of Health (NIH), by Dr. Carlos Zarate, Jr., Chief of the NIMH’s Experimental Therapeutics & Pathophysiology Branch and its Section on Neurobiology and Treatment of Mood and Anxiety Disorders, have focused on the antidepressant effects of an FDA-approved anesthetic, ketamine hydrochloride injection (ketamine), an ion-channel blocking NMDA receptor antagonist, in MDD patients with inadequate responses to multiple standard antidepressants. These NIMH studies, as well as clinical research at Yale University and other academic institutions, have demonstrated robust antidepressant effects in treatment-resistant MDD patients within twenty-four hours of a single sub-anesthetic dose of ketamine administered by intravenous (IV) injection.

We believe AV-101 may have potential to deliver ketamine-like antidepressant effects, without the need for IV administration and ketamine’s psychological and other negative side effects. As published in the October 2015 issue of the peer-reviewed Journal of Pharmacology and Experimental Therapeutics, in an article titled, The prodrug 4-chlorokynurenine causes ketamine-like antidepressant effects, but not side effects, by NMDA/glycineB-site inhibition, using well-established preclinical models of depression, AV-101 was shown to induce fast-acting, dose-dependent, persistent and statistically significant antidepressant-like responses following a single treatment. These responses were equivalent to those seen with a single sub-anesthetic control dose of ketamine. In addition, these studies confirmed that the fast-acting antidepressant effects of AV-101 were mediated through both inhibiting the GlyB site of the NMDA receptor and activating the AMPA receptor pathway in the brain.

Pursuant to our Cooperative Research and Development Agreement (CRADA) with the NIMH, the NIMH is currently funding, and Dr. Zarate, as Principal Investigator, and his team are currently conducting, a small Phase 2 clinical study of AV-101 monotherapy in subjects with treatment-resistant MDD (the NIMH AV-101 MDD Phase 2 Monotherapy Study). In October 2017, we received FDA authorization to launch our 180-patient Phase 2 multi-center, multi-dose, double blind, placebo-controlled efficacy and safety study of AV-101 as a new generation adjunctive treatment of MDD in adult patients with an inadequate therapeutic response to standard, FDA-approved antidepressants

(the AV-101 MDD Phase 2 Adjunctive Treatment Study). Subject to completion of this offering, we intend to launch the AV-101 MDD Phase 2 Adjunctive Treatment Study in the first quarter of 2018 with Dr. Maurizio Fava, Professor of Psychiatry at Harvard Medical School and Director, Division of Clinical Research, Massachusetts General Hospital (MGH) Research Institute, as the Principal Investigator. Dr. Fava was the co-Principal Investigator with Dr. A. John Rush of the STAR*D study, the largest clinical trial conducted in depression to date, whose findings were published in journals such as the New England Journal of Medicine (NEJM) and the Journal of the American Medical Association (JAMA). If started in the first quarter of 2018, we expect to complete this study by the end of 2018, with top line results available in the first quarter of 2019.

VistaStem Therapeutics (VistaStem) is our wholly owned subsidiary focused on applying human pluripotent stem cell (hPSC) technology, internally and with collaborators, to discover, rescue, develop and commercialize (i) proprietary new chemical entities (NCEs) for CNS and other diseases and (ii) regenerative medicine (RM) involving hPSC-derived blood, cartilage, heart and liver cells. Our internal drug rescue programs are designed to utilize CardioSafe 3D, our customized cardiac bioassay system, to develop small molecule NCEs for our pipeline. To advance potential RM applications of its cardiac stem cell technology, in December 2016, we exclusively sublicensed to BlueRock Therapeutics LP, a next generation RM company established by Bayer AG and Versant Ventures (BlueRock Therapeutics), rights to certain proprietary technologies relating to the production of cardiac stem cells for the treatment of heart disease (the BlueRock Agreement). In a manner similar to our exclusive sublicense agreement with BlueRock Therapeutics, we may pursue additional RM collaborations or licensing transactions involving blood, cartilage, and/or liver cells derived from hPSCs for (A) cell-based therapy, (B) cell repair therapy, and/or (C) tissue engineering.

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AV-101 and Major Depressive Disorder

Background

The World Health Organization (WHO) estimates that 300 million people worldwide are affected by depression. According to the NIH, major depression is one of the most common mental disorders in the U.S. The NIMH reports that, in 2014, approximately 16 million adults in the U.S. had at least one major depressive episode in the past year. According to the U.S. Centers for Disease Control and Prevention (CDC) one in 10 Americans over the age of 12 takes a standard, FDA-approved antidepressant.

Most standard antidepressants target neurotransmitter reuptake inhibition – either serotonin (antidepressants known as SSRIs) or serotonin/norepinephrine (antidepressants known as SNRIs). Even when effective, these standard antidepressants take many weeks to achieve adequate therapeutic effects. Nearly two out of every three drug-treated depression patients do not obtain adequate therapeutic benefit from initial treatment with a standard antidepressant. Even after treatment with many different standard antidepressants, nearly one out of every three drug-treated depression patients still do not achieve adequate therapeutic benefits from their antidepressant medication. Such patients with an inadequate response to standard antidepressants often seek to augment their treatment regimen by adding an atypical antipsychotic (drugs such as aripiprazole), despite only modest potential therapeutic benefit and the significant risk of additional side effects.

All standard antidepressants have risks of side effects, including, among others, anxiety, metabolic syndrome, sleep disturbance and sexual dysfunction. Adjunctive use of atypical antipsychotics to augment inadequately performing standard antidepressants may increase the risk of significant side effects, including, tardive dyskinesia, substantial weight gain, diabetes and heart disease, while offering only a modest potential increase in therapeutic benefit.

AV-101

AV-101 is our oral CNS product candidate in Phase 2 development in the United States, initially focused as a new generation antidepressant for the adjunctive treatment of MDD patients with an inadequate response to standard, FDA-approved antidepressants. As published in the October 2015 issue of the peer-reviewed, Journal of Pharmacology and Experimental Therapeutics, in an article titled, The prodrug 4-chlorokynurenine causes ketamine-like antidepressant effects, but not side effects, by NMDA/glycineB-site inhibition, using well-established preclinical models of depression, AV-101 was shown to induce fast-acting, dose-dependent, persistent and statistically significant ketamine-like antidepressant effects following a single treatment, responses equivalent to those seen with a single sub-anesthetic control dose of ketamine, but without the negative side effects seen with ketamine. In addition, these studies confirmed that the antidepressant effects of AV-101 were mediated through both inhibition of the GlyB site of NMDA receptors and activation of the AMPA receptor pathway in the brain, a key final common pathway feature of certain new generation antidepressants such as ketamine and AV-101, each with a MOA that is fundamentally different from all standard antidepressants and atypical antipsychotics used adjunctively to augment them.

We have completed two NIH-funded, randomized, double blind, placebo-controlled AV-101 Phase 1 safety studies. Pursuant to our CRADA with the NIMH and Dr. Carlos Zarate, Jr., the NIMH is funding, and Dr. Zarate, as Principal Investigator, and his team are conducting, a small NIMH AV-101 MDD Phase 2 Monotherapy Study. Although we are not involved in conducting this study, we currently anticipate that the NIMH will complete the NIMH AV-101 MDD Phase 2 Monotherapy Study during the first half of 2018.

We are preparing to begin the AV-101 MDD Phase 2 Adjunctive Treatment Study, which will test the safety, efficacy and tolerability of AV-101 as an adjunctive treatment of MDD in adult patients with an inadequate response to standard, FDA-approved antidepressants. Subject to receiving sufficient financing, , we intend to launch the AV-101 MDD Phase 2 Adjunctive Treatment Study in the first quarter of 2018. In connection with our preparation for this study, as well as potential Phase 3 development and commercialization of AV-101, we, together with our contract manufacturing organization (CMO), developed a novel process for the production of AV-101 drug substance. We believe our new proprietary production process will significantly improve AV-101 manufacturing efficiency, thereby reducing the current and future cost of manufacturing AV-101 drug substance and improving the yield of AV-101 drug substance manufactured. While developing our new manufacturing process, our CMO produced a batch of AV-101 drug substance that contained certain impurities not within the limits set out by the International Conference on Harmonisation of Technical Requirements for Registration of Pharmaceuticals for Human Use (the ICH Guidelines). The FDA routinely utilizes the ICH Guidelines as an industry standard for development stage programs such as our AV-101 Phase 2 program. Consequently, the FDA placed a clinical hold on the launch of the AV-101 MDD Phase 2 Adjunctive Treatment Study until we either (a) further improved our AV-101 manufacturing process to remove the impurities or reduce the impurities below applicable limits in the ICH Guidelines, or (b) conducted a bridging toxicology study to qualify the impurities as safe for clinical use. In response to the FDA's requests, we did both. We further improved our AV-101 manufacturing process, produced a new batch of AV-101 drug substance using the improved process, and now have analytical results from that batch showing that the impurities were reduced to a level below the limits of the ICH Guidelines. In addition, we conducted a bridging toxicology study, the results of which confirmed that the impurities found in the AV-101 drug substance tested were safe for clinical use.

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The FDA also requested additional contraceptive protection in the upcoming clinical study until we complete preclinical reproductive toxicology studies that are routinely conducted later in stages of clinical development. Although previous toxicology studies for AV-101 do not suggest any reproductive organ involvement, and we have confirmed with the FDA that our proposed study is a Phase 2 study, we have implemented additional contraceptive measures in the revised protocol for the AV-101 MDD Phase 2 Adjunctive Treatment Study that will remain in effect until standard reproductive toxicology studies are completed in the ordinary course prior to commencement of Phase 3 development. We have confirmed with the FDA that our recently implemented contraceptive measures are appropriate for the current stage of development of AV-101.

In September 2017, we requested, and were granted, a pre-IND Type A meeting with the FDA's Division of Psychiatry to discuss certain matters pertaining to our AV-101 development program and the AV-101 MDD Phase 2 Adjunctive Treatment Study. Subsequent to our Type A Meeting with the FDA, we submitted the supplemental data from our new manufacturing process, the bridging toxicology report and our study protocol, revised to address the FDA's comments. These documents addressed all concerns raised by the FDA to date. Consequently, on October 26, 2017, the FDA authorized us to proceed, under our Investigational New Drug (IND) application, with the AV-101 MDD Phase 2 Adjunctive Treatment Study. We believe preclinical studies and Phase 1 safety studies support our hypothesis that AV-101 also has potential as a non-opioid treatment alternative for neuropathic pain, as well as several additional CNS indications where modulation of the NMDA receptors, activation of AMPA pathways and/or key active metabolites of AV-101 may achieve therapeutic benefit, including Parkinson's disease levodopa induced dyskinesia (PD LID), epilepsy and Huntington's disease. We are beginning to plan additional Phase 2 clinical studies to further evaluate the therapeutic potential of AV-101 beyond MDD.

CardioSafe 3D™; NCE Drug Rescue and Regenerative Medicine

VistaStem Therapeutics is our wholly owned subsidiary focused on applying hPSC technology to discover, rescue, develop and commercialize proprietary small molecule NCEs for CNS and other diseases, as well as potential cellular therapies involving stem cell-derived blood, cartilage, heart and liver cells. CardioSafe 3D™ is our customized in vitro cardiac bioassay system capable of predicting potential human heart toxicity of small molecule NCEs in vitro, long before they are ever tested in animal and human studies. Potential commercial applications of our stem cell technology platform involve using CardioSafe 3D internally for NCE drug discovery and drug rescue to expand our proprietary drug candidate pipeline. Drug rescue involves leveraging substantial prior research and development investments by pharmaceutical companies and others related to public domain NCE programs terminated before FDA approval due to heart toxicity risks and RM and cellular therapies. To advance potential RM applications of our cardiac stem cell technology, in December 2016, we exclusively sublicensed to BlueRock Therapeutics LP, a next generation regenerative medicine company established by Bayer AG and Versant Ventures, rights to certain proprietary technologies relating to the production of cardiac stem cells for the treatment of heart disease. In a manner similar to the BlueRock Agreement, we may also pursue additional potential RM applications using blood, cartilage, and/or liver cells derived from hPSCs for (A) cell-based therapy (injection of stem cell-derived mature organ-specific cells obtained through directed differentiation), (B) cell repair therapy (induction of regeneration by biologically active molecules administered alone or produced by infused genetically engineered cells), or (C) tissue engineering (transplantation of in vitro grown complex tissues) using hPSC-derived blood, bone, cartilage, and/or liver cells.

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Our Strategy

Our core strategy is to develop and commercialize innovative small molecule drugs that address significant unmet medical needs related to CNS diseases and disorders. We have assembled a management team and a team of scientific, clinical, and regulatory advisors, including recognized experts in the fields of depression and other CNS disorders, with significant pharmaceutical industry and regulatory experience to lead and execute the development and commercialization of our CNS product candidate opportunities. Key elements of our strategy are to:

Develop and commercialize our lead CNS product candidate, AV-101, initially as a new generation adjunctive treatment for MDD patients with an inadequate response to standard, FDA-approved antidepressants. We are currently pursuing adjunctive treatment of MDD as our lead CNS indication for AV-101. We are preparing to launch our 180-patient Phase 2 study of AV-101 for the adjunctive treatment of MDD in patients with an inadequate response to standard antidepressants. We intend to develop AV-101 internally, through a pivotal Phase 3 clinical program focused on adjunctive treatment of MDD, accompanied by submission of our New Drug Application (NDA) for AV-101 to the FDA. If our NDA is approved by the FDA, we plan to commercialize AV-101 for this indication in the U.S. either by (A) collaborating with a large pharmaceutical company with a strong commercial presence in global depression and other CNS markets and/or (B) contracting for and/or establishing a specialty sales force support focused primarily on psychiatrists and long-term care physicians who prescribe standard antidepressants and atypical antipsychotics for treatment of their MDD patients under the current and evolving MDD drug treatment paradigm.

Leverage the commercial potential of AV-101 by expanding Phase 2 development to include additional CNS-related disorders and diseases. We intend to pursue clinical development and commercialization of AV-101 across multiple CNS-related indications that are underserved by currently available medicines and represent significant unmet medical needs. Based on AV-101 preclinical studies, our successful NIH-funded AV-101 Phase 1a and 1b clinical safety studies, and regulatory submissions related to the AV-101 MDD Phase 2 Adjunctive Treatment Study, we believe AV-101 also has potential as a non-opioid treatment alternative for neuropathic pain, as well as several additional CNS indications where modulation of the NMDA receptors, activation of AMPA pathways and/or key active metabolites of AV-101 may achieve therapeutic benefit, including PD LID, epilepsy and Huntington's disease.

Pursue in-licensing and acquisition of additional CNS product candidates. While our resources are currently focused primarily on development of AV-101 for MDD and additional CNS indications, we anticipate pursuing acquisition of additional CNS-related product candidates in the future. We believe that a diversified CNS product candidate portfolio, combined with our internal and collaborative network of CNS drug development expertise and ecosystem, will mitigate risks inherent in drug development and increase the likelihood of our success.

Capitalize on our drug rescue and RM opportunities using our stem cell technology platform. We are focused on using our cardiac stem cell technology to screen and develop proprietary NCEs through drug rescue programs intended to produce proprietary NCEs for our internal drug development pipeline, without incurring many of the substantial costs and risks typically inherent in new drug discovery and nonclinical drug development. In order to capitalize on our existing stem cell technology, we may establish additional strategic collaborations similar to the BlueRock Agreement, as well as investigating potential spin-off opportunities. As most of our resources are currently focused on the nonclinical and clinical development activities we believe are necessary to advance AV-101 through Phase 2, into pivotal Phase 3 development and ultimately to market approval, a strategic collaboration or spin-off involving our stem cell technology could allow us to capitalize on our existing stem cell technology and shift our focus exclusively

to developing our CNS pipeline.

AV-101 (L-4-cholorkyurenine or 4-Cl-KYN)

Overview and Mechanism of Action

AV-101 is an orally available, clinical-stage prodrug candidate that readily gains access to the CNS after systemic administration and is rapidly converted in vivo into its active metabolite, 7-chlorokynurenic acid (7-Cl-KYNA), a well-characterized, potent and highly selective antagonist of the NMDA receptor at its GlyB co-agonist site.

Current evidence suggests that AV-101's antagonism of NMDA receptor signaling may provide faster-acting antidepressant effects in the treatment of MDD than standard antidepressants. In addition, as confirmed in our AV-101 Phase 1 clinical studies, targeting the GlyB site of the NMDA receptor does not have the negative side effects typically associated with standard antidepressants, atypical antipsychotics often used adjunctively in the current MDD drug treatment paradigm to augment them, or classic ion channel-blocking NMDA receptor antagonists, such as ketamine.

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Major Depressive Disorder

Depression is a serious medical illness and a global public health concern. The WHO estimates that depression is the leading cause of disability worldwide, and is a major contributor to the global burden of disease, affecting 300 million people globally. According to the CDC, approximately one in every 10 Americans aged 12 and over takes antidepressant medication.

While most people will experience depressed mood at some point during their lifetime, MDD is different. MDD is the chronic, pervasive feeling of utter unhappiness and suffering, which impairs daily functioning. Symptoms of MDD include diminished pleasure in activities, changes in appetite that result in weight changes, insomnia or oversleeping, psychomotor agitation, loss of energy or increased fatigue, feelings of worthlessness or inappropriate guilt, difficulty thinking, concentrating or making decisions, and thoughts of death or suicide and attempts at suicide. Suicide is estimated to be the cause of death in up to 15% individuals with MDD.

Standard Antidepressants

For many people, depression cannot be controlled for any length of time without treatment. Standard antidepressant medications available in the multi-billion dollar global depression market, including commonly-prescribed SSRIs and SNRIs, have limited effectiveness, and, because of their mechanism of action, must be taken for several weeks before patients experience any significant therapeutic benefit. About two out of every three depression sufferers, including over an estimated 6.0 million drug-treated MDD patients in the U.S., do not receive adequate therapeutic benefits from their initial treatment with a standard antidepressant, and the likelihood of achieving remission of depressive symptoms declines with each successive treatment attempt. Even after multiple treatment attempts, approximately one out of every three depression sufferers still fails to find an adequately effective standard antidepressant. In addition, this trial and error process and the systemic effects of the various antidepressants involved may increase the risk of patient tolerability issues and serious side effects, including suicidal thoughts and behaviors in certain groups.

Ketamine and NIH Clinical Studies in Major Depressive Disorder

Ketamine hydrochloride is an FDA-approved, rapid-acting general anesthetic currently administered only by intravenous or intramuscular injection. The use of ketamine (an NMDA receptor antagonist which acts as an NMDA ion channel blocker) to treat MDD has been studied in several clinical trials conducted by depression experts at Yale University and other academic institutions and at the NIMH, part of the NIH, including by Dr. Carlos Zarate, Jr., the NIMH's Chief of Experimental Therapeutics & Pathophysiology Branch and of the Section on Neurobiology and Treatment of Mood and Anxiety Disorders. In randomized, placebo-controlled, double blind clinical trials reported by Dr. Zarate and others at the NIMH, a single sub-anesthetic dose of ketamine (0.5 mg/kg over 40 minutes) produced robust and rapid (within twenty-four hours) antidepressant effects in MDD patients who had not responded to standard antidepressants. These results were in sharp contrast to the very slow onset of standard antidepressants (SSRIs and SNRIs) that usually require many weeks of chronic usage to achieve similar antidepressant effects. The potential for widespread therapeutic use of current FDA-approved ketamine, a Schedule III drug, for MDD is limited by its potential for abuse, dissociative and psychosis-like side effects and by current practical challenges associated with the necessity of I.V. administration in a medical center. Notwithstanding these limitations, however, the discovery of ketamine's fast-acting antidepressant effects revolutionized thinking about the current MDD drug treatment paradigm and catalyzed development of a new generation of antidepressants with a faster-acting mechanisms of action (MOA) similar to ketamine's. Our oral CNS drug candidate, AV-101 is among a new generation of antidepressants with potential to deliver faster-acting antidepressant effects than standard antidepressants, without the side effects typically associated with standard antidepressants, atypical antipsychotics and ketamine.

AV-101, Mechanism of Action, and Major Depressive Disorder

AV-101 (4-Cl-KYN) is an orally available prodrug candidate that produces, in the brain, 7-Cl-KYNA, one of the most potent and selective antagonists of the GlyB site of the NMDA receptor, resulting in the down-regulation of NMDA receptor signaling. Growing evidence suggests that glutamatergic activation involving AMPA receptors is central to the neurobiology and treatment of MDD and other mood disorders.

AV-101's mechanism of action (MOA) is fundamentally differentiated from the MOA of all standard, FDA-approved antidepressants and all atypical antipsychotics often used adjunctively to augment inadequate response to standard antidepressants, placing AV-101 among a new generation of antidepressants with potential to treat millions of MDD sufferers worldwide who are poorly served by SSRIs, SNRIs and other current depression therapies. AV-101 is functionally similar to ketamine in that both induce final common pathway antidepressant activity via glutamatergic activation involving AMPA receptors. However, AV-101 inhibits NMDA receptor channel activity, whereas ketamine blocks the ion channel of the NMDA receptor. AV-101, as a prodrug, produces in the brain an antagonist that inhibits the NMDA receptor by selectively binding to its functionally required GlyB site. Experimental evidence confirms that inhibiting the NMDA receptor by targeting the GlyB site can produce potent antidepressive effects and bypass adverse effects that result when ketamine blocks the NMDA receptor ion channel. Experimental evidence also supports the conclusion that this NMDA receptor inhibition by AV-101 may then result in a glutamatergic activation that depends on the AMPA receptor pathway, resulting in an increase in neuronal connections that has been associated with the faster-acting antidepressant effects than those achieved by standard antidepressants, similar to those seen with ketamine.

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In peer-reviewed published preclinical studies, AV-101 caused fast-acting, ketamine-like antidepressant effects, including rapid onset and long duration of effect following a single treatment, without causing ketamine's negative side effects. In two NIH-funded randomized, double blind, placebo-controlled Phase 1 safety studies, AV-101 was safe, well-tolerated and not associated with any severe adverse events. There were no signs of sedation, hallucinations or psychological side effects often associated with ketamine and other channel-blocking NMDA receptor antagonists.

Building on over \$8.8 million of prior grant award funding from the NIH for preclinical and Phase 1 clinical development of AV-101, under our CRADA, we are collaborating with Dr. Carlos Zarate, Jr. and his team at the NIMH on the small NIMH AV-101 MDD Phase 2 Monotherapy Study. Pursuant to the CRADA, this ongoing study is being conducted at the NIMH by Dr. Zarate as Principal Investigator, and is being fully-funded by the NIMH. The primary objective of the NIMH AV-101 MDD Phase 2 Monotherapy Study will be to evaluate the ability of AV-101 to improve overall depressive symptomatology in subjects with MDD, specifically whether subjects with MDD have a greater and more rapid decrease in depressive symptoms when treated with AV-101 than with placebo. We currently anticipate that the NIMH will complete the NIMH AV-101 MDD Phase 2 Monotherapy Study during the first half of 2018.

We are preparing to begin the AV-101 MDD Phase 2 Adjunctive Treatment Study, which will test the safety, efficacy and tolerability of AV-101 as an adjunctive treatment of MDD in adult patients with an inadequate response to standard, FDA-approved antidepressants. Subject to receiving sufficient financing, we intend to launch the AV-101 MDD Phase 2 Adjunctive Treatment Study in the first quarter of 2018 and currently anticipate completing the study by the end of 2018, with top line results available in the first quarter of 2019. The Principal Investigator of our AV-101 MDD Phase 2 Adjunctive Treatment Study will be Dr. Maurizio Fava of Harvard Medical School. Dr. Fava was the co-Principal Investigator with Dr. A. John Rush of the largest clinical trial ever conducted in depression, STAR*D, whose findings were published in journals such as the New England Journal of Medicine and the Journal of the American Medical Association.

AV-101 and Parkinson's Disease Levodopa-Induced Dyskinesia (PD LID)

Parkinson's disease is a chronic, progressive motor disorder that causes tremors, rigidity, slowed movements and postural instability. The Parkinson's Disease Foundation estimates that there were approximately one million people living with Parkinson's disease in the United States in 2011. The most commonly-prescribed treatments for Parkinson's disease are levodopa-based therapies. In the body, levodopa is converted to dopamine to replace the dopamine loss caused by the disease. The therapeutic efficacy of levodopa is gradually lost over time, and abnormal involuntary movements, dyskinesias, gradually emerge as a prominent side-effect in response to previously beneficial doses of the drug. Parkinson's disease levodopa-induced dyskinesia can be severely disabling, rendering patients unable to perform routine daily tasks. Studies published in the New England Journal of Medicine and Movement Disorders have shown PD LID develops in approximately 45% of levodopa-treated Parkinson's disease patients after five years and 80% after 10 years of levodopa treatment.

In a monkey model of Parkinson's disease, AV-101 (250 mg/kg and 450 mg/kg) reduced by 30% the mean dyskinesia score associated with PD LID. Maximum dyskinesia scores were also reduced by 17%. Importantly, AV-101 did not reduce the anti-parkinsonian therapeutic benefit of levodopa. Moreover, the duration of levodopa response and delay to levodopa effect were not affected by treatment with AV-101. We believe these monkey data warrant exploratory Phase 2 clinical testing of AV-101 in Parkinson's disease patients diagnosed with PD LID.

AV-101 and Neuropathic Pain

Neuropathic pain is a complex, chronic pain state that results from problems with signals from nerves. There are various causes of neuropathic pain, including tissue injury, nerve damage or disease, diabetes, infection, toxins, certain types of drugs, such as antivirals and chemotherapeutic agents, certain cancers, and even chronic alcohol intake. With neuropathic pain, damaged, dysfunctional or injured nerve fibers send incorrect signals to other pain centers and impact nerve function both at the site of injury and areas around the injury. Many neuropathic pain treatments on the market today, including gabapentin, have side effects such as anxiety, depression, mild cognitive impairment and/or sedation.

The effects of AV-101 were assessed in published peer-reviewed studies involving four well-established non-clinical models of pain, both hyperalgesia and allodynia, to examine its analgesic and behavioral profile. The publication, titled: "Characterization of the effects of L-4-chlorokynurenine on nociception in rodents," by lead author, Tony L. Yaksh, Ph.D., Professor in Anesthesiology at the University of California, San Diego, was published in The Journal of Pain in April 2017 (J Pain. 18:1184-1196, 2017)). In these studies, systemic delivery of AV-101 yielded brain concentrations of AV-101's active metabolite, 7-Cl-KYNA. The high CNS levels of 7-Cl-KYNA that were calculated to exceed its IC₅₀ at the NMDA receptor GlyB site and resulted in robust, dose-dependent anti-nociceptive effects, similar to gabapentin, but with no discernable negative side effects. Gabapentin, a commonly used drug for neuropathic pain, causes sedation and mild cognitive impairment. Other commonly prescribed medications for pain include drugs targeting opioid receptors in the brain. Unfortunately, misuse of such drugs can lead to a significantly increased risk of addiction despite their potential therapeutic benefits. Therefore, we believe a drug candidate that does not target opioid receptors and is equally effective on pain, but is better tolerated than gabapentin or potentially addictive drugs targeting opioid receptors, could be an important treatment alternative for the millions of patients battling chronic neuropathic pain. Taken together with our successful AV-101 Phase 1a and 1b clinical safety studies, we believe the published results of these nonclinical studies support further clinical development of AV-101 in an exploratory Phase 2 clinical study to assess its potential as a non-opioid treatment to reduce debilitating neuropathic pain effectively, without causing gabapentin-like side effects or risk of addiction associated with pain medications targeting opioid receptors.

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AV-101 and Epilepsy

AV-101 has been shown to protect against seizures and neuronal damage in animal models of epilepsy, providing preclinical support for its potential as a novel treatment alternative for epilepsy. Epilepsy is one of the most prevalent neurological disorders, affecting almost 1% of the worldwide population. According to the Epilepsy Foundation, as many as three million Americans have epilepsy, and one-third of those suffering from epilepsy are not effectively treated with currently available medications. In addition, standard anticonvulsants can cause significant side effects, which frequently interfere with compliance.

Glutamate is a neurotransmitter that is critically involved in the pathophysiology of epilepsy. Through its stimulation of the NMDA receptor subtype, glutamate has been implicated in the neuropathology and clinical symptoms of the disease. In support of this, NMDA receptor antagonists are potent anticonvulsants. However, classic NMDA receptor antagonists are limited by adverse effects, such as neurotoxicity, declining mental status, and the onset of psychotic symptoms following administration of the drug. The endogenous amino acid glycine modulates glutamatergic neurotransmission by stimulating the GlyB co-agonist site of the NMDA receptor. GlyB site antagonists inhibit NMDA receptor function and are therefore anticonvulsant and neuroprotective. Importantly, GlyB site antagonists have fewer and less severe side effects than classic channel-blocking NMDA receptor antagonists and other antiepileptic agents, making them a safer potential alternative to, and one expected to be associated with greater patient compliance than, available anticonvulsant medications.

AV-101 has two additional therapeutically important properties as a drug candidate for treatment of epilepsy:

1. AV-101 is preferentially converted to 7-Cl-KYNA in brain areas related to neuronal injury. This is because astrocytes, which are responsible for the enzymatic transamination of 4-Cl-KYN prodrug to active 7-Cl-KYNA, are focally activated at sites of neuronal injury. Due to AV-101's highly focused site of conversion, local concentrations of newly formed 7-Cl-KYNA are greatest at the site of therapeutic need. In addition to delivering the drug where it is needed, this reduces the chance of systemic and dangerous side effects with long-term use of the drug; and
2. An active metabolite of AV-101, 4-Cl-3-hydroxyanthranilic acid, inhibits the synthesis of quinolinic acid, an endogenous NMDAR agonist that causes convulsions and excitotoxic neuronal damage.

AV-101's ability to activate astrocytes for focal delivery of an anti-epileptic principle, and its dual action as a NMDAR GlyB antagonist and quinolinic acid synthesis inhibitor, make AV-101 a potential Phase 2 development candidate for treatment of epilepsy.

AV-101 and Huntington's Disease

Working together with metabotropic glutamate receptors, the NMDA receptor ensures the establishment of long-term potentiation (LTP), a process believed to be responsible for the acquisition of information. These functions are mediated by calcium entry through the NMDA receptor-associated channel, which in turn influences a wide variety of cellular components, like cytoskeletal proteins or second-messenger synthases. However, over activation at the NMDA receptor triggers an excessive entry of calcium ions, initiating a series of cytoplasmic and nuclear processes that promote neuronal cell death through necrosis as well as apoptosis, and these mechanisms have been implicated in several neurodegenerative diseases.

Huntington's disease (HD) is an inherited disorder that causes degeneration of brain cells, called neurons, in motor control regions of the brain, as well as other areas. Symptoms of the disease, which gets progressively worse, include

uncontrolled movements (called chorea), abnormal body postures, and changes in behavior, emotion, judgment, and cognition. HD is caused by an expansion in the number of glutamine repeats beyond 35 at the amino terminal end of a protein termed “huntingtin.” Such a mutation in huntingtin leads to a sequence of progressive cellular changes in the brain that result in neuronal loss and other characteristic neuropathological features of HD. These are most prominent in the neostriatum and in the cerebral cortex, but also observed in other brain areas.

The tissue levels of two neurotoxic metabolites of the kynurenine pathway of tryptophan degradation, quinolinic acid (QUIN) and 3-hydroxykynurenine (3-HK) are increased in the striatum and neocortex, but not in the cerebellum, in early stage HD. QUIN and 3-HK and especially the joint action of these two metabolites, have long been associated with the neurodegenerative and other features of the pathophysiology of HD. The neuronal death caused by QUIN and 3-HK is due to both free radical formation and NMDA receptor overstimulation (excitotoxicity).

Based on the hypothesis that 3-HK and QUIN are involved in the progression of HD, early intervention aimed at affecting the kynurenine pathway in the brain may present a promising treatment strategy. We believe the ability of AV-101 to reduce the brain levels of neurotoxic QUIN and to potentially produce significant local concentrations of 7-Cl-KYNA on chronic administration, presents an exciting opportunity for exploratory Phase 2 clinical investigation of AV-101 as a potential chronic treatment alternative for certain symptoms of HD.

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AV-101 Phase 1 Clinical Safety Studies

The safety data from two NIH-funded AV-101 Phase 1 clinical safety studies indicate that AV-101 was safe and well tolerated in healthy subjects at all doses tested. There were no Adverse Effects (AEs) reported by subjects that received AV-101 that were graded as probably related to study drug. The type and distribution of AEs reported by subjects in the studies were considered to be typical for studies in healthy volunteers. All AEs were completely resolved. Further, no Serious Adverse Events (SAEs) were reported.

The Pharmacokinetics (PK) of AV-101 were fully characterized across the range of doses in these Phase 1a and 1b studies. Plasma concentration-time profiles obtained for 4-Cl-KYN (AV-101) and 7-Cl-KYNA after administration of a single escalating dose (Phase 1a) and multiple, once daily oral doses of 360, 1,080, or 1,440 mg for 14 days (Phase 1b) were consistent with rapid absorption of the oral dose and first-order elimination of both analytes, with evidence of multi-compartment kinetics, particularly for the AV-101's active metabolite, 7-Cl-KYNA.

Although the Phase 1 safety and PK studies were not designed to measure or evaluate the potential antidepressant effects of AV-101, approximately 9% (5/54) of the subjects receiving AV-101 and 0% of the 30 subjects receiving placebo reported "feelings of well-being" (coded as euphoric mood), similar to the fast-acting antidepressant effects reported in the literature with ketamine.

Phase 1a Study

A phase 1a, randomized, double blind, placebo-controlled study to evaluate the safety and PK of single doses of AV-101 in healthy volunteers was conducted. Seven cohorts (30, 120, 360, 720, 1,080, 1,440, and 1,800 mg) with six subjects per cohort (1:1, AV-101: placebo) were to be enrolled in the study. Nine subjects experienced 10 AEs, with four of the AEs occurring in subjects in the placebo group and two of the AEs occurring for one subject receiving 30 mg AV-101. For the AEs occurring in the AV-101-treated subjects, there were no meaningful differences in the number of AEs observed at the 30-mg dose (two AEs) when compared with that at the 120-mg dose (one AE), 360-mg dose (one AE), 720-mg dose (zero AEs), 1,080-mg dose (zero AEs), or 1,440-mg dose (two AEs). Eight of 10 AEs (80%) were considered mild, and two (20%, headache and gastroenteritis) were considered moderate. Four subjects on AV-101, one each in Cohorts 1 through 4 and two subjects on placebo in Cohort 5 reported AEs of headaches. Five headaches were mild with no concomitant treatment, and one was moderate with concomitant drug therapy administered. Most completely resolved the same day as onset and were considered not serious. One headache started the day after dosing and resolved approximately one week later on the same day as the concomitant drug therapy was administered. One case of contact dermatitis bilateral lower extremities was reported in Cohort 2 on placebo that was ongoing. One of the subjects with the headache also reported an AE of gastroenteritis that was unrelated to AV-101. This AE was considered moderate but did not require any drug therapy and was completely resolved within two days of onset. This AE was also considered not serious.

The PK of AV-101 was fully characterized across the range of doses in this Phase 1a study following a single oral administration. Plasma concentration-time profiles obtained for 4-Cl-KYN (AV-101) and 7-Cl-KYNA were consistent with rapid absorption of the oral dose and first-order elimination of both analytes, with evidence of multi-compartment kinetics, particularly for the metabolite 7-Cl-KYNA.

Even though this Phase 1a safety study was not designed to quantitatively assess effects on mood, during the interviews, two out of three subjects who received the highest dose (1440 mg) of AV-101 voluntarily acknowledged positive effects on their mood. Similar comments were not made by any of the 18 placebo group subjects.

Phase 1b Study

A Phase 1b clinical study was conducted as a single-site, dose-escalating study to evaluate the safety, tolerability, and PK of multiple doses of AV-101 administered daily in healthy volunteers. The antihyperalgesic effect of AV-101 on capsaicin-induced hyperalgesia was also assessed. Subjects were sequentially enrolled into one of three cohorts (360 mg, 1,080 mg, and 1,440 mg) and were randomized to AV-101 or placebo at a 12:4 (AV-101 to placebo) ratio. Subjects were dosed for 14 consecutive days. Each subject was given a paper diary and instructed to record daily dose administration, concomitant medications, and AEs during the 14-day treatment period.

For this study, the minimum toxic dose was to be (i) the dose at which a drug-related SAE occurred in an AV-101–treated subject, or (ii) the dose at which a severe AE that warranted stopping the study, as determined by the investigator and medical monitor, occurred in an AV-101–treated subject within a cohort. The minimum toxic dose was not reached in this study.

A total of 40 AEs were reported by 24 of 37 (64.9%) subjects receiving AV-101, and 17 AEs were reported by 10 of 13 (76.9%) subject receiving placebo. The frequency of AEs was similar among the treatment groups. Thirty-four subjects experienced a total of 57 AEs, with 16 (28.1% of the total AEs) in the 360-mg group, 14 (24.6% of the total AEs) in the 1,040-mg group, 10 (17.5% of the total AEs) in the 1,440-mg group, and 17 (29.8% of the total AEs) in the placebo group. All of the AEs were completely resolved, and no SAEs were reported.

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The majority of the reported AEs were nervous system disorders (23 subjects, 46% of subjects) and gastrointestinal disorders (seven subjects, 14.0%). The remaining AEs were classified as eye disorders (three subjects, 6.0%); psychiatric disorders (three subjects, 6.0%); respiratory, thoracic, and mediastinal disorders (three subjects, 6.0%); skin and subcutaneous tissue disorders (three subjects, 6.0%); general disorders and administration site conditions (two subjects, 4.0%); cardiac disorders one subject, 2.0%; infections and infestations (one subject, 2.0%); musculoskeletal and connective tissue disorders (one subject, 2.0%); and renal disorders (one subject, 2.0%).

The distribution of AEs by System Organ Class was similar among the cohorts with the exception of headaches and gastrointestinal disorders. Eight of the 18 (44.4%) reported headaches were in the placebo group, 6 (33.3%) were in the 1,080-mg group, three (16.7%) were in the 1,440-mg group, and one (5.6%) was in the 360-mg group. Three (42.9%) of the seven reported gastrointestinal disorders were in the 360-mg group, two (28.6%) were in the placebo group, one (14.3%) was in the 1,080-mg group, and one (14.3%) was in the 1,440-mg group.

The determination of the relationship of the AE to the study drug was made when the data were unblinded. Ten of the 15 AEs (66.7%) that occurred in the 360-mg AV-101 group, 10 of the 14 AEs (71.4%) that occurred in the 1,040-mg AV-101 group, seven of the 10 AEs (70.0%) that occurred in the 1,440-mg AV-101 group, and 13 of the 17 AEs (76.5%) that occurred in the placebo group were determined to be possibly related to study drug. One (5.9%) AE in the placebo group was probably related to study drug (rash around neck). Of the 57 reported AEs, 49 (85.9%) were of mild intensity and eight (14.0%) were of moderate intensity. There were two moderate intensity AEs in the 360-mg AV-101 group; one was unrelated pain in the right foot, and one was a possibly related headache. All other moderate AEs occurred in the placebo group and included nausea or vomiting (two AEs), headache (two AEs), and rash around the neck (one AE). No SAEs were reported.

Even though this Phase 1b safety study was not designed to quantitatively assess effects on mood, during the interviews certain subjects who received 360, 1080, and 1440 mg of AV-101, voluntarily acknowledged positive effects on mood. Similar comments were not made by any of the placebo-group subjects.

The PK of AV-101 was fully characterized across the range of doses in this Phase 1b study. Plasma concentration-time profiles obtained for 4-Cl-KYN (AV-101) and 7-Cl-KYNA following 14 daily oral administrations of 360, 1,080, or 1,440 mg were consistent with rapid absorption of the oral dose and first-order elimination of both analytes, with evidence of multi-compartment kinetics, particularly for the metabolite 7-Cl-KYNA.

VistaStem Therapeutics

VistaStem Therapeutics (VistaStem) is our wholly owned subsidiary focused on applying human pluripotent stem cell (hPSC) technology, internally and with collaborators, to discover, rescue, develop and commercialize (i) proprietary new chemical entities (NCEs) for CNS and other diseases and (ii) regenerative medicine (RM) involving hPSC-derived blood, cartilage, heart and liver cells. We used our hPSC-derived cardiomyocytes (human heart cells) to develop CardioSafe 3D™, our customized in vitro bioassay system for predicting heart toxicity of drug rescue NCEs. We believe CardioSafe 3D is more comprehensive and clinically predictive than the hERG assay, which currently is the only in vitro cardiac safety assay required by FDA guidelines, and provides us with new generation human cell-based technology to identify and evaluate drug rescue candidates and develop drug rescue NCEs.

Scientific Background

Stem cells are the building blocks of all cells of the human body. They have the potential to develop into many different mature cell types. Stem cells are defined by a minimum of two key characteristics: (i) their capacity to self-renew, or divide in a way that results in more stem cells; and (ii) their capacity to differentiate, or turn into

mature, specialized cells that make up tissues and organs. There are many different types of stem cells that come from different places in the body or are formed at different times throughout our lives, including pluripotent stem cells and adult or tissue-specific stem cells, which are limited to differentiating into the specific cell types of the tissues in which they reside. We focus exclusively on human pluripotent stem cells.

Human pluripotent stem cells can be differentiated into all of the more than 200 types of cells in the human body, can be expanded readily, and have diverse medical research, drug discovery, drug rescue, drug development and therapeutic applications. We believe hPSCs can be used to develop numerous cell types, tissues and customized assays that can mimic complex human biology, including heart and liver biology for drug rescue.

Human pluripotent stem cells are either embryonic stem cells (hESCs) or induced pluripotent stem cells (iPSCs). Both hESCs and iPSCs have the capacity to be maintained and expanded in an undifferentiated state indefinitely. We believe these features make them highly useful research and development tools and as a source of normal, functionally mature cell populations. We use multiple types of these mature cells as the foundation to design and develop novel, customized bioassay systems to test the safety and efficacy of NCEs in vitro. These cells also have potential for diverse regenerative medicine applications.

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Human Embryonic Stem Cells

According to the NIH, hESCs are derived from excess embryos that develop from eggs that have been fertilized in an in vitro fertilization (IVF) clinic and then donated for research purposes with the informed consent of the parental donors after a successful IVF procedure. Human embryonic stem cells are not derived from eggs fertilized in a woman's body. Human ESCs are isolated when the embryo is approximately 100 cells, well before organs, tissues or nerves have developed.

Human ESCs have the potential to both self-renew and differentiate. They undergo increasingly tissue-restrictive developmental decisions during their differentiation. These "fate decisions" commit the hESCs to becoming only a certain type of mature, functional cells and ultimately tissues. At one of the first fate decision points, hESCs differentiate into epiblasts. Although epiblasts cannot self-renew, they can differentiate into the major tissues of the body. This epiblast stage can be used, for example, as the starting population of cells that develop into millions of blood, heart, muscle, liver and insulin-producing pancreatic beta-islet cells, as well as neurons. In the next step, the presence or absence of certain growth factors, together with the differentiation signals resulting from the physical attributes of the cell culture techniques, induce the epiblasts to differentiate into neuroectoderm or mesendoderm cells. Neuroectoderm cells are committed to developing into cells of the skin and nervous systems. Mesendoderm cells are precursor cells that differentiate into mesoderm and endoderm. Mesoderm cells develop into muscle, bone and blood, among other cell types. Endoderm cells develop into the internal organs such as the heart, liver, pancreas and intestines, among other cell types.

Induced Pluripotent Stem Cells

It is also possible to obtain hPSC lines from individuals without the use of embryos. Induced PSCs are adult cells, typically human skin or fat cells that have been genetically reprogrammed to behave like hESCs by being forced to express genes necessary for maintaining the pluripotential properties of hESCs. Although researchers are exploring non-viral methods, most early iPSCs were produced by using various viruses to express three or four genes required for the immature pluripotential property similar to hESCs. It is not yet precisely known, however, how each gene actually functions to induce cellular pluripotency, nor whether each of the three or four genes is essential for this reprogramming. Although hESCs and iPSCs are believed to be similar in many respects, including their pluripotential ability to form all cells in the body and to self-renew, scientists do not yet know whether they differ in clinically significant ways or have the same ability to self-renew.

We believe the biology and differentiation capabilities of hESCs and iPSCs are likely to be comparable for most if not all purposes. There are, however, specific situations in which we may prefer to use one or the other type of hPSC. For example, we may prefer to use iPSCs for potential drug discovery applications based on the relative ease of generating iPSCs from:

individuals with specific inheritable diseases and conditions that predispose the individual to respond differently to drugs; or

individuals with specific variations in genes that directly affect drug levels in the body or alter the manner or efficiency of their metabolism, breakdown and/or elimination of drugs.

Because they can significantly affect the therapeutic and/or toxic effects of drugs, these genetic variations have an impact on drug discovery and development. We believe iPSC technologies may allow the rapid and efficient

generation of hPSCs from individuals with specific genetic variations. These hPSCs might then be used to produce cells to model specific diseases and genetic conditions for drug discovery and drug rescue purposes.

CardioSafe 3D

The limitations of current preclinical drug testing systems used by pharmaceutical companies and others contribute to the high failure rate of NCEs. Incorporating novel in vitro assays using hPSC-derived cardiomyocytes (hPSC-CMs) early in preclinical development offers the potential to improve clinical predictability, decrease development costs, and avoid adverse patient effects, late-stage clinical termination, and product recall from the market.

We produce fully functional, non-transformed hPSC-CMs at a high level of purity and with normal ratios of all important cardiac cell types. Importantly, our hPSC-CM differentiation protocols do not involve either genetic modification or antibiotic selection. This is important because genetic modification and antibiotic selection can distort the ratio of cardiac cell types and have a direct impact on the ultimate results and clinical predictivity of assays that incorporate hPSC-CMs produced in such a manner. In addition to normal expression all of the key ion channels of the human heart (calcium, potassium and sodium) and various cardiomyocytic markers of the human heart, our CardioSafe 3D cardiac toxicity assays screening for both direct cardiomyocyte cytotoxicity and arrhythmogenesis (or development of irregular beating patterns). We believe CardioSafe 3D is sensitive, stable, reproducible and capable of generating data enabling a more accurate prediction of the in vivo cardiac effects of NCEs than is possible with existing preclinical testing systems, particularly the hERG assay.

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Limited Clinical Predictivity of the FDA-Required hERG Assay vs. Broad Clinical Predictivity of CardioSafe 3D

The hERG assay, which uses either transformed hamster ovary cells or human kidney cells, is currently the only in vitro cardiac safety assay required by FDA Guidelines (ICH57B). We believe the clinical predictivity of the hERG assay is limited because it assesses only a single cardiac ion channel - the hERG potassium ion channel. It does not assess any other clinically relevant cardiac ion channels, including calcium, non-hERG potassium and sodium ion channels. Also, importantly, the hERG assay does not assess the normal interaction between these ion channels and their regulators. In addition, the hERG assay does not assess clinically relevant cardiac biological effects associated with cardiomyocyte viability, including apoptosis and other forms of cytotoxicity, as well as energy, mitochondria and oxidative stress. As a result of its limitations, results of the hERG assay can lead to false negative and false positive predictions regarding the cardiac safety of new drug candidates.

We have developed and validated two clinically relevant functional components of our CardioSafe 3D screening system to assess multiple categories of cardiac toxicities, including both direct cardiomyocyte cytotoxicity and arrhythmogenesis (or development of irregular beating patterns). The first functional component of CardioSafe 3D consists of a suite of five fluorescence or luminescence based high-throughput hPSC-CM assays. These five CardioSafe 3D assays measure the following important drug-induced cardiac biological effects:

1. cell viability;
2. apoptosis;
3. mitochondrial membrane depolarization;
4. oxidative stress; and
5. energy metabolism disruption.

These five CardioSafe 3D biological assays were correlated to reported clinical results using reference compounds known to be cardiotoxic in humans versus compounds known to be safe in humans. These reference compounds were representative of eight different drug classes, including:

1. ion channel blockers: amiodarone, nifedipine;
2. hERG trafficking blockers: pentamidine, amoxapine;
3. α -1 adrenoreceptors: doxazosin;
4. protein and DNA synthesis inhibitors: emetine;
5. DNA intercalating agents: doxorubicin;
6. antibiotics: ampicillin, cefazolin;
7. NSAID: aspirin; and
8. kinase inhibitors: staurosporine.

This suite of five CardioSafe 3D cytotoxicity assays provided measurement of cardiac drug effects with high sensitivity that are consistent with the expected cardiac responses to each of these compounds. Based on our results, we believe CardioSafe 3D provides valuable and more comprehensive bioanalytical tools for both assessing the effects of pharmaceutical compounds on cardiac cytotoxicity than the hERG assay and can elucidate for us and our strategic partners specific mechanisms of cardiac toxicity, thereby laying what we believe is a novel and advantageous foundation for our CardioSafe 3D drug rescue NCE programs.

The other component of our CardioSafe 3D assay system is a sensitive and reliable medium throughput multi-electrode array (MEA) assay developed to predict drug-induced alterations of electrophysiological function of the human heart, representing an integrated assessment of not only hERG potassium ion channel activity analogous to the FDA-mandated hERG assay but, in addition, non-hERG potassium channels, and calcium channels and sodium

channels, which are well beyond the scope of the hERG assay. Functional electrophysiological assessment is a key component of CardioSafe 3D, and has been validated with reported clinical results involving drugs with known toxic or non-toxic cardiac effects in humans.

We have validated that CardioSafe 3D is capable of assessing important electrophysiological activity of drug rescue NCEs, including spike amplitude, beat period and field potential duration. Our CardioSafe 3D MEA assay, which we refer to as ECG in a test tube™, was reproducible and consistent with the known human cardiac effects of all compounds studied, based on the mechanisms of action and dosage of the compounds. For instance, by using CardioSafe 3D, we were able to distinguish between the arrhythmogenic cardiac effects of terfenadine (Seldane™), withdrawn by the FDA due to cardiotoxicity, and the cardiac effects of the closely structurally-related compound, fexofenadine (Allegra™), a safe variant of terfenadine, which remains on the market. We believe our correlation data demonstrate that CardioSafe 3D provides valuable and more comprehensive bioanalytical tools for in vitro cardiac safety screening than the hERG assay. We believe CardioSafe 3D will contribute to our efficient and rapid identification of novel, potentially safer proprietary NCEs in our drug rescue programs.

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Using CardioSafe 3D to Develop Drug Rescue NCEs

Our drug rescue activities are focused on producing for our internal pipeline proprietary, safer variants of still-promising NCEs previously discovered, optimized and tested for efficacy by pharmaceutical companies and others but terminated before FDA approval due to unexpected heart toxicity or liver toxicity. Our current drug rescue strategy involves using CardioSafe 3D to assess the toxicity that caused certain NCEs available in the public to be terminated, and use that biological insight to produce and develop a new, potentially safer, and proprietary NCEs for our pipeline. We believe the pre-existing public domain knowledge base supporting the therapeutic and commercial potential of NCEs we target for our drug rescue programs will provide us with a valuable head start as we launch each of our drug rescue programs. Leveraging the substantial prior investments by global pharmaceutical companies and others in discovery, optimization and efficacy validation of the NCEs we identify in the public domain is an essential component of our drug rescue strategy.

By using CardioSafe 3D to enhance our understanding of the cardiac liability profile of NCEs, biological insight not previously available when the NCEs were originally discovered, optimized for efficacy and developed, we believe we can demonstrate preclinical proof-of-concept (POC) as to the efficacy and safety of new, safer drug rescue NCEs in standard in vitro and in vivo models, as well as in CardioSafe 3D, earlier in development and with substantially less investment in discovery and preclinical development than was required of pharmaceutical companies and others prior to their decision to terminate the original NCE.

Our goal in each drug rescue program will be to produce a proprietary drug rescue NCE and establish its preclinical POC, using standard preclinical in vitro and in vivo efficacy and safety models, as well as CardioSafe 3D. In this context, POC means that the lead drug rescue NCE, as compared to the original, previously-terminated NCE, demonstrates both (i) equal or superior efficacy in the same, or a similar, in vitro and in vivo preclinical efficacy models used by the initial developer of the previously-terminated NCE before it was terminated for safety reasons, and (ii) significant reduction of concentration dependent cardiotoxicity in CardioSafe 3D.

Regenerative Medicine (RM)

Although we believe the best and most valuable near term commercial application of our stem cell technology platform is for small molecule drug rescue, we also believe stem cell technology-based RM has the potential to transform healthcare in the U.S. over the next decade by providing new approaches for treating the fundamental mechanisms of disease. We currently intend to establish strategic collaborations to leverage our stem cell technology platform, our expertise in human biology, differentiation of human pluripotent stem cells to develop functional adult human cells and tissues involved in human disease, including blood, bone, cartilage, heart and liver cells, and our expertise in designing and developing novel, customized biological assay systems with the cells we produce, for RM purposes. In December 2016, we exclusively sublicensed to BlueRock Therapeutics LP, a next generation RM company established by Bayer AG and Versant Ventures, rights to certain proprietary technologies relating to the production of cardiac stem cells for the treatment of heart disease. In a manner similar to our exclusive sublicense agreement with BlueRock Therapeutics, we may pursue additional RM collaborations or licensing transactions involving blood, cartilage, and/or liver cells derived from hPSCs for (A) cell-based therapy, (B) cell repair therapy, and/or (C) tissue engineering.

Strategic Transactions and Relationships

Strategic collaborations are an important cornerstone of our corporate development strategy. We believe that our highly selective outsourcing of certain research and development activities gives us flexible access to chemistry broad range of research and development capabilities, manufacturing, clinical development and regulatory expertise at a

lower overall cost than developing and maintaining such expertise internally on a full-time basis. In particular, we contract with third parties for certain manufacturing, non-clinical development, clinical development and regulatory affairs support. The following are among our current third-party collaborators:

Cato Research Ltd.

Cato Research Ltd. is a CRO with international resources dedicated to helping biotechnology and pharmaceutical companies navigate the regulatory approval process in order to bring new biologics, drugs and medical devices to markets throughout the world. Cato Research is one of our CROs for development of AV-101, currently focused on all chemistry, manufacturing and controls (CMC) aspects of our Phase 2 development program in MDD. Cato Research's senior management team, including co-founders Allen Cato, M.D., Ph.D. and Lynda Sutton, have over 30 years of experience interacting with the FDA and international regulatory agencies and a successful track record of product approvals.

Cardiac Safety Research Consortium

We have joined the Cardiac Safety Research Consortium (CSRC) as an Associate Member. The CSRC, which is sponsored in part by the FDA, was launched in 2006 through an FDA Critical Path Initiative Memorandum of Understanding with Duke University to support research into the evaluation of cardiac safety of medical products. CSRC supports research by engaging stakeholders from industry, academia, and government to share data and expertise regarding several areas of cardiac safety evaluation, including novel stem cell-based approaches, from preclinical through post-market periods.

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Cardiac Safety Technical Committee of the Health and Environmental Sciences Institute – FDA’s CIPA Initiative

We have also joined the Cardiac Safety Technical Committee, Cardiac Stem Cell Working Group, and Proarrhythmia Working Group of the Health and Environmental Sciences Institute (HESI) to help advance, among other goals, the FDA’s Comprehensive In Vitro Proarrhythmia Assay (CIPA) initiative, which is focused on developing innovative preclinical systems for cardiac safety assessment during drug development. HESI is a global branch of the International Life Sciences Institute (ILSI), whose members include most of the world’s largest pharmaceutical and biotechnology companies.

The goal of the FDA’s CIPA initiative is to develop a new paradigm for cardiac safety evaluation of new drugs that provides a more comprehensive assessment of proarrhythmic potential by (i) evaluating effects of multiple cardiac ionic currents beyond hERG and ICH S7B Guidelines (inward and outward currents), (ii) providing more complete, accurate assessment of proarrhythmic effects on human cardiac electrophysiology, and (iii) focusing on Torsades de Pointes proarrhythmia rather than surrogate QT prolongation alone.

Centre for Commercialization of Regenerative Medicine

The Toronto-based Centre for Commercialization of Regenerative Medicine (CCRM) is a not-for-profit, public-private consortium funded by the Government of Canada, six Ontario-based institutional partners and more than 20 companies representing the key sectors of the regenerative medicine industry. CCRM supports the development of foundational technologies that accelerate the commercialization of stem cell- and biomaterials-based products and therapies.

We are a member of the CCRM’s Industry Consortium. Other members of CCRM’s Industry Consortium include Pfizer and GE Healthcare. The industry leaders that comprise the CCRM consortium benefit from proprietary access to certain licensing opportunities, academic rates on fee-for-service contracts at CCRM and opportunities to participate in large collaborative projects, among other advantages. Our CCRM membership reflects our strong association with CCRM and its core programs and objectives, both directly and through our strategic relationships with Dr. Gordon Keller and UHN. We believe our long-term sponsored research agreement with Dr. Keller, UHN and UHN’s McEwen Centre offers unique opportunities for expanding the commercial applications of our stem cell technology platform by building multi-party collaborations with CCRM and members of its Industry Consortium. We believe these collaborations have the potential to transform medicine and accelerate significant advances in human health and wellness that stem cell technologies and regenerative medicine promise.

Massachusetts General Hospital Clinical Trials Network and Institute

Massachusetts General Hospital Clinical Trials Network and Institute (CTNI) is an academic CRO, part of the Department of Psychiatry of the Massachusetts General Hospital (MGH), a leader in academic scientific and clinical research in psychiatry. By exploring the brain science, genetics, and neurobiology of psychiatric disorders, the MGH CTNI has been instrumental in the development of novel treatments and surrogate markers of illness and therapeutic response. Its scientific and clinical research has been instrumental in defining the standards for the state-of-the-art practice of psychiatry. We are working with MGH CTNI, including its principals, Dr. Maurizio Fava and Dr. Thomas Laughren, in connection with the planning and execution of our AV-101 MDD Adjunctive Treatment Study. Dr. Fava is acknowledged as a world-renowned expert in depressive disorders and psychopharmacology. He is Director of the Division of Clinical Research of the MGH Research Institute, Executive Vice Chair, Department of Psychiatry, at MGH, and Executive Director of MGH CTNI. He will serve as Principal Investigator of the AV-101 MDD Phase 2 Adjunctive Treatment Study. Dr. Laughren is the former FDA Division Director, Division of Psychiatry Products, Center for Drug Evaluation and Research (CDER).

United States National Institutes of Health

Since our inception in 1998, the NIH has awarded us \$11.3 million in non-dilutive research and development grants, including \$2.3 million to support research and development of our stem cell technology and \$8.8 million for non-clinical and Phase 1a and 1b clinical development of AV-101.

United States National Institute of Mental Health

The NIMH, part of the NIH, is the largest scientific organization in the world dedicated to mental health research. NIMH is one of 27 Institutes and Centers of the NIH, the world's leading biomedical research organization. The mission of NIMH is to transform the understanding and treatment of mental illnesses through basic and clinical research, paving the way for prevention, recovery and cure. Our CRADA with the NIH provides for NIMH sponsorship of the ongoing NIMH AV-101 MDD Phase 2 Monotherapy Study, a study being fully funded by the NIH and is being conducted at the NIMH by Dr. Carlos Zarate, the NIMH's Chief of Experimental Therapeutics & Pathophysiology Branch and Section on Neurobiology and Treatment of Mood and Anxiety Disorders.

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Intellectual Property

We rely upon patents as a major component of our intellectual property portfolio, as is typical for development-stage, biopharmaceutical companies. In addition, from time to time, we enter into patent license agreements to acquire rights to intellectual property. We also rely, in part, on trade secrets for protection of some of our discoveries. We seek to protect our trade secrets by entering into confidentiality agreements with employees, consultants, collaborators and third parties. We also own several registered and common-law trademarks.

To help protect our intellectual property rights, our employees, contractors and consultants also sign agreements in which they assign to us, for example, their interests in patents, trade secrets and copyrights arising from their work for us.

From time to time, we sponsor research with key scientists in academic institutions to advance or supplement our internal research and development activities and objectives. These sponsored research agreements generally provide us with an opportunity to negotiate a new license, or acquire a substantially prescribed license, to acquire intellectual property rights in the results of the sponsored research.

AV-101

AV-101 (4-Cl-KYN) is our oral CNS product candidate presently being investigated in the NIMH AV-101 MDD Phase 2 Monotherapy Study. Further, we are preparing to launch our AV-101 MDD Phase 2 Adjunctive Treatment Study to assess the safety and efficacy of AV-101 as a new generation adjunctive treatment of MDD in adult patients with an inadequate response to standard, FDA-approved antidepressants. We have developed a portfolio of intellectual property assets around AV-101, which involves patents, patent applications and trade secrets, primarily focused on depression. In addition, we plan to seek regulatory exclusivity to the use of AV-101, with emphasis on depression, as a central approach to protect the marketing of our product. This approach will complement certain of our AV-101 intellectual property rights.

Although the compound 4-Cl-KYN, per se, is no longer patented, and certain of its formulations are in the public domain and thus are no longer protectable, as part of our strategy to seek and secure broad commercial exclusivity for AV-101, we have filed and are pursuing several patent applications in Europe, the U.S. and selected major markets. Several of these patent applications have already been granted or allowed, on both (i) certain novel therapeutic methods of use of AV-101, including depression, and (ii) certain novel methods of producing AV-101. In Europe, the European Patent Office (EPO) has granted our patent related to methods of treating depression with AV-101 and certain other neurological indications. In the U.S. and selected major markets, we are currently pursuing a counterpart AV-101 patent application similar to the patent granted by the EPO. The U.S. Patent and Trademark Office (USPTO) has not yet allowed the counterpart application filed in the U.S. However, we believe that our counterpart patent applications in the USPTO and other countries ultimately will be granted.

In Europe, the U.S. and selected major markets, we are also prosecuting patent applications related to novel methods of producing AV-101. Both the USPTO and Chinese patent office, have granted patents regarding our manufacturing process for AV-101. The EPO has not yet allowed the counterpart application filed in Europe. However, just as our application at the USPTO was sufficient to obtain an allowed patent in the U.S., we believe our counterpart patent application at the EPO ultimately will be granted.

As noted elsewhere in this prospectus, we are currently involved with the NIMH AV-101 MDD Phase 2 Monotherapy Study being conducted by the NIMH. As part of our analysis of the study results, we will be evaluating the possibility of seeking additional patent protection in Europe, the U.S. and selected major markets based on the clinical data and

on clinical observations.

As mentioned above, a major component of our plans to obtain market exclusivity for approved therapeutic indications for AV-101 includes the use of New Drug Product Exclusivity provided by the FDA under section 505(c)(3)(E) and 505(j)(5)(F) of the Federal Food, Drug, and Cosmetic Act (FDCA). The FDA's New Drug Product Exclusivity is available for NCEs such as AV-101, which are innovative and have not been previously approved by the FDA, either alone or in combination with other drugs. The FDA's New Drug Product Exclusivity protection provides the holder of an FDA-approved NDA with up to five years of protection from competition in the U.S. marketplace for the innovation represented by its approved new drug product. This protection precludes FDA approval of certain generic drug applications under section 505(b)(2) of the FDCA, as well as certain abbreviated new drug applications (ANDAs), during the up to five-year exclusivity period, except that such applications may be submitted after four years if they contain a certification of patent invalidity or non-infringement. As and if applicable, we will pursue similar types of regulatory exclusivity in other regions, such as Europe, and in certain other countries.

There is no guarantee that we will be successful in obtaining any additional patents related to AV-101 in Europe, the U.S., or any other country, or that if we are successful in obtaining any such patents that we would also be successful in protecting those patents against challengers or in enforcing them to stop infringement. Outside the U.S. and Europe, we are pursuing patent rights in a limited number of countries that we believe are the major markets for pharmaceuticals where having patent rights will substantially facilitate commercialization of AV-101.

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Stem Cell Technology

We have obtained and are pursuing intellectual property rights to several stem cell technologies through a combination of our own patent properties, exclusive and non-exclusive patent and technology licenses, and participation in sponsored research relationships. Generally, our stem cell intellectual property portfolio relates to drug development and drug discovery. It also relates to novel production systems of enriched populations of certain cell types, such as cardiomyocytes and the use of various cell types that have been differentiated from pluripotent stem cells for those and other purposes including cell-based therapy. Additionally, we maintain certain trade secrets regarding stem cell technology, several of which are discussed below.

Overall, our stem cell patent portfolio includes several issued U.S. patents as well as several foreign counterpart patents in countries of commercial interest to us. The portfolio also includes several patent applications pending in the U.S. and in various foreign countries.

The patent properties in these families are based on discoveries from our internal research and development activities, research that has sponsored at various academic institutions, as well as from patent license agreements signed with the University Health Network (Toronto) and the Mount Sinai School of Medicine.

These license agreements generally require us to pay nominal annual license fees, and, in certain cases, patent prosecution and maintenance fees, and royalty payments that vary based on product sales and services that are covered by the licensed patent rights, as well fees for sublicensing. As noted above in the context of AV-101 intellectual property, there is no guarantee that we will successfully obtain patents in the countries in which we are pursuing patent rights or that we would be successful in enforcing granted patent rights against infringers.

In December 2016, we exclusively sublicensed to BlueRock Therapeutics, a stem cell research company recently established by Bayer AG and Versant Ventures, rights to certain proprietary technologies relating to the production of cardiac stem cells for the treatment of heart disease.

Trademarks

We have a federal trademark registration for the trademark “VISTAGEN”. Corresponding trademarks have been registered in the European Union and in Switzerland. We also use certain other trademarks in connection with our customized in vitro bioassay systems, such as CardioSafe 3D™.

U.S. Government Rights

We have received federal funding from both the NIH and the NIMH to support research and development of inventions disclosed in our patent applications relating to AV-101 and certain of our stem cell technology. Under the Bayh-Dole Act of 1980, if we do not take adequate steps to commercialize certain intellectual property rights, or certain other exigent circumstances relating to public health and safety prescribed under federal law become applicable, the U.S. government may acquire certain rights with respect to inventions made during programs funded by NIH or other federal grants.

Competition

The biopharmaceutical industry is highly competitive. There are many public and private biopharmaceutical companies, universities, governmental agencies, including the NIH and NIMH, and other research organizations actively engaged in the research and development of products that may be similar to our product candidates or address

similar markets. It is probable that the number of companies seeking to develop products and therapies similar to our products will increase.

Currently, there are no FDA-approved therapies for MDD with the mechanism of action of AV-101. However, products approved for other indications, for example, the anesthetic ketamine, are being or may be used off-label for treatment of MDD, as well as other CNS indications for which AV-101 may have therapeutic potential. Additionally, other treatment options, such as psychotherapy and electroconvulsive therapy, are sometimes used instead of and before standard antidepressant medications to treat patients with MDD.

In the field of new generation, orally available, adjunctive treatments of adult MDD patients with an inadequate response to standard antidepressants, we believe our principal competitor is Alkermes' orally available drug candidate in Phase 3 development, ALKS-5461, an opioid modulator.

Many of our potential competitors, alone or with their strategic partners, have substantially greater financial, technical and human resources than we do and significantly greater experience in the discovery and development of product candidates, obtaining FDA and other regulatory approvals of treatments and the commercialization of those treatments. We believe that a range of pharmaceutical and biotechnology companies have programs to develop small molecule drug candidates for the treatment of depression, including MDD, epilepsy, neuropathic pain, Parkinson's disease and other neurological conditions and diseases, including, but not limited to, Abbott Laboratories, Acadia, Alkermes, Allergan, AstraZeneca, Eli Lilly, GlaxoSmithKline, Johnson & Johnson, Lundbeck, Merck, Novartis, Minerva, Otsuka, Pfizer, Roche, Sage, Sanofi, Shire, Sumitomo Dainippon, and Takeda. Mergers and acquisitions in the biotechnology and pharmaceutical industries may result in even more resources being concentrated among a smaller number of our competitors. Our commercial opportunity could be reduced or eliminated if our competitors develop and commercialize products that are safer, more effective, have fewer or less severe side effects, are more convenient or are less expensive than any products that we may develop. Our competitors also may obtain FDA or other regulatory approval for their products more rapidly than we may obtain approval for ours, which could result in our competitors establishing a strong market position before we are able to enter the market. We expect that AV-101 will have to compete with a variety of therapeutic products and procedures.

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We believe that VistaStem's human pluripotent stem cell (hPSC) technology platform, the hPSC-derived human cells we produce, and the customized human cell-based assay systems we have formulated and developed are capable of being competitive in the diverse and growing global stem cell and regenerative medicine markets, including markets involving the sale of hPSC-derived cells to third-parties for their in vitro drug discovery and safety testing, contract predictive toxicology drug screening services for third parties, internal drug discovery, drug development and drug rescue of new NCEs, and regenerative medicine, including in vivo cell therapy research and development. A representative list of such biopharmaceutical companies pursuing one or more of these potential applications of adult and/or hPSC technology includes the following: Acea Biosciences, Astellas, Athersys, BioCardia, BioTime, Caladrius Biosciences, Collectis Bioresearch, Cellerant Therapeutics, Cytori Therapeutics, Fujifilm Holdings, HemoGenix, International Stem Cell, Neuralstem, Organovo Holdings, PluriStem Therapeutics, and Stemina BioMarker Discovery. Pharmaceutical companies and other established corporations such as Bristol-Myers Squibb, Charles River, GE Healthcare Life Sciences, GlaxoSmithKline, Novartis, Pfizer, Roche Holdings, Thermo Fisher Scientific and others have been and are expected to continue pursuing internally various stem cell-related research and development programs. Many of the foregoing companies have greater resources and capital availability and as a result, may be more successful in their research and development programs than us. We anticipate that acceptance and use of hPSC technology for drug development and regenerative medicine will continue to occur and increase at pharmaceutical and biotechnology companies in the future.

Government Regulation

Our business activities, including the manufacturing, research, development and marketing of our product candidates, are subject to extensive regulation by numerous governmental authorities in the United States and other countries. Before marketing in the United States, any new drug developed by us or our collaborators must undergo rigorous preclinical testing, clinical trials and an extensive regulatory clearance process implemented by the United States Food and Drug Administration (FDA) under the Federal Food, Drug, and Cosmetic Act, as amended. The FDA regulates, among other things, the development, testing, manufacture, safety, efficacy, record keeping, labeling, storage, approval, advertising, promotion, import, export, sale and distribution of biopharmaceutical products. The regulatory review and approval process, which includes preclinical testing and clinical trials of each product candidate, is lengthy, expensive and uncertain. Moreover, government coverage and reimbursement policies will both directly and indirectly impact our ability to successfully commercialize any future approved products, and such coverage and reimbursement policies will be impacted by enacted and any applicable future healthcare reform and drug pricing measures. In addition, we are subject to state and federal laws, including, among others, anti-kickback laws, false claims laws, data privacy and security laws, and transparency laws that restrict certain business practices in the pharmaceutical industry.

In the United States, drug product candidates intended for human use undergo laboratory and animal testing until adequate proof of safety is established. Clinical trials for new product candidates are then typically conducted in humans in three sequential phases that may overlap. Phase 1 trials involve the initial introduction of the product candidate into healthy human volunteers. The emphasis of Phase 1 trials is on testing for safety or adverse effects, dosage, tolerance, metabolism, distribution, excretion and clinical pharmacology. Phase 2 involves studies in a limited patient population to determine the initial efficacy of the compound for specific targeted indications, to determine dosage tolerance and optimal dosage, and to identify possible adverse side effects and safety risks. Once a compound shows evidence of effectiveness and is found to have an acceptable safety profile in Phase 2 evaluations, Phase 3 trials are undertaken to more fully evaluate clinical outcomes. Before commencing clinical investigations in humans, we or our collaborators must submit an Investigational New Drug Application (IND) to the FDA.

Regulatory authorities, Institutional Review Boards and Data Monitoring Committees may require additional data before allowing clinical studies to commence, continue or proceed from one phase to another, and could demand that

studies be discontinued or suspended at any time if there are significant safety issues. We have in the past and may in the future rely on assistance from our third-party collaborators and contract service providers to file our INDs and generally support our development and regulatory activities approval process for our potential products. Clinical testing must also meet requirements for clinical trial registration, institutional review board oversight, informed consent, health information privacy, and good clinical practices, or GCPs. Additionally, the manufacture of our drug product, must be done in accordance with current good manufacturing practices (GMPs).

To establish a new product candidate's safety and efficacy, the FDA requires companies seeking approval to market a drug product to submit extensive preclinical and clinical data, along with other information, for each indication for which the product will be labeled. The data and information are submitted to the FDA in the form of a New Drug Application (NDA), which must be accompanied by payment of a significant user fee unless a waiver or exemption applies. Generating the required data and information for an NDA takes many years and requires the expenditure of substantial resources. Information generated in this process is susceptible to varying interpretations that could delay, limit or prevent regulatory approval at any stage of the process. The failure to demonstrate adequately the quality, safety and efficacy of a product candidate under development would delay or prevent regulatory approval of the product candidate. Under applicable laws and FDA regulations, each NDA submitted for FDA approval is given an internal administrative review within 60 days following submission of the NDA. If deemed sufficiently complete to permit a substantive review, the FDA will "file" the NDA. The FDA can refuse to file any NDA that it deems incomplete or not properly reviewable. The FDA has established internal goals of eight months from submission for priority review of NDAs that cover product candidates that offer major advances in treatment or provide a treatment where no adequate therapy exists, and 12 months from submission for the standard review of NDAs. However, the FDA is not legally required to complete its review within these periods, these performance goals may change over time and the review is often extended by FDA requests for additional information or clarification. Moreover, the outcome of the review, even if generally favorable, may not be an actual approval but a "complete response letter" that describes additional work that must be done before the NDA can be approved. Before approving an NDA, the FDA can choose to inspect the facilities at which the product is manufactured and will not approve the product unless the manufacturing facility complies with GMPs. The FDA may also audit sites at which clinical trials have been conducted to determine compliance with GCPs and data integrity. The FDA's review of an NDA may also involve review and recommendations by an independent FDA advisory committee, particularly for novel indications. The FDA is not bound by the recommendation of an advisory committee.

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In addition, delays or rejections may be encountered based upon changes in regulatory policy, regulations or statutes governing product approval during the period of product development and regulatory agency review.

Before receiving FDA approval to market a potential product, we or our collaborators must demonstrate through adequate and well-controlled clinical studies that the potential product is safe and effective in the patient population that will be treated. In addition, under the Pediatric Research Equity Act, or PREA, an NDA or supplement to an NDA must contain data to assess the safety and effectiveness of the drug for the claimed indications in all relevant pediatric subpopulations and to support dosing and administration for each pediatric subpopulation for which the product is safe and effective, unless a waiver applies. If regulatory approval of a potential product is granted, this approval will be limited to those disease states and conditions for which the product is approved. Marketing or promoting a drug for an unapproved indication is generally prohibited. Furthermore, FDA approval may entail ongoing requirements for risk management, including post-marketing, or Phase 4, studies. Even if approval is obtained, a marketed product, its manufacturer and its manufacturing facilities are subject to payment of significant annual fees and continuing review and periodic inspections by the FDA. Discovery of previously unknown problems with a product, manufacturer or facility may result in restrictions on the product or manufacturer, including labeling changes, warning letters, costly recalls or withdrawal of the product from the market.

Any drug is likely to produce some toxicities or undesirable side effects in animals and in humans when administered at sufficiently high doses and/or for sufficiently long periods of time. Unacceptable toxicities or side effects may occur at any dose level at any time in the course of studies in animals designed to identify unacceptable effects of a product candidate, known as toxicological studies, or during clinical trials of our potential products. The appearance of any unacceptable toxicity or side effect could cause us or regulatory authorities to interrupt, limit, delay or abort the development of any of our product candidates. Further, such unacceptable toxicity or side effects could ultimately prevent a potential product's approval by the FDA or foreign regulatory authorities for any or all targeted indications or limit any labeling claims and market acceptance, even if the product is approved.

In addition, as a condition of approval, the FDA may require an applicant to develop a Risk Evaluation and Mitigation Strategy, or REMS. A REMS uses risk minimization strategies beyond the professional labeling to ensure that the benefits of the product outweigh the potential risks. To determine whether a REMS is needed, the FDA will consider the size of the population likely to use the product, seriousness of the disease, expected benefit of the product, expected duration of treatment, seriousness of known or potential adverse events, and whether the product is a new molecular entity. REMS can include medication guides, physician communication plans for healthcare professionals, and elements to assure safe use (ETASU). ETASU may include, but are not limited to, special training or certification for prescribing or dispensing, dispensing only under certain circumstances, special monitoring, and the use of patient registries. The FDA may require a REMS before approval or post-approval if it becomes aware of a serious risk associated with use of the product. The requirement for a REMS can materially affect the potential market and profitability of a product.

Any trade name that we intend to use for a potential product must be approved by the FDA irrespective of whether we have secured a formal trademark registration from the U.S. Patent and Trademark Office. The FDA conducts a rigorous review of proposed product names, and may reject a product name if it believes that the name inappropriately implies medical claims or if it poses the potential for confusion with other product names. The FDA will not approve a trade name until the NDA for a product is approved. If the FDA determines that the trade names of other products that are approved prior to the approval of our potential products may present a risk of confusion with our proposed trade name, the FDA may elect to not approve our proposed trade name. If our trade name is rejected, we will lose the benefit of any brand equity that may already have been developed for this trade name, as well as the benefit of our existing trademark applications for this trade name.

We and our collaborators and contract manufacturers also are required to comply with the applicable FDA GMP regulations. GMP regulations include requirements relating to quality control and quality assurance as well as the corresponding maintenance of records and documentation. Manufacturing facilities are subject to inspection by the FDA. These facilities must be approved before we can use them in commercial manufacturing of our potential products and must maintain ongoing compliance for commercial product manufacture. The FDA may conclude that we or our collaborators or contract manufacturers are not in compliance with applicable GMP requirements and other FDA regulatory requirements, which may result in delay or failure to approve applications, warning letters, product recalls and/or imposition of fines or penalties.

If a product is approved, we must also comply with post-marketing requirements, including, but not limited to, compliance with advertising and promotion laws enforced by various government agencies, including the FDA's Office of Prescription Drug Promotion, through such laws as the Prescription Drug Marketing Act, federal and state anti-fraud and abuse laws, including anti-kickback and false claims laws, healthcare information privacy and security laws, post-marketing safety surveillance, and disclosure of payments or other transfers of value to healthcare professionals and entities. In addition, we are subject to other federal and state regulation including, for example, the implementation of corporate compliance programs.

If we elect to distribute our products commercially, we must comply with state laws that require the registration of manufacturers and wholesale distributors of pharmaceutical products in a state, including, in certain states, manufacturers and distributors who ship products into the state even if such manufacturers or distributors have no place of business within the state. Some states also impose requirements on manufacturers and distributors to establish the pedigree of product in the chain of distribution, including some states that require manufacturers and others to adopt new technology capable of tracking and tracing product as it moves through the distribution chain.

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Outside of the United States, our ability to market a product is contingent upon receiving a marketing authorization from the appropriate regulatory authorities. The requirements governing the conduct of clinical trials, marketing authorization, pricing and reimbursement vary widely from country to country. At present, foreign marketing authorizations are applied for at a national level, although within the European Community (EC), centralized registration procedures are available to companies wishing to market a product in more than one EC member state. If the regulatory authority is satisfied that adequate evidence of safety, quality and efficacy has been presented, marketing authorization will be granted. This foreign regulatory development and approval process involves all of the risks associated with achieving FDA marketing approval in the U.S. as discussed above. In addition, foreign regulations may include applicable post-marketing requirements, including safety surveillance, anti-fraud and abuse laws, and implementation of corporate compliance programs and reporting of payments or other transfers of value to healthcare professionals and entities.

Reimbursement

Potential sales of AV-101 or any other future product candidate, if approved, will depend, at least in part, on the extent to which such products will be covered by third-party payors, such as government health care programs, commercial insurance and managed healthcare organizations. These third-party payors are increasingly limiting coverage and/or reducing reimbursements for medical products and services. A third-party payor's decision to provide coverage for a drug product does not imply that an adequate reimbursement rate will be approved. Further, one payor's determination to provide coverage for a drug product does not assure that other payors will also provide coverage for the drug product. In addition, the U.S. government, state legislatures and foreign governments have continued implementing cost-containment programs, including price controls, restrictions on reimbursement and requirements for substitution of generic products. Adoption of price controls and cost-containment measures, and adoption of more restrictive policies in jurisdictions with existing controls and measures, could further limit our future revenues and results of operations. Decreases in third-party reimbursement or a decision by a third-party payor to not cover AV-101, if approved, or any future approved products could reduce physician usage of our products, and have a material adverse effect on our sales, results of operations and financial condition.

In the United States, the Medicare Part D program provides a voluntary outpatient drug benefit to Medicare beneficiaries for certain products. We do not know whether AV-101, if approved, or any other future product candidate will be eligible for coverage under Medicare Part D, but individual Medicare Part D plans offer coverage subject to various factors such as those described above. In addition, while Medicare Part D plans have historically included "all or substantially all" drugs in the following designated classes of "clinical concern" on their formularies: anticonvulsants, antidepressants, antineoplastics, antipsychotics, antiretrovirals, and immunosuppressants, the Centers for Medicare and Medicaid Services (CMS) has in the past proposed, but not adopted, changes to this policy. If this policy is changed in the future and if CMS no longer considers the antidepressant class to be of "clinical concern", Medicare Part D plans would have significantly more discretion to reduce the number of products covered in that class. Furthermore, private payors often follow Medicare coverage policies and payment limitations in setting their own coverage policies.

Healthcare Laws and Regulations

Sales of AV-101, if approved, or any other future product candidate will be subject to healthcare regulation and enforcement by the federal government and the states and foreign governments in which we might conduct our business. The healthcare laws and regulations that may affect our ability to operate include the following:

The federal Anti-Kickback Statute makes it illegal for any person or entity to knowingly and willfully, directly or indirectly, solicit, receive, offer, or pay any remuneration that is in exchange for or to induce the referral of business, including the purchase, order, lease of any good, facility, item or service for which payment may be made under a federal healthcare program, such as Medicare or Medicaid. The term “remuneration” has been broadly interpreted to include anything of value.

Federal false claims and false statement laws, including the federal civil False Claims Act, prohibits, among other things, any person or entity from knowingly presenting, or causing to be presented, for payment to, or approval by, federal programs, including Medicare and Medicaid, claims for items or services, including drugs, that are false or fraudulent.

The U.S. federal Health Insurance Portability and Accountability Act of 1996 (HIPAA) created additional federal criminal statutes that prohibit among other actions, knowingly and willfully executing, or attempting to execute, a scheme to defraud any healthcare benefit program, including private third-party payors or making any false, fictitious or fraudulent statement in connection with the delivery of or payment for healthcare benefits, items or services.

HIPAA, as amended by the Health Information Technology for Economic and Clinical Health Act of 2009 (HITECH) and their implementing regulations, impose obligations on certain types of individuals and entities regarding the electronic exchange of information in common healthcare transactions, as well as standards relating to the privacy and security of individually identifiable health information.

The federal Physician Payments Sunshine Act requires certain manufacturers of drugs, devices, biologics and medical supplies for which payment is available under Medicare, Medicaid or the Children’s Health Insurance Program, with specific exceptions, to report annually to CMS information related to payments or other transfers of value made to physicians and teaching hospitals, as well as ownership and investment interests held by physicians and their immediate family members.

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Also, many states have similar laws and regulations, such as anti-kickback and false claims laws that may be broader in scope and may apply regardless of payor, in addition to items and services reimbursed under Medicaid and other state programs. Additionally, we may be subject to state laws that require pharmaceutical companies to comply with the federal government's and/or pharmaceutical industry's voluntary compliance guidelines, state laws that require drug manufacturers to report information related to payments and other transfers of value to physicians and other healthcare providers or marketing expenditures, as well as state and foreign laws governing the privacy and security of health information, many of which differ from each other in significant ways and often are not preempted by HIPAA.

Additionally, to the extent that our product is sold in a foreign country, we may be subject to similar foreign laws.

Stem Cell Technology - United States

With respect to our stem cell research and development in the U.S., the U.S. government has established requirements and procedures relating to the isolation and derivation of certain stem cell lines and the availability of federal funds for research and development programs involving those lines. All of the stem cell lines that we are using were either isolated under procedures that meet U.S. government requirements and are approved for funding from the U.S. government, or were isolated under procedures that meet U.S. government requirements.

All procedures we use to obtain clinical samples, and the procedures we use to isolate hESCs, are consistent with the informed consent and ethical guidelines promulgated by the U.S. National Academy of Science, the International Society of Stem Cell Research (ISSCR), or the NIH. These procedures and documentation have been reviewed by an external Stem Cell Research Oversight Committee, and all cell lines we use have been approved under one or more of these guidelines.

The U.S. government and its agencies on July 7, 2009 published guidelines for the ethical derivation of hESCs required for receiving federal funding for hESC research. Should we seek further NIH funding for our stem cell research and development, our request would involve the use of hESC lines that meet the NIH guidelines for NIH funding. In the U.S., the President's Council on Bioethics monitors stem cell research, and may make recommendations from time to time that could place restrictions on the scope of research using human embryonic or fetal tissue. Although numerous states in the U.S. are considering, or have in place, legislation relating to stem cell research, including California whose voters approved Proposition 71 to provide up to \$3 billion of state funding for stem cell research in California, it is not yet clear what affect, if any, state actions may have on our ability to commercialize stem cell technologies.

Subsidiaries and Inter-Corporate Relationships

VistaGen Therapeutics, Inc., a California corporation, dba VistaStem (VistaStem), is our wholly-owned subsidiary and has two wholly-owned subsidiaries: VistaStem Canada Inc., a corporation incorporated pursuant to the laws of the Province of Ontario, and Artemis Neuroscience, Inc., a corporation incorporated pursuant to the laws of the State of Maryland. The operations of VistaStem, and each of its wholly owned subsidiaries are managed by our senior management team based in South San Francisco, California.

Employees

As of December 7, 2017, we employed nine full-time employees, four of whom have doctorate degrees. Five full-time employees work in research and development and laboratory support services and four full-time employees work in general and administrative roles. Staffing for all other functional areas is achieved through our network of strategic relationships with service providers and consultants, each of whom provides services on a real-time, as-needed basis,

including human resources and payroll, information technology, facilities, legal, investor relations and website maintenance, regulatory affairs, and FDA program management.

We have never had a work stoppage, and none of our employees is represented by a labor organization or under any collective bargaining agreement. We consider our employee relations to be good.

Facilities

We lease our office and laboratory space, which consists of approximately 10,900 square feet located in South San Francisco, California, under a lease expiring on July 31, 2022.

Legal Proceedings

None.

Environmental Regulation

Our business does not require us to comply with any extraordinary environmental regulations.

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DIRECTORS AND EXECUTIVE OFFICERS

Our senior management is composed of individuals with significant management experience. Our directors and executive officers as of December 6, 2017 are as follows:

Name	Age	Position
Shawn K. Singh	54	Chief Executive Officer and Director
H. Ralph Snodgrass, Ph.D.	67	Founder, President, Chief Scientific Officer and Director
Mark A. Smith, M.D., Ph.D.	62	Chief Medical Officer
Jerrold D. Dotson	64	Vice President, Chief Financial Officer and Secretary
Mark McPartland	51	Vice-President, Corporate Development
Jon S. Saxe (1)	81	Director
Brian J. Underdown, Ph.D. (2)	76	Director
Jerry B. Gin, Ph.D., MBA (3)	74	Director

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- (1) Chairman of the audit committee and member of the compensation committee and corporate governance and nominating committee.
- (2) Chairman of the compensation committee and member of the audit committee and corporate governance and nominating committee.
- (3) Chairman of the corporate governance and nominating committee and member of the audit committee and compensation committee.

Executive Officers

Shawn K. Singh has served as our Chief Executive Officer since August 2009, first as the Chief Executive Officer of VistaGen Therapeutics, Inc., a California corporation (VistaGen California), then as Chief Executive Officer of the Company after the merger by and between VistaGen California and the Company on May 11, 2011 (the Merger), at which time VistaGen California became a wholly-owned subsidiary of the Company. Mr. Singh first joined the Board of Directors of VistaGen California in 2000 and served on the VistaGen California management team (part-time) from late-2003, following VistaGen California's acquisition of Artemis Neuroscience, of which he was President, to August 2009. In connection with the Merger, Mr. Singh was appointed as a member of our Board in 2011. Mr. Singh has over 25 years of experience working with biotechnology, medical device and pharmaceutical companies, both private and public. From February 2001 to August 2009, Mr. Singh served as Managing Principal of Cato BioVentures, a life science venture capital firm, and as Chief Business Officer and General Counsel of Cato Research Ltd, a profitable global contract research organization (CRO) affiliated with Cato BioVentures. Mr. Singh served as President (part-time) of Echo Therapeutics (NASDAQ: ECTE), a medical device company developing a non-invasive, wireless continuous glucose monitoring (CGM) system, from September 2007 to June 2009, and as a member of its Board of Directors from September 2007 through December 2011. He also served as Chief Executive Officer (part-time) of Hemodynamic Therapeutics, a private biopharmaceutical company affiliated with Cato BioVentures, from November 2004 to August 2009. From late-2000 to February 2001, Mr. Singh served as Managing Director of Start-Up Law, a management consulting firm serving biotechnology companies. Mr. Singh also served as Chief Business Officer of SciClone Pharmaceuticals (NASDAQ: SCLN), a revenue-generating, specialty pharmaceutical company with a substantial commercial business in China and a product portfolio spanning major therapeutics markets, including oncology, infectious diseases and cardiovascular disorders, from late-1993 to late-2000, and as a corporate finance associate of Morrison & Foerster LLP, an international law firm, from 1991 to late-1993. Mr. Singh currently serves as a member of the Board of Directors of Armour Therapeutics, a private biotechnology company focused on prostate cancer. Mr. Singh earned a B.A. degree, with honors, from the University of California, Berkeley, and a Juris Doctor degree from the University of Maryland School of Law. Mr. Singh is a member of the State Bar of California.

We selected Mr. Singh to serve on our Board of Directors due to his substantial practical experience and expertise in senior leadership roles with multiple private and public biotechnology, pharmaceutical and medical device companies, and his extensive experience in corporate finance, venture capital, corporate governance, drug development, intellectual property, regulatory affairs and strategic collaborations.

H. Ralph Snodgrass, Ph.D. co-founded VistaGen California with Dr. Gordon Keller in 1998 and served as the Chief Executive Officer of VistaGen California until August 2009. Dr. Snodgrass has served as the President and Chief Scientific Officer of VistaGen California from inception to the present, and in the same positions with the Company following the completion of the Merger. He served as a member of the Board of Directors of VistaGen California from 1998 to 2011, and was appointed to serve on our Board after the completion of the Merger. Prior to founding VistaGen California, Dr. Snodgrass served as a key member of the executive management team that led Progenitor, Inc., a biotechnology company focused on developmental biology, through its initial public offering, and was its Chief Scientific Officer from June 1994 to May 1998, and its Executive Director from July 1993 to May 1994. He received his Ph.D. in immunology from the University of Pennsylvania, and has 24 years of experience in senior biotechnology management and over 10 year's research experience as an assistant professor at the Lineberger Comprehensive Cancer Center, University of North Carolina Chapel Hill School of Medicine, and as a member of the Institute for Immunology, Basel, Switzerland. Dr. Snodgrass is a past Board Member of the Emerging Company Section of the Biotechnology Industry Organization (BIO), and past member of the International Society Stem Cell Research (ISSCR) Industry Committee. Dr. Snodgrass has published more than 50 scientific papers, is the inventor on more than 17 patents and a number of patent applications, is, or has been, the Principal Investigator on U.S. federal and private foundation sponsored research grants with budgets totaling more than \$14.5 million and is recognized as an expert in stem cell biology with more than 31 years' experience in the uses of stem cells as biological tools for research, drug discovery and development.

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We selected Dr. Snodgrass to serve on our Board of Directors due to his expertise in biotechnology focused on developmental biology, including stem cell biology, his extensive senior management experience leading biotechnology companies at all stages of development, as well as his reputation and standing in the fields of biotechnology and stem cell research, allow him to bring to us and the Board of Directors a unique understanding of the challenges and opportunities associated with pluripotent stem cell biology, as well as credibility in the markets in which we operate.

Mark A. Smith, M.D., Ph.D. joined VistaGen as our Chief Medical Officer effective June 18, 2016. Dr. Smith served as the Clinical Lead for Neuropsychiatry at Teva Pharmaceuticals from November 2013 through June 2016. He served as Senior Director of Experimental Medicine, Global Clinical Development and Innovation at Shire Pharmaceuticals from September 2012 to October 2013 and at AstraZeneca Pharmaceutical Company as Executive Director of Clinical Development and in other senior positions from June 2000 through September 2012. He served as a Senior Investigator and Principal Research Scientist in CNS Diseases Research at DuPont Pharmaceutical Company from 1996 to 2000 and in the Biological Psychiatry and Clinical Neuroendocrinology Branches of the National Institute of Mental Health from 1987 through 1996. Dr. Smith has significant expertise in drug discovery and development and clinical trial design and execution, having directed approximately fifty clinical trials from Phase 0 through Phase II B and served as project leader in both the discovery and development of approximately twenty investigational new drugs aimed at depression, anxiety, schizophrenia and other disorders. Dr. Smith received his Bachelor of Science and Master of Science degrees in Molecular Biophysics and Biochemistry from Yale University; his M.D and Ph.D. in Physiology and Pharmacology from the University of California, San Diego and completed his residency at Duke University Medical Center.

Jerrold D. Dotson, CPA has served as our Chief Financial Officer since September 2011, as our Corporate Secretary since October 2013 and as a Vice President since February 2014. Mr. Dotson served as Corporate Controller for Discovery Foods Company, a privately held Asian frozen foods company from January 2009 to September 2011. From February 2007 through September 2008, Mr. Dotson served as Vice President, Finance and Administration (principal financial and accounting officer) for Calypte Biomedical Corporation (OTCBB: CBMC), a publicly held biotechnology company. Mr. Dotson served as Calypte's Corporate Secretary from 2001 through September 2008. He also served as Calypte's Director of Finance from January 2000 through July 2005 and was a financial consultant to Calypte from August 2005 through January 2007. Prior to joining Calypte, from 1988 through 1999, Mr. Dotson worked in various financial management positions, including Chief Financial Officer, for California & Hawaiian Sugar Company, a privately held company. Mr. Dotson is licensed as a CPA in California and received his B.S. degree in Business Administration with a concentration in accounting from Abilene Christian College.

Mark A. McPartland has served as our Vice-President, Corporate Development since October 2016. Mr. McPartland previously served as the Vice President of Corporate Development and Communications at Stellar Biotechnologies, Inc. (NASDAQ: SBOT), a leader in sustainable manufacture of KLH, an immune-stimulating protein widely used in the field of active immunotherapy, from November 2013 to September 2016. While at Stellar, Mr. McPartland was responsible for transforming and expanding its capital markets and corporate communications strategy, while also supporting its global business development activities. From September 2011 to November 2013, Mr. McPartland served as Senior Vice President at MZ Group, a subsidiary of @titude Global, the world's largest independent global investor relations consulting firm, and from January 2005 to January 2011, he served as Vice President and Partner at Alliance Advisors, LLC where he specialized in the implementation of capital markets strategy, market positioning and financial communications, and Regional Vice President of Hayden Communications, Inc. where he led investor relations and corporate communications programs for micro and small cap companies. Mr. McPartland received his Bachelors in Business Administration and Marketing from Coastal Carolina University.

Directors

Jon S. Saxe, J.D., LL.M. has served as Chairman of our Board since 2000, first as Chairman of the Board of Directors of VistaGen California, then as Chairman of our Board after completion of the Merger. He also serves as the Chairman of our Audit Committee. Mr. Saxe is the retired President and was a director of PDL BioPharma from 1989 to 2008. From 1989 to 1993, he was President, Chief Executive Officer and a director of Synergen, Inc. (acquired by Amgen). Mr. Saxe served as Vice President, Licensing & Corporate Development for Hoffmann-Roche from 1984 through 1989, and Head of Patent Law for Hoffmann-Roche from 1978 through 1989. Mr. Saxe currently is a director of Durect Corporation (NASDAQ: DRRX), and six private life science companies, Arbor Vita Corporation, Arcuo Medical, LLC, Cancer Prevention Pharmaceuticals, Inc., Lumos Pharma, Inc., Trellis Bioscience, Inc. and Epalex Corporaiton. Mr. Saxe also has served as a director of other biotechnology and pharmaceutical companies, including ID Biomedical (acquired by GlaxoSmithKline), Sciele Pharmaceuticals, Inc. (acquired by Shionogi), Amalyte (acquired by Kemin Industries), Cell Pathways (acquired by OSI Pharmaceuticals), and other companies, both public and private. Mr. Saxe has a B.S.Ch.E. from Carnegie-Mellon University, a J.D. degree from George Washington University and an LL.M. degree from New York University.

We selected Mr. Saxe to serve as Chairman of our Board of Directors due to his numerous years of experience as a senior executive with major pharmaceutical and biotechnology companies, including Protein Design Labs, Inc., Synergen, Inc. and Hoffmann-Roche, Inc., as well as his extensive experience serving as a director of numerous private and public biotechnology and pharmaceutical companies, serving as Chairman, and Chair and member of audit, compensation and governance committees of both private and public companies. Mr. Saxe provides us and our Board of Directors with highly valuable insight and perspective into the biotechnology and pharmaceutical industries, as well as the strategic opportunities and challenges that we face.

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Brian J. Underdown, Ph.D. has served as a member of our Board of Directors since November 2009, first as a director of VistaGen California, then as a member of our Board after the completion of the Merger. Dr. Underdown is currently a Venture Partner with Lumira Capital Corp. having served as a Managing Director with Lumira from September 1997 through December 2015. His investment focus has been on therapeutics in both new and established companies in both Canada and the United States. Prior to joining Lumira and its antecedent company MDS Capital Corp., Dr. Underdown held a number of senior management positions in the biopharmaceutical industry and at universities. Dr. Underdown's current board positions include the following private companies: Kisoji Biotechnology Inc., Naegis Pharmaceuticals, Inc. and Osteo QC. Some of Dr. Underdown's previous board roles include: Argos Therapeutics (NASDAQ: ARGS), ID Biomedical (acquired by GlaxoSmithKline), enGene Inc. and Ception Therapeutics (acquired by Cephalon). He has served on a number of Boards and advisory bodies of government-sponsored research organizations including CANVAC, the Canadian National Centre of Excellence in Vaccines, Ontario Genomics Institute (Chair), Allergen Plc., the Canadian National Centre of Excellence in Allergy and Asthma. Dr. Underdown obtained his Ph.D. in immunology from McGill University and undertook post-doctoral studies at Washington University School of Medicine.

We selected Dr. Underdown to serve on our Board of Directors due to his extensive background working in the biotechnology and pharmaceutical industries, as a director of numerous private and public companies, as well as his substantial corporate finance and venture capital experience funding and advising startup and established biopharmaceutical companies focused on development and commercialization of novel therapeutics.

Jerry B. Gin, Ph.D., M.B.A was appointed to serve on our Board of Directors on March 29, 2016. Dr. Gin is currently the co-founder and CEO of Nuvora, Inc., a private company founded in 2006 with a drug delivery platform for the sustained release of ingredients through the mouth for such indications as dry mouth, biofilm reduction and sore throat/cough relief. Dr. Gin is also co-founder and Chairman of Livionex, a private platform technology company founded in 2009 and focused on oral care, ophthalmology and wound care. Previously, Dr. Gin co-founded Oculex Pharmaceuticals in 1993, which developed technology for controlled release delivery of drugs to the interior of the eye, specifically to treat macular edema, and served as President and CEO until it was acquired by Allergan in 2003. Prior to forming Oculex, Dr. Gin co-founded and took public ChemTrak, which developed a home cholesterol test commonly available in drug stores today. Prior to ChemTrak, Dr. Gin was Director of New Business Development and Strategic Planning for Syva, the diagnostic arm of Syntex Pharmaceuticals, Director for Pharmaceutical and Diagnostic businesses for Dow Chemical, and Director of BioScience Labs (now Quest Laboratories), the clinical laboratories of Dow Chemical. Dr. Gin received his Bachelor's degree in Chemistry from the University of Arizona, his Ph.D. in Biochemistry from the University of California, Berkeley, his M.B.A. from Loyola College, and conducted his post-doctoral research at the National Institutes of Health.

We selected Dr. Gin to serve on our Board of Directors due to his extensive experience in the healthcare industry, focusing his substantial business and scientific expertise on founding and developing numerous biopharmaceutical, diagnostic and biotechnology companies and propelling them to their next platforms of growth and value.

Election of Executive Officers

Our executive officers are elected by, and serve at the discretion of, our Board of Directors. Each of our executive officers devotes his full time to our affairs. There are no family relationships among any of our directors or executive officers.

Board Composition

Our amended and restated bylaws provide that the authorized number of directors of the Company shall be not less than one nor more than seven, with the exact number of directors currently fixed at seven. The exact number may be amended only by the vote or written consent of a majority of the outstanding shares of our voting stock. Our Board of Directors currently consists of five members. Accordingly, there are currently two vacancies on our Board of Directors. Our Board of Directors anticipates filling each of such vacancies as soon as practicable. All actions of the Board of Directors require the approval of a majority of the directors in attendance at a meeting at which a quorum is present.

Board Committees

Our Board of Directors has established an Audit Committee, a Compensation Committee and a Corporate Governance and Nominating Committee. The composition and responsibilities of each committee are described below. Members serve on these committees until their resignation or until otherwise determined by our Board of Directors. Effective on April 1, 2017, our independent directors, Mr. Saxe, Dr. Underdown and Dr. Gin, serve as members of each of these committees.

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Audit Committee

Our Audit Committee is comprised of Mr. Saxe, who serves as the committee chairman, Dr. Underdown and Dr. Gin. Mr. Saxe is also our Audit Committee financial expert, as that term is defined under SEC rules implementing Section 407 of the Sarbanes Oxley Act of 2002, and possesses the requisite financial sophistication, as defined under applicable rules. The Audit Committee operates under a written charter. Our Audit Committee charter is available on our website. Under its charter, our Audit Committee is primarily responsible for, among other things:

overseeing our accounting and financial reporting process;

selecting, retaining and replacing our independent auditors and evaluating their qualifications, independence and performance;

reviewing and approving scope of the annual audit and audit fees;

monitoring rotation of partners of independent auditors on engagement team as required by law;

discussing with management and independent auditors the results of annual audit and review of quarterly financial statements;

reviewing adequacy and effectiveness of internal control policies and procedures;

approving retention of independent auditors to perform any proposed permissible non-audit services;

overseeing internal audit functions and annually reviewing audit committee charter and committee performance; and

preparing the audit committee report that the SEC requires in our annual proxy statement.

Compensation Committee

Our Compensation Committee is comprised of Dr. Underdown, who serves as the committee chairman, Mr. Saxe, and Dr. Gin. Our Compensation Committee charter is available on our website. Under its charter, the Compensation Committee is primarily responsible for, among other things:

reviewing and approving our compensation programs and arrangements applicable to our executive officers (as defined in Rule 16a-I (f) of the Exchange Act), including all employment-related agreements or arrangements under which compensatory benefits are awarded or paid to, or earned or received by, our executive officers, including, without limitation, employment, severance, change of control and similar agreements or arrangements;

determining the objectives of our executive officer compensation programs;

ensuring corporate performance measures and goals regarding executive officer compensation are set and determining the extent to which they are achieved and any related compensation earned;

establishing goals and objectives relevant to CEO compensation, evaluating CEO performance in light of such goals and objectives, and determining CEO compensation based on the evaluation;

endeavoring to ensure that our executive compensation programs are effective in attracting and retaining key employees and reinforcing business strategies and objectives for enhancing stockholder value, monitoring the administration of incentive-compensation plans and equity-based incentive plans as in effect and as adopted from time to time by the board;

reviewing and approving any new equity compensation plan or any material change to an existing plan; and

reviewing and approving any stock option award or any other type of award as may be required for complying with any tax, securities, or other regulatory requirement, or otherwise determined to be appropriate or desirable by the committee or board.

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Corporate Governance and Nominating Committee

Our Corporate Governance and Nominating Committee is comprised of Dr. Gin, who serves as the committee chairman, Mr. Saxe and Dr. Underdown. Our Corporate Governance and Nominating Committee charter is available on our website. Under its charter, the Corporate Governance and Nominating Committee is primarily responsible for, among other things:

monitoring the size and composition of the board;

making recommendations to the board with respect to the nominations or elections of our directors;

reviewing the adequacy of our corporate governance policies and procedures and our Code of Business Conduct and Ethics, and recommending any proposed changes to the board for approval; and

considering any requests for waivers from our Code of Business Conduct and Ethics and ensure that we disclose such waivers as may be required by the exchange on which we are listed, if any, and rules and regulations of the SEC.

Code of Business Conduct and Ethics

We have adopted a Code of Business Conduct and Ethics applicable to our employees, officers and directors. Our Code of Business Conduct and Ethics is available on our website at www.vistagen.com. We intend to disclose any future amendments to certain provisions of our Code of Business Conduct and Ethics, or waivers of these provisions, on our website or in filings with the SEC under the Exchange Act.

Board Attendance at Board of Directors, Committee and Stockholder Meetings

Our Board of Directors met four times and acted by unanimous written consent six times during our fiscal year ended March 31, 2017. Our Audit Committee met four times. Our Compensation Committee requested action by the entire Board of Directors for grants of various equity securities and for amendments of employment agreements. Our Nominating and Corporate Governance Committee requested action by the entire Board of Directors with respect to resolutions to be presented to our stockholders at the annual meeting of stockholders and Board committee assignments. With the exception of Dr. Underdown, who was unable to attend one Board meeting due to international travel, each director serving during Fiscal 2017 attended all of the meetings of the Board and the committees of the Board upon which such director served that were held during the term of his service.

We do not have a formal policy regarding attendance by members of the Board at our annual meeting of stockholders, but directors are encouraged to attend. Mr. Saxe and Dr. Gin attended our annual meeting of stockholders held on September 26, 2016. Dr. Underdown was unavailable to participate due to international travel.

Compensation Committee Interlocks and Insider Participation

Our Compensation Committee consists of Dr. Underdown, Mr. Saxe and Dr. Gin, each of whom is a non-employee director. None of the members of the Compensation Committee has a relationship that would constitute an interlocking relationship with executive officers or directors of another entity.

Section 16 Beneficial Ownership Reporting Compliance

Section 16(a) of the Exchange Act requires our officers, directors and persons who beneficially own more than ten percent of our common stock (collectively, Reporting Persons) to file reports of ownership on Form 3 and changes in ownership on Form 4 or Form 5 with the SEC. The Reporting Persons are also required by SEC rules to furnish us with copies of all reports that they file pursuant to Section 16(a). We believe that during our fiscal year ended March 31, 2017, all Reporting Persons, other than PLTG and/or its affiliate, Montsant Partners LLC, and Cato Holding Company complied with all applicable reporting requirements.

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Executive Compensation

Our Compensation Objectives

Our compensation practices are designed to attract key employees and to retain, motivate and reward our executive officers for their performance and contribution to our long-term success. Our Board of Directors, through the compensation committee, seeks to compensate our executive officers by combining short and long-term cash and equity incentives. It also seeks to reward the achievement of corporate and individual performance objectives, and to align executive officers' incentives with stockholder value creation. When possible, the compensation committee seeks to tie individual goals to the area of the executive officer's primary responsibility. These goals may include the achievement of specific financial or business development goals. Also, when possible and appropriate taking into account the Company's financial condition and other related facts and circumstances, the compensation committee seeks to set performance goals that reach across all business areas and include achievements in finance/business development and corporate development.

The Compensation Committee makes decisions regarding salaries, annual bonuses, if any, and equity incentive compensation for our executive officers, approves corporate goals and objectives relevant to the compensation of the Chief Executive Officer and our other executive officers. The Compensation Committee solicits input from our Chief Executive Officer regarding the performance of our other executive officers. Finally, the Compensation Committee also administers our incentive compensation and benefit plans.

Although we have no formal policy for a specific allocation between current and long-term compensation, or cash and non-cash compensation, when possible and appropriate taking into account the Company's financial condition and other related facts and circumstances, we seek to implement a pay mix for our officers with a relatively equal balance of both, providing a competitive salary with a significant portion of compensation awarded on both corporate and personal performance.

Compensation Components

As a general rule, and when possible and appropriate taking into account the Company's financial condition and other related facts and circumstances, our compensation consists primarily of three elements: base salary, annual bonus and long-term equity incentives. We describe each element of compensation in more detail below.

Base Salary

Base salaries for our executive officers are established based on the scope of their responsibilities and their prior relevant experience, taking into account competitive market compensation paid by other companies in our industry for similar positions and the overall market demand for such executives, both initially at the time of hire and thereafter, to ensure that we retain our executive management team. An executive officer's base salary is also determined by reviewing the executive officer's other compensation to ensure that the executive officer's total compensation is in line with our overall compensation philosophy.

Base salaries are reviewed periodically as deemed necessary by the Compensation Committee and increased for merit reasons, based on the executive officers' success in meeting or exceeding individual objectives. Additionally, we may adjust base salaries as warranted throughout the year for promotions or other changes in the scope or breadth of an executive officer's role or responsibilities.

Annual Bonus

The Compensation Committee assesses the level of the executive officer's achievement of meeting individual goals, as well as that executive officer's contribution towards our corporate-wide goals. The amount of the cash bonus depends on the level of achievement of the individual performance goals, with a target bonus generally set as a percentage of base salary and based on the achievement of pre-determined milestones. To conserve our cash resources, our management team voluntarily decided to not seek and, in accordance with our management team's election, our Compensation Committee did not award, cash bonuses in any fiscal year from Fiscal 2012 through Fiscal 2015. The Compensation Committee authorized cash bonuses to officers who served during Fiscal 2016, which bonuses were paid in July 2016.

Long-Term Equity Incentives

The Compensation Committee believes that to attract and retain management, key employees and non-management directors the compensation paid to these persons should include, in addition to base salary and potential annual cash incentives, equity based compensation that is competitive with peer companies. The Compensation Committee determines the amount and terms of equity-based compensation granted under our stock option plans or pursuant to other awards made to our executives and key employees.

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Summary Compensation Table

The following table shows information regarding the compensation of our Named Executive Officers (NEO's) for services performed in the fiscal years ended March 31, 2017 and 2016:

Name and Principal Position	Fiscal Year	Salary (\$)	Bonus (\$)	Option and Warrant Awards (5) (\$)	All Other Compensation (\$)	Total (\$)
Shawn K. Singh (1) Chief Executive Officer	2017	385,107	173,750	752,210	(6) -	1,316,067
	2016	347,500	-	1,629,574	(7) -	1,977,074
H. Ralph Snodgrass, Ph.D. (2) President, Chief Scientific Officer	2017	340,625	152,500	520,946	(6) -	1,014,071
	2016	305,000	-	985,025	(7) -	1,290,025
Mark A. Smith, M.D., Ph.D. (3) Chief Medical Officer	2017	275,737	-	654,238	(6) -	929,975
	2016	-	-	-	-	-
Jerrold D. Dotson (4) Vice President, Chief Financial Officer, Secretary	2017	289,583	100,000	318,018	(6) -	707,601
	2016	250,000	-	635,297	(7) -	885,297

(1)

Mr. Singh became Chief Executive Officer of VistaGen Therapeutics, Inc. (a California corporation) (VistaGen California) on August 20, 2009 and our Chief Executive Officer in May 2011, in connection with the Merger. In our fiscal years ended March 31, 2017 and 2016, Mr. Singh's annual base cash salary, pursuant to his January 2010 employment agreement, as amended in June 2016, was contractually set at \$395,000 and \$347,500, respectively. Pursuant to his employment agreement, Mr. Singh is eligible to receive an annual cash incentive bonus of up to fifty percent (50%) of his base cash salary. To conserve cash for our operations during our fiscal year ended March 31, 2016, Mr. Singh voluntarily refrained from receiving any cash bonus.

(2)

Through August 20, 2009, Dr. Snodgrass served as VistaGen California's President and Chief Executive Officer, at which time he became its President and Chief Scientific Officer. He became our President and Chief Scientific Officer in May 2011, in connection with the Merger. In our fiscal years ended March 31, 2017 and 2016, Dr. Snodgrass' annual base cash salary, pursuant to his January 2010 employment agreement, as amended in June 2016, was contractually set at \$350,000 and \$305,000, respectively. Pursuant to his employment agreement, Dr. Snodgrass is eligible to receive an annual cash incentive bonus of up to fifty percent (50%) of his base cash salary. To conserve cash for our operations during our fiscal years ended March 31, 2016 and 2015, Dr. Snodgrass voluntarily refrained from receiving any cash bonus.

(3)

Dr. Smith became our Chief Medical Officer upon his employment effective June 18, 2016. During our fiscal year ended March 31, 2017, Dr. Smith's annual base cash salary was \$350,000.

(4)

Mr. Dotson served as Chief Financial Officer on a part-time contract basis from September 19, 2011 through August 2012, at which time he became our full-time employee. In our fiscal years ended March 31, 2017 and 2016, Mr. Dotson's annual base cash salary was \$300,000 and \$250,000, respectively. To conserve cash for our operations, Mr. Dotson did not receive a cash bonus in our fiscal year ended March 31, 2016.

(5)

The amounts in the Option and Warrant Awards column represent the aggregate grant date fair value of options and/or warrants to purchase restricted shares of our common stock awarded to Mr. Singh, Dr. Snodgrass, Dr. Smith and Mr. Dotson, and, in Fiscal 2016, the effect of modifications to prior grants of warrants, occurring during the fiscal year presented, computed in accordance with the Financial Accounting Standards Board's Accounting Standards Codification Topic 718, Compensation – Stock Compensation (ASC 718). The amounts in this column do not represent any cash payments actually received by Mr. Singh, Dr. Snodgrass, Dr. Smith or Mr. Dotson with respect to any of such options or warrants to purchase restricted shares of our common stock awarded to them or modified during the periods presented. To date, Mr. Singh, Dr. Snodgrass, Dr. Smith and Mr. Dotson have not exercised any of such options or warrants to purchase common stock, and there can be no assurance that any of them will ever realize any of the ASC 718 grant date fair value amounts presented in the Option and Warrant Awards column.

(6)

The table below provides information regarding the option awards we granted to Mr. Singh, Dr. Snodgrass, Dr. Smith and Mr. Dotson during Fiscal 2017 and the assumptions used in the Black Scholes Option Pricing Model to determine the grant date fair values of the respective awards and modifications.

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	Option Grant	Option Grant	
	6/19/2016	11/9/2016	Total
Singh	\$484,700	\$272,510	\$757,210
Snodgrass	302,938	218,008	520,946
Smith	436,230	218,008	654,238
Dotson	181,763	136,255	318,018
	\$1,405,631	\$844,781	\$2,250,412
Market price per share	\$3.49	\$3.80	
Exercise price per share	\$3.49	\$3.80	
Risk-free interest rate	1.31%	1.71%	
Volatility	79.82%	83.17%	
Expected term (years)	6.25	6.25	
Dividend rate	0%	0%	
Fair value per share	\$2.42	\$2.73	
Aggregate shares	580,000	310,000	

(7)

The table below provides information regarding the warrant awards and modifications we granted to Mr. Singh, Dr. Snodgrass and Mr. Dotson during fiscal 2016 and the assumptions used in the Black Scholes Option Pricing Model to determine the grant date fair values of the respective awards and modifications.

	Warrant Grant	Warrant Modification	
	9/2/2015	11/11/2015	Total
Singh	\$1,420,332	\$209,242	\$1,629,574
Snodgrass	852,199	132,826	985,025
Dotson	568,133	67,164	635,297
	\$2,840,664	\$409,232	\$3,249,896

Weighted Average

(except shares)

		Before	After
Market price per share	\$9.11	\$6.50	\$6.50
Exercise price per share	\$9.25	\$9.99	\$7.00
Risk-free interest rate	1.15%	1.75%	1.76
Volatility	77.19%	78.8%	78.75%
Expected term (years)	5	5.17	5.19
Dividend rate	0%	0%	0%
Fair value per share	\$5.68	\$3.67	\$4.09
Aggregate shares	500,000	952,803	952,803

Mr. Singh, Dr. Snodgrass and Mr. Dotson were granted warrants to purchase 250,000, 150,000 and 100,000 restricted shares of our common stock, respectively. We modified warrants to purchase an aggregate of 477,803 shares, 310,000 shares and 165,000 shares held by Mr. Singh, Dr. Snodgrass and Mr. Dotson, respectively.

None of the NEOs is entitled to perquisites or other personal benefits that, in the aggregate, are worth over \$50,000 or over 10% of their base salary.

Benefit Plans

401(k) Plan

We maintain, through a registered agent, a retirement and deferred savings plan for our officers and employees. This plan is intended to qualify as a tax-qualified plan under Section 401(k) of the Internal Revenue Code of 1986, as amended. The retirement and deferred savings plan provides that each participant may contribute a portion of his or her pre-tax compensation, subject to statutory limits. Under the plan, each employee is fully vested in his or her deferred salary contributions. Employee contributions are held and invested by the plan's trustee. The retirement and deferred savings plan also permits us to make discretionary contributions subject to established limits and a vesting schedule. To date, we have not made any discretionary contributions to the retirement and deferred savings plan on behalf of participating employees.

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Options and Warrants Granted to NEOs

The following table provides information regarding each unexercised stock option and warrant to purchase restricted shares of our common stock held by each of the named executive officers as of March 31, 2017:

	Number of Securities Underlying Unexercised Options (#) Exercisable	Number of Securities Underlying Unexercised Options (#) Unexercisable	Option Exercise Price (\$)	Option Expiration Date
Shawn K. Singh	2,000	-	14.40	5/17/2017
	1,000	-	10.00	1/17/2018
	1,000	-	10.00	1/17/2018
	3,000	-	10.00	3/24/2019
	1,125	-	10.00	6/17/2019
	50,000	-	10.00	11/4/2019
	21,250	-	10.00	12/30/2019
	5,000	-	10.00	4/26/2021
	4,017	-	7.00	3/19/2019
	1,786	-	7.00	3/19/2019
	72,000	-	7.00	3/3/2023
	150,000	-	7.00	1/11/2020
	250,000	-	7.00	9/2/2020
	-	200,000	(1) 3.49	6/19/2026
	11,111	(2) 88,889	(2) 3.80	11/9/2026
Total:	673,289	288,889		
H. Ralph Snodgrass, Ph.D.	2,500	-	10.00	3/24/2019
	1,250	-	10.00	6/17/2019
	12,500	-	10.00	12/30/2019
	50,000	-	7.00	3/3/2023
	2,500	-	7.00	3/19/2024
	7,500	-	7.00	3/19/2024
	100,000	-	7.00	1/11/2020
	150,000	-	7.00	9/20/2020
	-	125,000	(1) 3.49	6/19/2026
	8,888	(2) 71,112	(2) 3.80	11/9/2026
Total:	335,138	196,112		
Mark A. Smith, M.D. Ph.D.	-	180,000	(1) 3.49	6/19/2026
	8,888	(2) 71,112	(2) 3.80	11/9/2026
Total:	8,888	251,112		
Jerrold D. Dotson	5,001	-	10.00	10/30/2022
	1,000	-	8.00	10/27/2023
	10,000	-	7.00	3/3/2023

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	5,000	-	7.00	3/19/2024
	50,000	-	7.00	1/11/2020
	-	75,000	(1) 3.49	6/19/2026
	5,555	(2) 44,445	(2) 3.80	11/9/2026
Total:	76,556	119,445		

Represents an option to purchase shares of our common stock granted on June 19, 2016 when the market price of our common stock was \$3.49 per share. The option will become exercisable for 25% of the shares granted on (1) June 19, 2017 with the remaining shares becoming exercisable ratably monthly through June 19, 2020, when all shares granted will be fully exercisable.

(2) Represents an option to purchase shares of our common stock granted on November 9, 2016 when the market price of our common stock was \$3.80 per share. The option becomes exercisable for 1/36th of the shares granted each month beginning December 9, 2016 through November 9, 2019, when all shares granted will be fully exercisable.

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Subsequent to March 31, 2017, in April 2017 we granted options to our NEOs to purchase an aggregate total of 525,000 shares of common stock at \$1.96 per share, of which 175,000 options were granted to Mr. Singh, 125,000 options were granted to each of Drs. Snodgrass and Smith, and 100,000 options were granted to Mr. Dotson. In September 2017, we granted options to our NEOs to purchase an aggregate total of 425,000 shares of common stock at \$1.56 per share, of which 125,000 options were granted to Mr. Singh, 100,000 options were granted to each of Drs. Snodgrass and Smith, and 100,000 options were granted to Mr. Dotson.

Employment or Severance Agreements

We currently have employment agreements with Mr. Singh and Dr. Snodgrass.

Singh Agreement

We entered into an employment agreement with Mr. Singh on April 28, 2010. Under the agreement, as amended on June 22, 2016, Mr. Singh's base salary was increased from \$347,500 per year to \$395,000 per year, effective June 16, 2016. Although under his agreement, Mr. Singh is eligible to receive an annual incentive cash bonus of up to 50% of his base salary, he has foregone any such cash bonus payment to conserve cash for our operations during our fiscal years 2012 through 2015. Mr. Singh received a cash bonus in the amount of \$173,750 in July 2016, for his service during fiscal 2016. Payment of his annual incentive bonus is at the discretion of our Board of Directors. In the event we terminate Mr. Singh's employment without cause, he is entitled to receive severance in an amount equal to:

twelve months of his then-current base salary payable in the form of salary continuation;

a pro-rated portion of the incentive cash bonus that the Board of Directors determines in good faith that Mr. Singh earned prior to his termination; and

such amounts required to reimburse him for Consolidated Omnibus Budget Reconciliation Act (COBRA) payments for continuation of his medical health benefits for such twelve-month period.

In addition, in the event Mr. Singh terminates his employment with good reason following a change of control, he is entitled to twelve months of his then-current base salary payable in the form of salary continuation.

Snodgrass Agreement

We entered into an employment agreement with Dr. Snodgrass on April 28, 2010. Under the agreement, as amended on June 22, 2016, Dr. Snodgrass's base salary was increased from \$305,000 per year to \$350,000 per year, effective June 16, 2016. Although under his agreement, Dr. Snodgrass is eligible to receive an annual incentive cash bonus of up to 50% of his base salary, he has foregone any such cash bonus payment to conserve cash for our operations during our fiscal years 2012 through 2015. Dr. Snodgrass received a cash bonus in the amount of \$152,500 in July 2016, for his service during fiscal 2016. Payment of his annual incentive bonus is at the discretion of the Board of Directors. In the event we terminate Dr. Snodgrass's employment without cause, he is entitled to receive severance in an amount equal to:

twelve months of his then-current base salary payable in the form of salary continuation;

a pro-rated portion of the incentive bonus that the Board of Directors determines in good faith that Dr. Snodgrass earned prior to his termination; and

such amounts required to reimburse him for COBRA payments for continuation of his medical health benefits for such twelve-month period.

In addition, in the event Dr. Snodgrass terminates his employment with good reason, he is entitled to twelve months of his then-current base salary payable in the form of salary continuation.

Change of Control Provisions

Pursuant to each of their respective employment agreements, Dr. Snodgrass is entitled to severance if he terminates his employment at any time for “good reason” (as defined below), while Mr. Singh is entitled to severance if he terminates his employment for good reason after a change of control. Under their respective agreements, “good reason” means any of the following events, if the event is affected by us without the executive’s consent (subject to our right to cure):

a material reduction in the executive’s responsibility; or

a material reduction in the executive’s base salary except for reductions that are comparable to reductions generally applicable to similarly situated executives of VistaGen.

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Furthermore, pursuant to their respective employment agreements and their stock option award agreements, as amended, in the event we terminate the executive without cause within twelve months of a change of control, the executive's remaining unvested option shares become fully vested and exercisable. Upon a change of control in which the successor corporation does not assume the executive's stock options, the stock options granted to the executive become fully vested and exercisable.

Pursuant to their respective employment agreements, a change of control occurs when: (i) any "person" as such term is used in Sections 13(d) and 14(d) of the Exchange Act (other than VistaGen, a subsidiary, an affiliate, or a VistaGen employee benefit plan, including any trustee of such plan acting as trustee) becoming the "beneficial owner" (as defined in Rule 13d-3 under the Exchange), directly or indirectly, of securities of VistaGen representing 50% or more of the combined voting power of VistaGen's then outstanding securities; (ii) a sale of substantially all of VistaGen's assets; or (iii) any merger or reorganization of VistaGen whether or not another entity is the survivor, pursuant to which the holders of all the shares of capital stock of VistaGen outstanding prior to the transaction hold, as a group, fewer than 50% of the shares of capital stock of VistaGen outstanding after the transaction.

In the event that, following termination of employment, amounts are payable to an executive pursuant to his employment agreement, the executive's eligibility for severance is conditioned on executive having first signed a release agreement.

Pursuant to their respective employment agreements, the estimated amount that could be paid by us assuming that a change of control occurred on the last business day of our current fiscal year, is \$395,000 for Mr. Singh and \$350,000 for Dr. Snodgrass, excluding the imputed value of accelerated vesting of incentive stock options, if any.

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DIRECTOR COMPENSATION

We do not have a formal compensation plan for our non-employee directors. We adopted a director compensation policy for our independent directors, as independence is defined by the NASDAQ Stock Market, which became effective for our fiscal year beginning April 1, 2014. Under the independent director compensation policy, our independent directors are entitled to receive a \$25,000 annual retainer, payable in cash or shares of common stock. For service on a committee of the board, an independent director is entitled to receive an additional annual cash retainer as follows: \$7,500 for audit and compensation committee members and \$5,000 for nominating and governance committee members. In lieu of the annual cash retainer for committee participation, each independent director serving as a chair of a board committee shall receive the following annual cash retainer: \$15,000 for audit and compensation committee chairs and \$10,000 for the nominating and governance committee chairs. We paid our independent directors cash compensation consistent with the policy noted above during our fiscal year ended March 31, 2017. To conserve cash for our operations, we had accrued, but had not paid, our independent directors any cash compensation during the period January 1, 2012 through March 31, 2016. We paid all such unpaid amounts, aggregating \$278,500, during Fiscal 2017.

Under our director compensation policy, as updated in March 2016, each independent director will also receive an annual grant of an option or warrant to purchase a minimum of 12,000 shares of our common stock, which will vest monthly over a one-year period from the date of grant. Prorated grants will be made for partial years of service. In June 2016, we granted options to purchase 25,000 shares of our common stock at \$3.49 per share to each of our three independent directors. In November 2016, we granted options to purchase an additional 25,000 shares of our common stock at \$3.80 to each of the three independent directors. Subsequent to March 31, 2017, in April 2017 we granted options to purchase 35,000 shares of our common stock at \$1.96 per share, and in September 2017 we granted options to purchase 50,000 shares of our common stock at \$1.56 per share to each of our three independent directors.

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The following table sets forth a summary of the compensation earned by our non-employee directors in our fiscal year ended March 31, 2017.

Name	Fees Earned or Paid in Cash (1) (\$)	Option Awards (2) (\$)	Other Compensation (\$)	Total (\$)
Jon S. Saxe (3)	\$ 52,500	\$ 159,196 (6)	\$ –	\$ 211,696
Brian J. Underdown, Ph.D. (4)	\$ 57,500	\$ 159,196 (6)	\$ –	\$ 216,696
Jerry B. Gin, Ph.D., M.B.A (5)	\$ 32,500	\$ 159,196 (6)	\$ –	\$ 191,696

(1) The amounts shown represent fees earned for service on our Board of Directors, and Audit Committee, Compensation Committee and Corporate Governance and Nominating Committee during the fiscal year ended March 31, 2017, which amounts were paid in full during the fiscal year then ended. Fees paid during Fiscal 2017 for prior years' Board and committee service, \$136,500 to Mr. Saxe, and \$142,000 to Dr. Underdown, are excluded from the amounts shown as they had been reported, as appropriate, in the year in which they were accrued.

(2) The amounts in the Option Awards column represent the aggregate grant date fair value of options to purchase shares of our common stock awarded to Mr. Saxe, Dr. Underdown and Dr. Gin during our fiscal year ended March 31, 2017, computed in accordance with the Financial Accounting Standards Board's Accounting Standards Codification Topic 718, Compensation – Stock Compensation (ASC 718). The amounts in this column do not represent any cash payments actually received by Mr. Saxe, Dr. Underdown or Dr. Gin with respect to any of such warrants or options to purchase shares of our common stock awarded to them during the fiscal year ended March 31, 2017. To date, Mr. Saxe, Dr. Underdown and Dr. Gin have not exercised such warrants or options to purchase common stock, and there can be no assurance that any of them will ever realize any of the ASC 718 grant date fair value amounts presented in the Option and Warrant Awards column.

(3) Mr. Saxe has served as the Chairman of our Board of Directors, the Chairman of our Audit Committee and a member of our Compensation Committee and Corporate Governance and Nominating Committee throughout our fiscal year ended March 31, 2017. At March 31, 2017, Mr. Saxe holds: (i) 1,875 restricted shares of our common stock; (ii) options to purchase 61,875 registered shares of our common stock, of which options to purchase 14,652 shares are exercisable; and (iii) warrants to purchase 83,250 restricted shares of our common stock, all of which are exercisable.

(4) Dr. Underdown has served as a member of our Board of Directors, as the Chairman of our Compensation Committee and Corporate Governance and Nominating Committee and as a member of our Audit Committee throughout our fiscal year ended March 31, 2017. At March 31, 2017, Dr. Underdown holds: (i) options to purchase 59,250 registered shares of our common stock, of which options to purchase 12,027 shares are exercisable; and (ii) warrants to purchase 82,500 restricted shares of our common stock, all of which are exercisable.

(5) Dr. Gin was appointed to our Board of Directors and as a member of our Audit Committee on March 29, 2016 and served in those capacities throughout our fiscal year ended March 31, 2017. Effective on April 1, 2017, Dr. Gin was also appointed as a member of the Compensation Committee and assumed chairmanship of the Corporate Governance and Nominating Committee. At March 31, 2017, Dr. Gin holds options to purchase

75,000 registered shares of our common stock of which 27,777 are exercisable.

- (6) The table below provides information regarding the option awards we granted to Mr. Saxe, Dr. Underdown and Dr. Gin during Fiscal 2017 and the assumptions used in the Black Scholes Option Pricing Model to determine the grant date fair values of the respective awards and modifications.

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	Option Grant	Option Grant	
	6/19/2016	11/9/2016	Total
Saxe	\$76,803	\$82,393	\$159,196
Underdown	76,803	82,393	159,196
Gin	76,803	82,393	159,196
	\$230,409	\$247,179	\$477,588
Market price per share	\$3.49	\$3.80	
Exercise price per share	\$3.49	\$3.80	
Risk-free interest rate	1.62%	2.07%	
Volatility	96.16%	91.65%	
Expected term (years)	10.00	10.00	
Dividend rate	0%	0%	
Fair value per share	\$3.07	\$3.30	
Aggregate shares	75,000	75,000	

Mr. Saxe, Dr. Underdown and Dr. Gin were each granted options to purchase 25,000 shares of our common stock on both of the dates indicated.

Director Independence

Our securities are currently listed on The NASDAQ Capital Market, which has a requirement that a majority of our directors be independent. Accordingly, we evaluate independence by the standards for director independence established by applicable laws, rules, and listing standards, including, without limitation, the standards for independent directors established by the SEC and the NASDAQ Stock Market.

Subject to some exceptions, these standards generally provide that a director will not be independent if (a) the director is, or in the past three years has been, an employee of ours; (b) a member of the director's immediate family is, or in the past three years has been, an executive officer of ours; (c) the director or a member of the director's immediate family has received more than \$120,000 per year in direct compensation from us other than for service as a director (or for a family member, as a non-executive employee); (d) the director or a member of the director's immediate family is, or in the past three years has been, employed in a professional capacity by our independent public accountants, or has worked for such firm in any capacity on our audit; (e) the director or a member of the director's immediate family is, or in the past three years has been, employed as an executive officer of a company where one of our executive officers serves on the compensation committee; or (f) the director or a member of the director's immediate family is an executive officer of a company that makes payments to, or receives payments from, us in an amount which, in any twelve-month period during the past three years, exceeds the greater of \$1.0 million or two percent of that other company's consolidated gross revenues.

Our Board of Directors has undertaken a review of its composition, the composition of its committees and the independence of each director. Based upon information requested from and provided by each director concerning his

background, employment and affiliations, including family relationships, our Board of Directors has determined that Mr. Saxe, Dr. Underdown and Dr. Gin are “independent” as that term is defined under the applicable rules and regulations of the SEC and NASDAQ Stock Market. Our Board of Directors has also determined that Mr. Saxe, Dr. Underdown and Dr. Gin, who together comprise our audit committee, compensation committee, and corporate governance and nominating committee, satisfy the independence standards for those committees established by applicable SEC rules and NASDAQ Stock Market rules. In making these determinations, our Board of Directors considered the current and prior relationships that each non-employee director has with the Company and all other facts and circumstances that our Board of Directors deemed relevant.

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SECURITY OWNERSHIP OF CERTAIN BENEFICIAL OWNERS AND MANAGEMENT

The following table sets forth certain information with respect to the beneficial ownership of our common stock as of December 6, 2017 for:

each stockholder known by us to be the beneficial owner of more than 5% of our common stock;

each of our directors;

each of our named executive officers; and

all of our directors and executive officers as a group.

Applicable percentage ownership is based on 11,848,974 shares of common stock outstanding at December 6, 2017. In computing the number of shares of common stock beneficially owned by a person, we deemed to be outstanding all shares of common stock subject to options or warrants and all shares of preferred stock held by that person or entity that are currently exercisable or exchangeable or that will become exercisable or exchangeable within 60 days of December 6, 2017. In computing the percentage of shares beneficially owned, we deemed to be outstanding all shares of common stock subject to options or warrants and all shares of preferred stock held by that person or entity that are currently exercisable or exchangeable or that will become exercisable or exchangeable within 60 days of December 6, 2017. Unless otherwise noted below, the address of each beneficial owner listed in the table is c/o VistaGen Therapeutics, Inc., 343 Allerton Avenue, South San Francisco, California 94080.

Name and address of beneficial owner	Number of shares beneficially owned	Percent of shares beneficially owned (1)
Executive officers and directors:		
Shawn K. Singh (2)	760,021	6.04%
H. Ralph Snodgrass, Ph.D. (3)	511,508	4.16%
Mark A. Smith, M.D., Ph.D. (4)	146,804	1.22%
Jerrold D. Dotson (5)	265,262	2.19%
Mark McPartland (6)	79,443	*
Jon S. Saxe (7)	138,840	1.16%
Brian J. Underdown, Ph.D. (8)	133,589	1.11%
Jerry B. Gin, Ph.D., MBA (9)	216,839	1.81%
5% Stockholders:		
Platinum Long Term Growth Fund VII/Montsant Partners, LLC (10)	4,417,842	27.72%

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Sphera Global Healthcare Master Fund (11)	1,058,387	8.93%
Cato BioVentures (12)	907,294	7.66%
All executive officers and directors as a group (8 persons) (13)	2,252,296	16.19%

* less than 1%

(1) Based on 11,848,974 shares of common stock issued and outstanding as of December 6, 2017.

Includes options to purchase 255,984 shares of common stock exercisable within 60 days of December 6, 2017
(2) and warrants to purchase 477,803 restricted shares of common stock exercisable within 60 days of December 6, 2017.

Includes options to purchase 141,284 shares of common stock exercisable within 60 days of December 6, 2017
(3) and warrants to purchase 310,000 restricted shares of common stock exercisable within 60 days of December 6, 2017.

(4) Includes options to purchase 146,804 shares of common stock exercisable within 60 days of December 6, 2017.

Includes options to purchase 100,252 shares of common stock exercisable within 60 days of December 6, 2017,
(5) including options to purchase 676 shares of common stock held by Mr. Dotson's wife, and warrants to purchase 165,000 restricted shares of common stock exercisable within 60 days of December 6, 2017.

(6) Includes options to purchase 79,443 shares of common stock exercisable within 60 days of December 6, 2017.

(7) Includes options to purchase 53,714 shares of common stock exercisable within 60 days of December 6, 2017 and warrants to purchase 83,250 restricted shares of common stock exercisable within 60 days of December 6, 2017.

(8) Includes options to purchase 51,089 shares of common stock exercisable within 60 days of December 6, 2017 and warrants to purchase 82,500 restricted shares of common stock exercisable within 60 days of December 6, 2017.

Includes 50,000 restricted shares of common stock held by Dr. Gin's wife, options to purchase 66,839 shares of common stock exercisable within 60 days of December 6, 2017 and warrants to purchase 50,000 unregistered
(9) shares of common stock, including 25,000 shares held by Dr. Gin's wife, exercisable within 60 days of December 6, 2017. Excludes warrants to purchase an aggregate of 50,000 shares of unregistered common stock (including warrants to purchase 25,000 shares held by Dr. Gin's wife) not exercisable within 60 days of December 6, 2017.

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Based upon information contained in SEC Form 13G/A filed on February 18, 2015 by Platinum Long Term Growth Fund VII (PLTG) and adjusted to give effect to the transactions consummated between PLTG, Montsant Partners, LLC (Montsant), a PLTG affiliate, and Platinum Partners Value Arbitrage Fund, L.P. (In Official Liquidation) (PPVA), and us through December 6, 2017. The number of beneficially owned shares reported includes 637,500 restricted shares of common stock that may currently be acquired by Montsant upon fixed exchange of 425,000 restricted shares of our Series A Preferred Stock (Series A Preferred). Pursuant to the October 11, 2012 Note Exchange and Purchase Agreement by and between us and PLTG. There is, however, a limitation on exchange such that the number of shares of our common stock that may be acquired by PLTG or its affiliates upon exchange of the Series A Preferred is limited to the extent necessary to ensure that, following such exchange, the total number of shares of our common stock then beneficially owned by PLTG or its affiliates does (10) not exceed 9.99% of the total number of our then issued and outstanding shares of common stock without providing us with 61 days' prior notice thereof. Further, the reported number of shares beneficially owned by Montsant also includes 1,131,669 shares of common stock pursuant to its ownership of 1,131,669 shares of our Series B 10% Convertible Preferred Stock (Series B Preferred), immediately convertible into a like number of shares of our common stock. Pursuant to the terms of the Certificate of Designation of the Relative Rights and Preferences of the Series B 10% Convertible Preferred Stock, there is, however, a limitation on conversion of the Series B Preferred such that the number of shares of common stock that Montsant may beneficially acquire upon such conversion is limited to the extent necessary to ensure that, following such conversion, the total number of shares of common stock then beneficially owned by PLTG or Montsant does not exceed 9.99% of the total number of then issued and outstanding shares of our common stock without providing us with 61 days' prior notice thereof.

Further, the reported number of shares beneficially owned by Montsant also includes 2,318,012 shares of common stock pursuant to its ownership of 2,318,012 shares of our Series C Convertible Preferred Stock (Series C Preferred), immediately convertible on a fixed 1:1 conversion basis into a like number of shares of our restricted common stock. Pursuant to the terms of the Certificate of Designation of the Relative Rights and Preferences of the Series C Convertible Preferred Stock, there is, however, a limitation on conversion of the Series C Preferred such that the number of shares of common stock that Montsant may beneficially acquire upon such conversion is limited to the extent necessary to ensure that, following such conversion, the total number of shares of common stock then beneficially owned by PLTG or Montsant does not exceed 9.99% of the total number of then issued and outstanding shares of our common stock without providing us with 61 days' prior notice thereof. Excluding the shares otherwise subject to the beneficial ownership restrictions noted above, PLTG, Montsant and PPVA may be deemed to be the beneficial owner of 330,661 shares or 2.97% of our common stock.

Matthew Wright, Operating Manager of RHSW (Cayman) Ltd., and/or Moshe Feuer, Chief Executive Officer and authorized signatory of BAM may, subject to certain restrictions, be deemed to have voting and investment control over the shares held by PPVA, PLTG and/or Montsant. The address for PLTG, PPVA and Montsant is c/o BAM Administrative Services LLC, 105 Madison Avenue, 19th Floor, New York, NY 10016.

(11) Based upon information contained in SEC Form 13F filed on August 9, 2017, as updated to give effect to transactions through December 6, 2017 as recorded on our books. The number of shares reported excludes immediately exercisable warrants to purchase 482,965 registered shares of our common stock, which warrants are subject to a limitation on exercise such that the number of shares of common stock that Sphera Global Healthcare Master Fund and HFR HE Sphera Global Healthcare Master Trust (together, "Sphera") may beneficially acquire upon such exercise is limited to the extent necessary to ensure that, following such exercise, the total number of shares of common stock then beneficially owned by Sphera does not exceed 4.99% of the total number of issued and outstanding shares of our common stock without providing us with 61 days' prior notice thereof. Further excludes warrants to purchase an aggregate of 520,849 registered shares of our common

stock not exercisable within 60 days of December 6, 2017 but also subject to the 4.99% ownership limitation. The primary business address of Sphera Global Healthcare Master Fund and its affiliates is c/o Sphera Funds Management Ltd., 21 Ha'arba'ah Street, Tel Aviv 64739, Israel. Moshe Arkin and Sphera Funds Management Ltd. have joint voting and investment control over the shares held by Sphera.

- (12) Based upon information contained in SEC Form 4 filed on January 9, 2012, as updated to give effect to transactions through December 6, 2017 as recorded on our books. Lynda Sutton has voting and investment authority over the shares held by Cato Holding Company, dba Cato BioVentures. The primary business address of Cato BioVentures is 4364 South Alston Avenue, Durham, North Carolina 27713.

- (13) Includes options to purchase an aggregate of 804,574 shares of common stock exercisable within 60 days of December 6, 2017 and warrants to purchase an aggregate of 1,118,553 restricted shares of common stock exercisable within 60 days of December 6, 2017.

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Securities Authorized for Issuance Under Equity Compensation Plans

Equity Grants

As of March 31, 2017, options to purchase a total of 1,659,324 registered shares of our common stock were outstanding at a weighted average exercise price of \$4.76 per share, of which 351,532 options were vested and exercisable at a weighted average exercise price of \$8.27 per share and 1,307,792 were unvested and not exercisable at a weighted average exercise price of \$3.81 per share. These options were issued under our 2016 Plan and our 1999 Plan, each as described below. At March 31, 2017, an additional 1,184,911 shares remained available for future equity grants under our 2016 Plan.

Plan category	Number of securities to be issued upon exercise of outstanding options, warrants and rights (a)	Weighted-average exercise price of outstanding options, warrants and rights (b)	Number of securities remaining available for future issuance under equity compensation plans (excluding securities reflected in column (a)) (c)
Equity compensation plans approved by security holders	1,650,089	\$4.72	1,184,911
Equity compensation plans not approved by security holders	9,235	\$10.95	--
Total	1,659,324	\$4.76	1,184,911

Amended and Restated 2016 Stock Incentive Plan

Our Board unanimously approved the Company's Amended and Restated 2016 Stock Incentive Plan, formerly titled the 2008 Stock Incentive Plan (the 2016 Plan), on July 26, 2016, and the 2016 Plan was approved by our stockholders at our 2016 Annual Meeting of Stockholders on September 26, 2016, and further amended on September 15, 2017. The 2016 Plan provides for the grant of stock options, restricted shares of common stock, stock appreciation rights and dividend equivalent rights, collectively referred to as "Awards". Stock options granted under the 2016 Plan may be either incentive stock options under the provisions of Section 422 of the Internal Revenue Code of 1986, as amended (the Code), or non-qualified stock options. We may grant incentive stock options only to employees of the Company or any parent or subsidiary of the Company. Awards other than incentive stock options may be granted to employees, directors and consultants. A total of 10.0 million shares of our common stock are currently authorized for issuance under the 2016 Plan. As of December 7, 2017, 6,062,162 shares remain available for future equity grants under our 2016 Plan.

Below is a summary of the terms and conditions of the 2016 Plan. Unless otherwise indicated, all capitalized terms have the same meaning as defined in the 2016 Plan. This summary does not purport to be complete, and is qualified, in its entirety, by the specific language of the Amended and Restated 2016 Equity Incentive Plan.

Description of the 2016 Plan

The 2016 Plan provides for the grant of stock options, restricted shares of common stock, stock appreciation rights and dividend equivalent rights, collectively referred to as “Awards”. Stock options granted under the 2016 Plan may be either incentive stock options under the provisions of Section 422 of the Code, or non-qualified stock options. We may grant incentive stock options only to employees of the Company or any parent or subsidiary of the Company. Awards other than incentive stock options may be granted to employees, directors and consultants.

The Compensation Committee of the Board of Directors, referred to as the “Committee”, administers the 2016 Plan, including selecting the Award recipients, determining the number of shares to be subject to each Award, the exercise or purchase price of each Award and the vesting and exercise periods of each Award.

The exercise price of all incentive stock options granted under the 2016 Plan must be at least equal to 100% of the fair market value of the shares on the date of grant. If, however, incentive stock options are granted to an employee who owns stock possessing more than 10% of the voting power of all classes of our stock or the stock of any of our subsidiaries, the exercise price of any incentive stock option granted may not be less than 110% of the fair market value on the grant date. The maximum term of incentive stock options granted to employees who own stock possessing more than 10% of the voting power of all classes of our stock or the stock of any of our subsidiaries may not exceed five years. The maximum term of an incentive stock option granted to any other participant may not exceed 10 years. The Committee determines the term and exercise or purchase price of all other Awards granted under the 2016 Plan.

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Under the 2016 Plan, incentive stock options may not be sold, pledged, assigned, hypothecated, transferred or disposed of in any manner other than by will or by the laws of descent or distribution and may be exercised, during the lifetime of the participant, only by the participant. Other Awards shall be transferable:

by will and by the laws of descent and distribution; and

during the lifetime of the participant, to the extent and in the manner authorized by the Committee by gift or pursuant to a domestic relations order to members of the participant's Immediate Family (as defined in the 2016 Plan).

The 2016 Plan permits the designation of beneficiaries by holders of Awards, including incentive stock options. In the event of termination of a participant's service for any reason other than disability or death, such participant may, but only during the period specified in the Award agreement of not less than 30 days (generally 90 days) commencing on the date of termination (but in no event later than the expiration date of the term of such Award as set forth in the Award Agreement), exercise the portion of the Grantee's Award that was vested at the date of such termination or such other portion of the Grantee's Award as may be determined by the Committee. The Grantee's Award Agreement may provide that upon the termination of the participant's service for cause, the participant's right to exercise the Award shall terminate concurrently with the termination of the participant's service. In the event of a participant's change of status from employee to consultant, an employee's incentive stock option shall convert automatically into a non-qualified stock option on the day three months and one day following such change in status. To the extent that the Grantee's Award was unvested at the date of termination, or if the participant does not exercise the vested portion of the Grantee's Award within the period specified in the Award Agreement of not less than 30 days commencing on the date of termination, the Award shall terminate. If termination was caused by death or disability, any options that have become exercisable prior to the time of termination, will remain exercisable for twelve months from the date of termination (unless a shorter or longer period of time is determined by the Committee).

The maximum number of shares with respect to which options and stock appreciation rights may be granted to any participant in any calendar year will be 300,000 shares of common stock. In connection with a participant's commencement of service with the Company, a participant may be granted options and stock appreciation rights for up to an additional 50,000 shares that will not count against the foregoing limitation. In addition, for Awards of restricted stock and restricted shares of common stock that are intended to be "performance-based compensation" (within the meaning of Section 162(m) of the Code), the maximum number of shares with respect to which such Awards may be granted to any participant in any calendar year will be 300,000 shares of common stock. The limits described in this paragraph are subject to adjustment in the event of any change in our capital structure as described below.

The terms and conditions of Awards are determined by the Committee, including the vesting schedule and any forfeiture provisions. Awards under the 2016 Plan may vest upon the passage of time or upon the attainment of certain performance criteria. Although we do not currently have any Awards outstanding that vest upon the attainment of performance criteria, the Committee may establish criteria based on any one of, or combination of, the following:

increase in share price;

earnings per share;

total stockholder return;

operating margin;
gross margin;
return on equity;
return on assets;
return on investment;
operating income;
net operating income;
pre-tax profit;
cash flow;
revenue;
expenses;
earnings before interest, taxes and depreciation;
economic value added; and
market share.

Subject to any required action by our stockholders, the number of shares of common stock covered by outstanding Awards, the number of shares of common stock that have been authorized for issuance under the 2016 Plan, the exercise or purchase price of each outstanding Award, the maximum number of shares of common stock that may be granted subject to Awards to any participant in a calendar year, and the like, shall be proportionally adjusted by the Committee in the event of any increase or decrease in the number of issued shares of common stock resulting from certain changes in our capital structure as described in the 2016 Plan.

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Effective upon the consummation of a Corporate Transaction (as defined below), all outstanding Awards under the 2016 Plan will terminate unless the acquirer assumes or replaces such Awards. The Committee has the authority, exercisable either in advance of any actual or anticipated Corporate Transaction or Change in Control (as defined below) or at the time of an actual Corporate Transaction or Change in Control and exercisable at the time of the grant of an Award under the 2016 Plan or any time while an Award remains outstanding, to provide for the full or partial automatic vesting and exercisability of one or more outstanding unvested Awards under the 2016 Plan and the release from restrictions on transfer and repurchase or forfeiture rights of such Awards in connection with a Corporate Transaction or Change in Control, on such terms and conditions as the Committee may specify. The Committee also has the authority to condition any such Award vesting and exercisability or release from such limitations upon the subsequent termination of the service of the grantee within a specified period following the effective date of the Corporate Transaction or Change in Control. The Committee may provide that any Awards so vested or released from such limitations in connection with a Change in Control, shall remain fully exercisable until the expiration or sooner termination of the Award.

Under the 2016 Plan, a Corporate Transaction is generally defined as:

an acquisition of securities possessing more than fifty percent (50%) of the total combined voting power of our outstanding securities but excluding any such transaction or series of related transactions that the Committee determines shall not be a Corporate Transaction;

a reverse merger in which we remain the surviving entity but: (i) the shares of common stock outstanding immediately prior to such merger are converted or exchanged by virtue of the merger into other property, whether in the form of securities, cash or otherwise; or (ii) in which securities possessing more than fifty percent (50%) of the total combined voting power of our outstanding securities are transferred to a person or persons different from those who held such securities immediately prior to such merger;

a sale, transfer or other disposition of all or substantially all of the assets of the Company;

a merger or consolidation in which the Company is not the surviving entity; or

a complete liquidation or dissolution.

Under the 2016 Plan, a Change in Control is generally defined as: (i) the acquisition of more than 50% of the total combined voting power of our stock by any individual or entity which a majority of our Board of Directors (who have served on our board for at least 12 months) do not recommend our stockholders accept; (ii) or a change in the composition of our Board of Directors over a period of 12 months or less.

Unless terminated sooner, the 2016 Plan will automatically terminate in 2026. Our Board of Directors may at any time amend, suspend or terminate the 2016 Plan. To the extent necessary to comply with applicable provisions of U.S. federal securities laws, state corporate and securities laws, the Code, the rules of any applicable stock exchange or national market system, and the rules of any non-U.S. jurisdiction applicable to Awards granted to residents therein, we will obtain stockholder approval of any such amendment to the 2016 Plan in such a manner and to such a degree as required.

1999 Stock Incentive Plan

VistaGen California's Board of Directors adopted the 1999 Plan on December 6, 1999. The 1999 Plan terminated under its own terms in December 2009, and as a result, no awards may currently be granted under the 1999 Plan. However, the options and awards that have been granted pursuant to the 1999 Plan prior to its expiration remain operative.

The 1999 Plan permitted VistaGen California to make grants of incentive stock options, non-qualified stock options and restricted stock awards. VistaGen California initially reserved 45,000 restricted shares of its common stock for the issuance of awards under the 1999 Plan, which number was subject to adjustment in the event of a stock split, stock dividend or other change in capitalization. Prior to the 1999 Plan's expiration, shares that were forfeited or cancelled from awards under the 1999 Plan were generally available for future awards.

The 1999 Plan could be administered by either VistaGen California's Board of Directors or a committee designated by its Board of Directors. VistaGen California's Board of Directors designated its Compensation Committee as the committee with full power and authority to select the participants to whom awards were granted, to make any combination of awards to participants, to accelerate the exercisability or vesting of any award and to determine the specific terms and conditions of each award, subject to the provisions of the 1999 Plan. All directors, executive officers, and certain other key persons (including employees, consultants and advisors) of VistaGen California were eligible to participate in the 1999 Plan.

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The exercise price of incentive stock options awarded under the 1999 Plan could not be less than the fair market value of the common stock on the date of the option grant and could not be less than 110% of the fair market value of the common stock to persons owning stock representing more than 10% of the voting power of all classes of our stock. The exercise price of non-qualified stock options could not be less than 85% of the fair market value of the common stock. The term of each option granted under the 1999 Plan could not exceed ten years (or five years, in the case of an incentive stock option granted to a 10% stockholder) from the date of grant. VistaGen California's Compensation Committee determined at what time or times each option might be exercised (provided that in no event could it exceed ten years from the date of grant) and, subject to the provisions of the 1999 Plan, the period of time, if any, after retirement, death, disability or other termination of employment during which options could be exercised.

The 1999 Plan also permitted the issuance of restricted stock awards. Restricted stock awards issued by VistaGen California were shares of common stock that vest in accordance with terms and conditions established by VistaGen California's Compensation Committee. The Compensation Committee could impose conditions to vesting that it determined to be appropriate. Shares of restricted stock that did not vest were subject to our right of repurchase or forfeiture. VistaGen California's Compensation Committee determined the number of shares of restricted stock granted to any employee. Our 1999 Plan also gave VistaGen California's Compensation Committee discretion to grant stock awards free of any restrictions.

Unless the Compensation Committee provided otherwise, the 1999 Plan did not generally allow for the transfer of incentive stock options and other awards and only the recipient of an award could exercise an award during his or her lifetime. Non-qualified stock options were transferable only to the extent provided in the award agreement, in a manner consistent with the applicable law, and by will and by the laws of descent and distribution. In the event of a change in control of the Company, as defined in the 1999 Plan, the outstanding options will automatically vest unless our Board of Directors and the Board of Directors of the surviving or acquiring entity make appropriate provisions for the continuation or assumption of any outstanding awards under the 1999 Plan.

As of December 6, 2017, we have options outstanding under the 1999 Plan to purchase an aggregate of 4,533 shares of our common stock, the last of which, if not exercised before, expire in March 2018.

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CERTAIN RELATIONSHIPS AND RELATED TRANSACTIONS

Sales of Securities to Cato Holding Company

Cato Holding Company (CHC), doing business as Cato BioVentures (CBV), the parent of Cato Research Ltd. (CRL), was one of our largest institutional common stockholders at March 31, 2017, holding approximately 7% of our outstanding common stock. Shawn Singh, our Chief Executive Officer and member of our Board, served as Managing Principal of CBV and Chief Business Officer, and General Counsel of CRL from February 2001 until August 2009. In October 2012, we issued to CHC an unsecured promissory note in the principal amount of \$310,443 (the 2012 CHC Note) and a five-year warrant to purchase 12,500 restricted shares of the Company's common stock at a price of \$30.00 per share (the CHC Warrant).

Also in October 2012, we issued to CRL: (i) an unsecured promissory note in the initial principal amount of \$1.0 million, which was payable solely in restricted shares of our common stock and which accrued interest at the rate of 7.5% per annum, compounded monthly (the CRL Note), as payment in full for all contract research and development services and regulatory advice rendered to us by CRL through December 31, 2012 with respect to the preclinical and clinical development of AV-101, and (ii) a five-year warrant to purchase, at a price of \$20.00 per share, 50,450 restricted shares of our common stock (the CRL Warrant). Each of the CRL Note and 2012 CHC Note were scheduled to mature on March 31, 2016. In June 2015, the outstanding balance of the 2012 CHC Note, the CRL Note and all other outstanding amounts owed to CRL for CRO services were converted into 328,571 shares of our Series B Preferred, and the exercise prices of the CHC Warrant and the CRL Warrant were each reduced to \$7.00 per share. CHC participated in the February 2016 warrant exchange for common stock, exchanging the CHC Warrant and the CRL Warrant, as adjusted to reflect accrued interest, for an aggregate of 54,894 shares of our unregistered common stock. In May 2016, subsequent to our consummation of the May 2016 Public Offering, all of the shares of Series B Preferred held by CHC were automatically converted into registered shares of our common stock.

Contract Research and Development Agreement with Cato Research Ltd.

During 2007, we entered into a contract research organization master services agreement with CRL related to the development of and regulatory services related to AV101. In July 2017, we entered into a Master Services Agreement (MSA) with CRL, which replaced a similar May 2007 agreement, pursuant to which CRL may assist us in the evaluation, development, commercialization and marketing of our potential product candidates, including AV-101, and provide regulatory and strategic consulting services as requested from time to time. Specific projects or services will be delineated in individual work orders negotiated from time-to-time under the MSA. During the quarter ended September 30, 2014, we issued to CRL an aggregate of 300,000 unregistered shares of our common stock, having an aggregate fair value of \$525,000 on the date of issuance, for various services provided and to be provided under the terms of five negotiated AV-101- related work orders.

Under work orders related to such agreement, we incurred expenses of expenses of \$612,200 and \$78,200 for the six months ended September 30, 2017 and 2016, respectively and expenses of \$254,600 and \$52,600 for the fiscal years ended March 31, 2017 and 2016, respectively.

Participation in Spring 2017 Private Placement by Dr. Jerry Gin

Between late-March 2017 and August 2017, we sold to accredited investors, in a self-placed private placement, units consisting of an aggregate of 523,572 unregistered shares of our common stock and warrants to purchase up to an aggregate of 276,071 unregistered shares of our common stock pursuant to which we received proceeds of approximately \$1.04 million (the Spring 2017 Private Placement). Included with the accredited investors that

participated in the Spring 2017 Private Placement was Dr. Jerry Gin, a member of our Board of Directors, and his spouse, who together purchased an aggregate total of 100,000 shares of unregistered common stock and warrants to purchase up to 50,000 shares of common stock for an aggregate purchase price of \$200,000.

In September 2017, the Board approved the reduction of the exercise price from \$4.00 per share to \$2.00 per share for all warrants issued during the Spring 2017 Private Placement, as well as the issuance of additional warrants to purchase shares of the Company's common stock, with an exercise price of \$2.00 per share, to each of the participants in the Spring 2017 Private Placement. Accordingly, on September 15, 2017, the exercise price of aforementioned warrants issued to Dr. Gin and his spouse was decreased from \$4.00 to \$2.00 per share, and Dr. Gin and his spouse received additional warrants to purchase up to 50,000 shares of the Company's common stock for \$2.00 per share.

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DESCRIPTION OF SECURITIES

General

Our authorized capital stock consists of 100.0 million shares of our common stock, \$0.001 par value per share, and 10.0 million shares of preferred stock, \$0.001 par value per share. The following is a description of our common stock and certain provisions of our Articles, and our amended and restated bylaws (Bylaws), and certain provisions of Nevada law. This summary does not purport to be complete and is qualified in its entirety by the provisions of our Articles and our Bylaws, copies of which have been filed with the SEC as exhibits to our periodic filings under the Exchange Act.

As of December 6, 2017, there were issued and outstanding, or reserved for issuance:

11,848,974 shares of common stock held by approximately 1,300 stockholders of record;

750,000 shares of common stock reserved for issuance upon exchange of 500,000 outstanding shares of our Series A Preferred, all of which is held by one institutional investor and one accredited investor;

1,160,240 shares of common stock reserved for issuance upon exchange of our Series B Preferred held by two institutional investors and 663,460 shares reserved for issuance as payment of accrued dividends on outstanding shares of Series B Preferred;

2,318,012 shares of common stock reserved for issuance upon exchange of our Series C Preferred, all of which is held by one institutional investor;

7,107,157 shares of common stock that have been reserved for issuance upon exercise of outstanding warrants, with a weighted average exercise price of \$4.70 per share; and

3,279,871 shares of common stock reserved for issuance upon exercise of outstanding stock options under our 1999 Stock Incentive Plan and our Amended and Restated 2016 Stock Incentive Plan (2016 Plan), with a weighted average exercise price of \$3.23 per share.

Common Stock

Except as otherwise expressly provided in our Articles, or as required by applicable law, all shares of our common stock have the same rights and privileges and rank equally, share ratably and are identical in all respects as to all matters, including, without limitation, those described below. All outstanding shares of common stock are fully paid and nonassessable.

Voting Rights

Each holder of our common stock is entitled to cast one vote for each share of common stock held on all matters submitted to a vote of stockholders. Cumulative voting for election of directors is not allowed under our Articles, which means that a plurality of the shares voted can elect all of the directors then outstanding for election. Except as otherwise provided under Nevada law or our Articles and Bylaws, on matters other than election of directors, action on a matter is approved if the votes cast favoring the action exceed the votes cast opposing the action.

Dividend Rights

The holders of outstanding shares of our common stock are entitled to receive dividends out of funds legally available, if our board of directors, in its discretion, determines to issue dividend, and only at the times and in the amounts that our board of directors may determine. Our board of directors is not obligated to declare a dividend. We have not paid any dividends in the past and we do not intend to pay dividends in the foreseeable future. See “Dividend Policy” for more information.

Liquidation Rights

Upon our liquidation, dissolution or winding-up, the holders of our common stock will be entitled to share equally, identically and ratably in all assets remaining, subject to the prior satisfaction of all outstanding debt and liabilities and the preferential rights and payment of liquidation preferences, if any, on any outstanding shares of preferred stock.

No Preemptive or Similar Rights

Our common stock is not subject to conversion, redemption, sinking fund or similar provisions.

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Preferred Stock

We are authorized, subject to limitations prescribed by Nevada law, to issue up to 10.0 million shares of preferred stock in one or more series, to establish from time to time the number of shares to be included in each series and to fix the designation, powers, preferences and rights of the shares of each series and any of its qualifications, limitations or restrictions. Our board of directors can increase or decrease the number of shares of any series, but not below the number of shares of that series then outstanding, without any further vote or action by our stockholders. Our board of directors may authorize the issuance of preferred stock with voting or conversion rights that could adversely affect the voting power or other rights of the holders of the common stock. The issuance of preferred stock, while providing flexibility in connection with possible acquisitions and other corporate purposes, could, among other things, have the effect of delaying, deferring or preventing a change in control of the Company and may adversely affect the market price of our common stock and the voting and other rights of the holders of our common stock.

Series A Preferred

General

In December 2011, our board of directors authorized the creation of a series of up to 500,000 shares of Series A Preferred. The Certificate of Designation of the Relative Rights and Preferences of the Series A Convertible Preferred Stock was filed with the Nevada Secretary of State effective December 20, 2011.

Conversion and Rank

At December 6, 2017, there were 500,000 shares of Series A Preferred outstanding, which shares are exchangeable at the option of the holders into an aggregate of 750,000 shares of our common stock. The Series A Preferred ranks prior to our common stock for purposes of liquidation preference.

Conversion Restriction

At no time may a holder of shares of Series A Preferred convert shares of the Series A Preferred if the number of shares of common stock to be issued pursuant to such conversion would result in such holder beneficially owning (as determined in accordance with Section 13(d) of the Exchange Act and the rules thereunder) more than 9.99% of all of the common stock outstanding at such time; provided, however, that this limitation may be waived upon sixty-one (61) days notice to us.

Dividend Rights

The Series A Preferred has no separate dividend rights. However, whenever the board of directors declares a dividend on the common stock, each holder of record of a share of Series A Preferred, or any fraction of a share of Series A Preferred, on the date set by the board of directors to determine the owners of the common stock of record entitled to receive such dividend (Record Date) shall be entitled to receive out of any assets at the time legally available therefor, an amount equal to such dividend declared on one share of common stock multiplied by the number of shares of common stock into which such share, or such fraction of a share, of Series A Preferred could be exchanged on the Record Date.

Voting Rights

Except with respect to transactions upon which the Series A Preferred shall be entitled to vote separately as a class, the Series A Preferred has no voting rights. The common stock into which the Series A Preferred is exchangeable shall, upon issuance, have all of the same voting rights as other issued and outstanding shares of our common stock.

Liquidation Rights

In the event of the liquidation, dissolution or winding up of our affairs, after payment or provision for payment of our debts and other liabilities, the holders of Series A Preferred then outstanding shall be entitled to receive, out of our assets, if any, an amount per share of Series A Preferred calculated by taking the total amount available for distribution to holders of all of our outstanding common stock before deduction of any preference payments for the Series A Preferred, divided by the total of (x), all of the then outstanding shares of our common stock, plus (y) all of the shares of our common stock into which all of the outstanding shares of the Series A Preferred can be exchanged before any payment shall be made or any assets distributed to the holders of the common stock or any other junior stock.

Series B Preferred

General

In May 2015, our board of directors authorized the creation of a series of up to 4.0 million shares of Series B 10% Convertible Preferred Stock (Series B Preferred). The Certificate of Designation of the Relative Rights and Preferences of the Series B 10% Convertible Preferred Stock was filed with the Nevada Secretary of State on May 7, 2015 (the Series B Certificate of Designation).

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Conversion

Each share of Series B Preferred is convertible, at the option of the holder (Voluntary Conversion), into one (1) share of the Company's common stock. All outstanding shares of Series B Preferred are also automatically convertible into common stock (Automatic Conversion) upon the closing or effective date of any of the following transactions or events: (i) a strategic transaction involving AV-101 with an initial up front cash payment to the Company of at least \$10.0 million; (ii) a registered public offering of Common Stock with aggregate gross proceeds to the Company of at least \$10.0 million; or (iii) for 20 consecutive trading days the Company's Common Stock trades at least 20,000 shares per day with a daily closing price of at least \$12.00 per share; provided, however, that Automatic Conversion and Voluntary Conversion are subject to certain beneficial ownership blockers set forth in Section 6 of the Certificate of Designation.

At December 6, 2017, there were 1,160,240 shares of Series B Preferred outstanding, which shares are exchangeable at the option of the holder into an aggregate of 1,160,240 shares of our common stock.

Conversion Restriction

At no time may a holder of shares of Series B Preferred convert shares of the Series B Preferred, either by Voluntary Conversion or Automatic Conversion, if the number of shares of common stock to be issued pursuant to such conversion would result in such holder beneficially owning (as determined in accordance with Section 13(d) of the Exchange Act and the rules thereunder) more than 9.99% of all of the common stock outstanding at such time; provided, however, that this limitation may be waived upon sixty-one (61) days notice to us.

Rank

The Series B Preferred ranks prior to our common stock, and pari passu with the Series A Preferred for purposes of liquidation preference.

Dividend Rights

Prior to either a Voluntary Conversion or Automatic Conversion, shares of Series B Preferred will accrue dividends, payable only in common stock, at a rate of 10% per annum (the Accrued Dividend). The Accrued Dividend will be payable on the date of either a Voluntary Conversion or Automatic Conversion solely in that number of shares of Common Stock equal to the Accrued Dividend.

Voting Rights

Except with respect to transactions upon which the Series B Preferred shall be entitled to vote separately as a class, the Series B Preferred shall have no voting rights. The common stock into which the Series B Preferred shall be exchangeable shall, upon issuance, have all of the same voting rights as other issued and outstanding shares of our common stock.

Liquidation Rights

Upon any liquidation, dissolution, or winding-up of the Company, whether voluntary or involuntary, the holders of Series B Preferred are entitled to receive out of the Company's assets, whether capital or surplus, an amount equal to the stated value of the Series B Preferred (\$7.00 per share), plus any accrued and unpaid dividends thereon, before any distribution or payment shall be made to the holders of any junior securities, including holders of our common stock.

If the assets of the Company are insufficient to pay, in full, such amounts, then the entire assets to be distributed to the holders of the Series B Preferred shall be ratably distributed among the holders in accordance with the respective amounts that would be payable on such shares if all amounts payable thereon were paid in full.

Series C Preferred

General

In January 2016, our board of directors authorized the creation of a series of up to 3.0 million shares of Series C Convertible Preferred Stock (Series C Preferred). The Certificate of Designation of the Relative Rights and Preferences of the Series C Convertible Preferred Stock was filed with the Nevada Secretary of State, effective January 25, 2016 (the Series C Certificate of Designation).

Conversion and Rank

At December 6, 2017, there were 2,318,012 shares of Series C Preferred outstanding, which shares of Series C Preferred are exchangeable at the option of the holder into 2,318,012 shares of our common stock. The Series C Preferred ranks prior to our common stock for purposes of liquidation preference, and pari passu with the Series A Preferred and Series B Preferred.

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Conversion Restriction

At no time may a holder of shares of Series C Preferred convert shares of the Series C Preferred if the number of shares of common stock to be issued pursuant to such conversion would result in such holder beneficially owning (as determined in accordance with Section 13(d) of the Exchange Act and the rules thereunder) more than 9.99% of all of the common stock outstanding at such time; provided, however, that this limitation may be waived upon sixty-one (61) days notice to us.

Dividend Rights

The Series C Preferred has no separate dividend rights. However, whenever the board of directors declares a dividend on the common stock, each holder of record of a share of Series C Preferred, or any fraction of a share of Series C Preferred, on the date set by the board of directors to determine the owners of the common stock of record entitled to receive such dividend (Record Date) shall be entitled to receive out of any assets at the time legally available therefor, an amount equal to such dividend declared on one share of common stock multiplied by the number of shares of common stock into which such share, or such fraction of a share, of Series C Preferred could be exchanged on the Record Date.

Voting Rights

Except with respect to transactions upon which the Series C Preferred shall be entitled to vote separately as a class, the Series C Preferred has no voting rights. The common stock into which the Series C Preferred is exchangeable shall, upon issuance, have all of the same voting rights as other issued and outstanding shares of our common stock.

Liquidation Rights

In the event of the liquidation, dissolution or winding up of our affairs, after payment or provision for payment of our debts and other liabilities, the holders of Series C Preferred then outstanding shall be entitled to receive, out of our assets, if any, an amount per share of Series C Preferred calculated by taking the total amount available for distribution to holders of all of our outstanding common stock before deduction of any preference payments for the Series C Preferred, divided by the total of (x), all of the then outstanding shares of our common stock, plus (y) all of the shares of our common stock into which all of the outstanding shares of the Series C Preferred can be exchanged before any payment shall be made or any assets distributed to the holders of the common stock or any other junior stock.

Options

As of December 6, 2017, we had options to purchase 3,279,871 shares of our common stock outstanding pursuant to our 1999 Plan and our 2016 Plan.

Warrants

As of December 6, 2017, warrants to purchase 7,107,157 shares of our common stock were outstanding, with a weighted average exercise price of \$4.70 per share.

Warrants offered hereby

We are offering 8,333,333 shares of our common stock and warrants to purchase up to 8,333,333 shares of our common stock. Each share of common stock will be accompanied by a warrant to purchase one share of common stock at an exercise price per share equal to 100% of the combined public offering price per share and related warrant. The common stock and warrants are immediately separable but can only be purchased together in this offering.

The following is a brief summary of certain terms and conditions of the warrants to be issued in connection with this Offering and are subject in all respects to the provisions contained in the warrants.

Form

The warrants will be issued in electronic book-entry form to the investors. You should review a copy of the form of warrant, which is filed as an exhibit to the registration statement of which this prospectus forms a part, for a complete description of the terms and conditions applicable to the warrants.

Exercisability

The warrants are exercisable at any time after their original issuance, and at any time up to the date that is five years after their original issuance. Each of the warrants may be exercised, in whole or in part, by delivering to us a written notice of election to exercise the applicable warrant and delivering to us cash payment of the exercise price, if applicable. The exercise price and the number of shares of our common stock issuable upon exercise of the warrants is subject to adjustment in the event of certain subdivisions and combinations, including by any stock split or reverse stock split, stock dividend, recapitalization or otherwise.

Cashless Exercise: If, at any time during the term of the warrants, the issuance or resale of shares of our common stock upon exercise of the warrants is not covered by an effective registration statement, the holder is permitted to effect a cashless exercise of the warrants (in whole or in part) in which case the holder would receive upon such exercise the net number of shares of common stock determined according to the formula set forth in the warrants. Shares issued pursuant to a cashless exercise would be deemed to have been issued pursuant to the exemption from registration provided by Section 3(a)(9) of the Securities Act of 1933, and amended (the Securities Act), and the shares of common stock issued upon such cashless exercise would take on the characteristics of the Warrants being exercised, including, for purposes of Rule 144(d) promulgated under the Securities Act, a holding period beginning from the original issuance date of the warrants.

Adjustment Provisions: The exercise price and the number and type of securities purchasable upon exercise of the warrants are subject to adjustment upon certain corporate events, including certain subdivisions, combinations and similar events. If we declare any dividend or distribution of assets (including cash, stock or other securities, evidence of indebtedness, purchase rights or other property), each holder of a warrant will be entitled to participate in such distribution to the same extent that the holder would have participated had the applicable warrant been exercised immediately before the record date for the distribution. The terms of the warrants may make it difficult for us to raise additional capital at prevailing market terms in the future.

Transferability: Subject to applicable laws, the warrants may be offered for sale, sold, transferred or assigned without our consent. However, as of the date of this prospectus supplement there is no established trading market for the warrants and it is not expected that a trading market for the warrants will develop in the future. Without an active trading market, the liquidity of the warrants will be limited.

Listing: We will not apply to list the warrants on NASDAQ Capital Market. We do not intend to list the warrants on any securities exchange or other quotation system. Without an active market, the liquidity of the warrants will be limited.

Rights as a stockholder: Except as set forth in the warrants or by virtue of such holders' ownership of shares of our common stock, the holders of the warrants do not have the rights or privileges of holders of our common stock, including any voting rights, until they exercise the warrants.

Limitations on Exercise: The exercise of the warrants may be limited in certain circumstances if, after giving effect to such exercise, the holder or any of its affiliates would beneficially own (as determined in accordance with the terms of the warrants) more than 4.99% (or, at the election of the holder, 9.99%) of our outstanding common stock immediately after giving effect to the exercise.

Fundamental Transactions: In the event of certain fundamental transactions, as described in the warrants and generally including any merger or consolidation with or into another entity, the holders of the warrants shall thereafter have the right to exercise the applicable warrant for the same amount and kind of securities, cash or property as it would have been entitled to receive upon the occurrence of such fundamental transaction if it had been, immediately prior to such fundamental transaction, the holder of shares of common stock issuable upon exercise in full of the warrant. In the event of a Change of Control (as defined in the warrants) (other than a Change of Control which was not approved by the Board of Directors, as to which this right shall not apply), at the request of the holder delivered before the 30th day after such Change of Control, a holder of a warrant will have the right to require us or any successor entity to purchase the holder's warrant for the Black-Scholes Value of the remaining unexercised portion of the warrant on the effective date of such Change of Control (determined in accordance with a formula specified in the warrants), payable in cash; provided, that if the applicable Change of Control was not approved by our Board of Directors, such amount shall be payable, at our option in either (x) shares of our common stock or the consideration receivable by holders of common stock in the Change of Control transaction, as applicable, valued at the value of the consideration received by the shareholders in such Change of Control, or (y) cash.

Dividends and Other Distributions: If we declare or make any dividend or other distribution of our assets to holders of shares of our common stock (including any distribution of cash, stock or other securities, property, options, evidence of indebtedness or any other assets), then, subject to certain limitation on exercise described in the warrants, each holder of a warrant shall receive the distributed assets that such holder would have been entitled to receive in the distribution had the holder exercised the warrant immediately prior to the record date for the distribution.

Transfer Agent and Registrar

The transfer agent and registrar for our common stock is Computershare Trust Company, N.A., Jersey City, New Jersey.

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UNDERWRITING

We have entered into an underwriting agreement with Oppenheimer & Co. Inc., as the representative of the several underwriters named below. The underwriting agreement provides for the purchase by the several underwriters of the shares of common stock and related warrants offered hereby.

Subject to the terms and conditions of the underwriting agreement, we have agreed to sell to the underwriters, and each underwriter has severally agreed to purchase the number of shares of common stock and related warrants set forth opposite its name below:

Name	Number of Shares	Number of Warrants
Oppenheimer & Co. Inc.		
Chardan Capital Markets, LLC		
Total		

The underwriters have agreed to purchase all of the shares and related warrants offered by this prospectus if any are purchased.

The underwriters have advised us that they propose to offer the shares of common stock and related warrants directly to the public at the combined public offering price set forth on the cover page of this prospectus. In addition, the underwriters may offer some of the shares and related warrants to other securities dealers at such price less a concession of \$ per share and related warrants. The underwriters may also allow, and such dealers may re-allow, a concession not in excess of \$ per share and related warrants to other dealers. After the public offering of the shares of common stock and related warrants, the combined public offering price and other selling terms may be changed by the underwriters.

The following table shows the underwriting discount to be paid to the underwriters in connection with this offering.

	Per Share and Related Warrant	Total
Combined public offering price	\$	\$
Underwriting discount	\$	\$

We estimate that our total expenses of the offering, excluding the estimated underwriting discount, will be approximately \$340,000, which includes the costs and expenses for which we have agreed to reimburse the underwriters for certain expenses, including for fees and expenses of their legal counsel up to an amount of \$125,000, provided that any such costs and expenses may not exceed \$145,000 in the aggregate.

We have engaged Oppenheimer through September 30, 2018 to assist us in our evaluation of financing alternatives and to act as the sole underwriter, sole placement agent, sole selling agent, sole initial purchaser and/or sole dealer-manager, or other appropriate sole role as the case may be, on any capital-raising transaction, including the sole financial advisor on any negotiated exercise of our outstanding warrants, excluding any self-placed private placements by us of our securities to accredited individual investors. If during the term of Oppenheimer's engagement, we complete a financing or enter into an agreement that subsequently results in a completed financing, we have agreed to

pay Oppenheimer fees and commissions equal to 7% of the gross proceeds raised in the financing, subject to certain exceptions. We also agreed to reimburse Oppenheimer for all of its reasonable expenses (including reasonable fees and expenses of its legal counsel), subject to certain agreed upon caps.

We have agreed to indemnify the underwriters against certain liabilities, including liabilities under the Securities Act.

We and our officers and directors have agreed to a 90-day “lock-up” with respect to shares of our common stock and other of our securities that they beneficially own, including securities that are convertible into shares of common stock and securities that are exchangeable or exercisable for shares of common stock. This means that, subject to certain exceptions, for a period of 90 days following the date of this prospectus, we and such persons may not offer, sell, pledge or otherwise dispose of these securities without the prior written consent of Oppenheimer & Co. Inc.

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The underwriters have advised us that they do not intend to conduct any stabilization or over-allotment activities in connection with this offering.

Electronic Delivery of Preliminary Prospectus: A prospectus in electronic format may be delivered to potential investors by the underwriters. The prospectus in electronic format will be identical to the paper version of such preliminary prospectus. Other than the prospectus in electronic format, the information on any underwriter's website and any information contained in any other website maintained by such underwriters is not part of this prospectus or the registration statement of which this prospectus forms a part.

Other: The underwriters and their affiliates have provided in the past and may provide from time to time in the future certain commercial banking, financial advisory, investment banking and other services for us and our affiliates in the ordinary course of their business, for which they may receive customary fees and commissions. Oppenheimer acted as the sole underwriter of the September 2017 Public Offering. In connection therewith, Oppenheimer received a discount of approximately \$168,000 and reimbursement of expenses in the aggregate amount of \$95,000. Pursuant to engagement letters, each dated September 24, 2017, we retained each of Oppenheimer and Chardan Capital Markets, LLC (Chardan) to assist us in our evaluation of certain financial and strategic alternatives and, in connection therewith, issued to each firm 75,000 unregistered shares of our common stock and agreed to reimburse each of them for their reasonable expenses incurred in connection therewith. In addition, from time to time, the underwriters and their affiliates may effect transactions for their own accounts or the accounts of customers, and hold on behalf of themselves or their customers, long or short positions in our debt or equity securities or loans, and may do so in the future. The 75,000 shares of common stock issued to Oppenheimer, and the 75,000 shares of common stock issued to Chardan are deemed to be compensation by the Financial Industry Regulatory Authority, Inc., or FINRA, and are therefore subject to FINRA Rule 5110(g)(1). In accordance with FINRA Rule 5110(g)(1), such shares may not be sold, transferred, assigned, pledged, or hypothecated, or be the subject of any hedging, short sale, derivative, put, or call transaction that would result in the effective economic disposition of such securities by any person for a period of 180 days immediately following the date of effectiveness or commencement of sales of the common stock in this offering, except the transfer of any shares:

by operation of law or by reason of our reorganization;

to any FINRA member firm participating in this offering and the officers or partners thereof, if all shares so transferred remain subject to the lock-up restriction described above for the remainder of the time period;

if the aggregate amount of our securities held by either the underwriters or a related person do not exceed 1% of the securities being offered;

that is beneficially owned on a pro-rata basis by all equity owners of an investment fund, provided that no participating member manages or otherwise directs investments by the fund, and participating members in the aggregate do not own more than 10% of the equity in the fund; or

the exercise or conversion of any security, if all securities received remain subject to the lock-up restriction set forth above for the remainder of the time period.

Oppenheimer owns 75,000 shares of our common stock. Chardan owns 97,429 shares of our common stock.

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NOTICE TO INVESTORS

Offer Restrictions Outside the United States

Other than in the United States, no action has been taken by us or the underwriters that would permit a public offering of the securities offered by this prospectus in any jurisdiction where action for that purpose is required. The securities offered by this prospectus may not be offered or sold, directly or indirectly, nor may this prospectus or any other offering material or advertisements in connection with the offer and sale of any such securities be distributed or published in any jurisdiction, except under circumstances that will result in compliance with the applicable rules and regulations of that jurisdiction. Persons into whose possession this prospectus comes are advised to inform themselves about and to observe any restrictions relating to the offering and the distribution of this prospectus. This prospectus does not constitute an offer to sell or a solicitation of an offer to buy any securities offered by this prospectus in any jurisdiction in which such an offer or a solicitation is unlawful.

European Economic Area

In relation to each Member State of the European Economic Area which has implemented the Prospectus Directive (each, a Relevant Member State) an offer to the public of any securities which are the subject of the offering contemplated by this prospectus supplement may not be made in that Relevant Member State other than the offers contemplated in this prospectus in name(s) of Member State(s) where prospectus will be approved or passported for the purposes of a non-exempt offer once this prospectus has been approved by the competent authority in such Member State and published and passported in accordance with the Prospectus Directive as implemented in name(s) of relevant Member State(s) except that an offer to the public in that Relevant Member State of any securities may be made at any time under the following exemptions under the Prospectus Directive, if they have been implemented in that Relevant Member State:

- (a) to legal entities which are authorized or regulated to operate in the financial markets or, if not so authorized or regulated, whose corporate purpose is solely to invest in securities;
- (b) to any legal entity which has two or more of (1) an average of at least 250 employees during the last financial year; (2) a total balance sheet of more than €43,000,000 and (3) an annual net turnover of more than €50,000,000, as shown in its last annual or consolidated accounts;
- (c) by the representative to fewer than 100 natural or legal persons (other than qualified investors as defined in the Prospectus Directive); or
- (d) in any other circumstances falling within Article 3(2) of the Prospectus Directive, provided that no such offer of securities shall result in a requirement for the publication by the Company or any underwriter of a prospectus pursuant to Article 3 of the Prospectus Directive.

For the purposes of this provision, the expression an “offer to the public” in relation to any securities in any Relevant Member State means the communication in any form and by any means of sufficient information on the terms of the offer and any securities to be offered so as to enable an investor to decide to purchase any securities, as the same may be varied in that Member State by any measure implementing the Prospectus Directive in that Member State and the expression “Prospectus Directive” means Directive 2003/71/EC and includes any relevant implementing measure in each Relevant Member State.

United Kingdom

The underwriters have represented, warranted and agreed that:

(a) they have only communicated or caused to be communicated and will only communicate or cause to be communicated any invitation or inducement to engage in investment activity (within the meaning of section 21 of the Financial Services and Markets Act 2000 (the FSMA)) received by them in connection with the issue or sale of any securities in circumstances in which section 21(1) of the FSMA does not apply to the Company; and

(b) they have complied with and will comply with all applicable provisions of the FSMA with respect to anything done by them in relation to the securities in, from or otherwise involving the United Kingdom.

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Israel

In the State of Israel, the securities offered hereby may not be offered to any person or entity other than the following:

- (a) a fund for joint investments in trust (i.e., mutual fund), as such term is defined in the Law for Joint Investments in Trust, 5754-1994, or a management company of such a fund;
- (b) a provident fund as defined in Section 47(a)(2) of the Income Tax Ordinance of the State of Israel, or a management company of such a fund;
- (c) an insurer, as defined in the Law for Oversight of Insurance Transactions, 5741-1981;
- (d) a banking entity or satellite entity, as such terms are defined in the Banking Law (Licensing), 5741-1981, other than a joint services company, acting for their own account or for the account of investors of the type listed in Section 15A(b) of the Securities Law 1968;
- (e) a company that is licensed as a portfolio manager, as such term is defined in Section 8(b) of the Law for the Regulation of Investment Advisors and Portfolio Managers, 5755-1995, acting on its own account or for the account of investors of the type listed in Section 15A(b) of the Securities Law 1968;
- (f) a company that is licensed as an investment advisor, as such term is defined in Section 7(c) of the Law for the Regulation of Investment Advisors and Portfolio Managers, 5755-1995, acting on its own account;
- (g) a company that is a member of the Tel Aviv Stock Exchange, acting on its own account or for the account of investors of the type listed in Section 15A(b) of the Securities Law 1968;
- (h) an underwriter fulfilling the conditions of Section 56(c) of the Securities Law, 5728-1968;
- (i) a venture capital fund (defined as an entity primarily involved in investments in companies which, at the time of investment, (i) are primarily engaged in research and development or manufacture of new technological products or processes and (ii) involve above-average risk);
- (j) an entity primarily engaged in capital markets activities in which all of the equity owners meet one or more of the above criteria; and
- (k) an entity, other than an entity formed for the purpose of purchasing securities in this offering, in which the shareholders equity (including pursuant to foreign accounting rules, international accounting regulations and U.S. generally accepted accounting rules, as defined in the Securities Law Regulations (Preparation of Annual Financial Statements), 1993) is in excess of NIS 50 million.

Any offeree of the securities offered hereby in the State of Israel shall be required to submit written confirmation that it falls within the scope of one of the above criteria. This prospectus will not be distributed or directed to investors in the State of Israel who do not fall within one of the above criteria.

In Canada

The securities subject to this offering are not qualified for sale in Canada and may not be offered or sold in Canada, directly or indirectly, on our behalf.

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LEGAL MATTERS

The validity of the securities offered by this prospectus will be passed upon by Disclosure Law Group, a Professional Corporation, San Diego, California (DLG). Partners of DLG beneficially own an aggregate of 84,487 registered and/or restricted shares of our common stock. Lowenstein Sandler LLP, New York, NY, is acting as counsel for the underwriters in connection with this offering.

EXPERTS

The financial statements as of March 31, 2017 and 2016, and for the years then ended, included in this prospectus, have been audited by OUM & Co. LLP, an independent registered public accounting firm, as stated in their report appearing herein. Such financial statements have been so included in reliance upon the report of such firm given upon their authority as experts in accounting and auditing.

WHERE YOU CAN FIND MORE INFORMATION

We are a public company and file annual, quarterly and special reports, proxy statements and other information with the SEC. You may read and copy any document we file at the SEC's public reference room at 100 F Street, NE, Washington, D.C. 20549. You can request copies of these documents by writing to the SEC and paying a fee for the copying cost. Please call the SEC at 1-800-SEC-0330 for more information about the operation of the public reference room. Our SEC filings are also available, at no charge, to the public at the SEC's website at <http://www.sec.gov>.

We have filed with the SEC a Registration Statement on Form S-1 under the Securities Act with respect to the shares of common stock being offered by us under this prospectus. This prospectus is part of that registration statement. This prospectus does not contain all of the information set forth in the registration statement or the exhibits to the registration statement. For further information with respect to us and the securities we are offering pursuant to this prospectus, you should refer to the registration statement and its exhibits. Statements contained in this prospectus as to the contents of any contract, agreement or other document referred to are not necessarily complete, and you should refer to the copy of that contract or other documents filed as an exhibit to the registration statement. You may read or obtain a copy of the registration statement at the SEC's public reference facilities and Internet site referred to above.

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REPORT OF INDEPENDENT REGISTERED PUBLIC ACCOUNTING FIRM

To the Board of Directors and Stockholders
VistaGen Therapeutics, Inc.

We have audited the accompanying consolidated balance sheets of VistaGen Therapeutics, Inc. as of March 31, 2017 and 2016 and the related consolidated statements of operations and comprehensive loss, cash flows, and stockholders' equity (deficit) for each of the two fiscal years in the period ended March 31, 2017. These financial statements are the responsibility of the Company's management. Our responsibility is to express an opinion on these consolidated financial statements based on our audits.

We conducted our audits in accordance with the standards of the Public Company Accounting Oversight Board (United States). Those standards require that we plan and perform the audit to obtain reasonable assurance about whether the financial statements are free of material misstatement. The Company is not required to have, nor were we engaged to perform, an audit of its internal control over financial reporting. Our audits included consideration of internal control over financial reporting as a basis for designing audit procedures that are appropriate in the circumstances, 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. An audit also includes examining, on a test basis, evidence supporting the amounts and disclosures in the financial statements, assessing the accounting principles used and significant estimates made by management, as well as evaluating the overall financial statement presentation. We believe that our audits provide a reasonable basis for our opinion.

In our opinion, the consolidated financial statements referred to above present fairly, in all material respects, the consolidated financial position of VistaGen Therapeutics, Inc. at March 31, 2017 and 2016, and the consolidated results of its operations and its cash flows for each of the two fiscal years in the period ended March 31, 2017, in conformity with U.S. generally accepted accounting principles.

The accompanying consolidated financial statements have been prepared assuming that the Company will continue as a going concern. As discussed in Note 2 to the consolidated financial statements, the Company has not yet generated sustainable revenues, has suffered recurring losses and negative cash flows from operations and has minimal stockholders' equity, all of which raise substantial doubt about its ability to continue as a going concern. The consolidated financial statements do not include any adjustments that might result from the outcome of this uncertainty.

/s/ OUM & Co. LLP

San Francisco, California
June 28, 2017

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VISTAGEN THERAPEUTICS, INC.

CONSOLIDATED BALANCE SHEETS

(Amounts in dollars, except share amounts)

	March 31,	March 31,
	2017	2016
ASSETS		
Current assets:		
Cash and cash equivalents	\$2,921,300	\$428,500
Prepaid expenses and other current assets	456,600	426,800
Total current assets	3,377,900	855,300
Property and equipment, net	286,500	87,600
Security deposits and other assets	47,800	46,900
Total assets	\$3,712,200	\$989,800
LIABILITIES AND STOCKHOLDERS' EQUITY (DEFICIT)		
Current liabilities:		
Accounts payable	\$867,300	\$936,000
Accrued expenses	443,000	814,000
Current portion of notes payable and accrued interest	54,800	43,600
Capital lease obligations	2,400	1,100
Total current liabilities	1,367,500	1,794,700
Non-current liabilities:		
Notes payable	-	27,200
Accrued dividends on Series B Preferred Stock	1,577,800	2,089,600
Deferred rent liability	139,200	55,500
Capital lease obligations	11,900	-
Total non-current liabilities	1,728,900	2,172,300
Total liabilities	3,096,400	3,967,000
Commitments and contingencies		

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Stockholders' equity (deficit):

Preferred stock, \$0.001 par value; 10,000,000 shares authorized at March 31, 2017 and March 31, 2016:

Series A Preferred, 500,000 shares authorized and outstanding at March 31, 2017 and March 31, 2016	500	500
Series B Preferred; 4,000,000 shares authorized at March 31, 2017 and March 31, 2016; 1,160,240 shares and 3,663,077 shares issued and outstanding at March 31, 2017 and March 31, 2016, respectively	1,200	3,700
Series C Preferred: 3,000,000 shares authorized at March 31, 2017 and March 31, 2017; 2,318,012 shares issued and outstanding at March 31, 2017 and March 31, 2016	2,300	2,300
Common stock, \$0.001 par value; 30,000,000 shares authorized at March 31, 2017 and March 31, 2016;		
8,974,386 and 2,623,145 shares issued at March 31, 2017 and March 31, 2016, respectively	9,000	2,600
Additional paid-in capital	146,569,600	132,725,000
Treasury stock, at cost, 135,665 shares of common stock held at March 31, 2017 and March 31, 2016	(3,968,100)	(3,968,100)
Accumulated deficit	(141,998,700)	(131,743,200)
Total stockholders' equity (deficit)	615,800	(2,977,200)
Total liabilities and stockholders' equity (deficit)	\$3,712,200	\$989,800

See accompanying notes to consolidated financial statements.

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VISTAGEN THERAPEUTICS, INC.

CONSOLIDATED STATEMENTS OF OPERATIONS AND COMPREHENSIVE LOSS

(Amounts in dollars, except share amounts)

	Fiscal Years Ended March 31,	
	2017	2016
Revenues:		
Sublicense fees	\$1,250,000	\$-
Total revenues	1,250,000	-
Operating expenses:		
Research and development	5,203,700	3,931,600
General and administrative	6,294,800	13,918,600
Total operating expenses	11,498,500	17,850,200
Loss from operations	(10,248,500)	(17,850,200)
Other expenses, net:		
Interest expense, net	(4,600)	(770,800)
Change in warrant liability	-	(1,894,700)
Loss on extinguishment of debt	-	(26,700,200)
Other expense	-	(2,300)
Loss before income taxes	(10,253,100)	(47,218,200)
Income taxes	(2,400)	(2,300)
Net loss and comprehensive loss	(10,255,500)	(47,220,500)
Accrued dividend on Series B Preferred stock	(1,257,000)	(2,140,500)
Deemed dividend on Series B Preferred Units	(111,100)	(2,058,000)
Net loss attributable to common stockholders	\$(11,623,600)	\$(51,419,000)
Basic and diluted net loss attributable to common stockholders per common share	\$(1.54)	\$(29.08)
Weighted average shares used in computing basic and diluted net loss attributable to common stockholders per common share		