VistaGen Therapeutics, Inc.
Form 10-K
June 25, 2014

UNITED STATES

SECURITIES AND EXCHANGE COMMISSION
Washington, D.C. 20549

Form 10-K

x Annual Report Pursuant to Section 13 or 15(d) of the Securities Exchange Act of 1934

For the fiscal year ended: March 31, 2014

or

o Transition Report Pursuant to Section 13 or 15(d) of the Securities Exchange Act of 1934

Commission file number: 000-54014

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

Nevada	20-5093315
(State or other jurisdiction of incorporation or organization)	(I.R.S. Employer Identification No.)

343 Allerton Avenue
South San Francisco, California 94080
(650) 577-3600
(Address, including zip code, and telephone number, including area code, of registrant's principal executive office)

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

None

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

Common Stock, par value $0.001 per share

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

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

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

o

Indicate by check mark whether the registrant has submitted electronically and posted on its corporate Web site, if any, every Interactive Data File required to be submitted and posted pursuant to Rule 405 of Regulation S-T (§232.405 of this chapter) during the preceding 12 months (or for such shorter period that the registrant was required to submit and post such files). Yes S No o

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

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

Large accelerated filer o Accelerated filer o Non-accelerated filer o Smaller reporting company x
(Do not check if a smaller reporting company)

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

The aggregate market value of the common stock of the registrant held by non-affiliates of the registrant on September 30, 2013, the last business day of the registrant's second fiscal quarter was: $9,655,600.

As of June 19, 2014 there were 25,451,877 shares of the registrant's common stock outstanding.

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Special Note Regarding Forward-Looking Statements

This Annual Report on Form 10-K includes forward-looking statements. All statements contained in this Annual Report on Form 10-K other than statements of historical fact, including statements regarding our future results of operations and financial position, our business strategy and plans, and our objectives for future operations, are forward- looking statements. The words “believe,” “may,” “estimate,” “continue,” “anticipate,” “intend,” “expect” and expressions are intended to identify forward-looking statements. 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 financial condition, results of operations, business strategy, short-term and long-term business operations and objectives, and financial needs. These forward-looking statements are subject to a number of risks, uncertainties and assumptions, including those described in the “Risk Factors” section. Moreover, we operate in a very competitive and rapidly changing environment. New risks emerge from time to time. It is not possible for our management to predict all risks, nor can we assess the impact of all factors on our business or the extent to which any factor, or combination of factors, may cause actual results to differ materially from those contained in any forward-looking statements we may make. In light of these risks, uncertainties and assumptions, the future events and trends discussed in this Annual Report on Form 10-K may not occur and actual results could differ materially and adversely from those anticipated or implied in the forward-looking statements.

You should not rely upon forward-looking statements as predictions of future events. The events and circumstances reflected in the forward-looking statements may not be achieved or occur. Although we believe that the expectations reflected in the forward-looking statements are reasonable, we cannot guarantee future results, levels of activity, performance or achievements. We are under no duty to update any of these forward-looking statements after the date of this Annual Report on Form 10-K or to conform these statements to actual results or revised expectations. If we do update one or more forward-looking statements, no inference should be drawn that we will make additional updates with respect to those or other forward looking statements.

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PART I

Item 1. Business

We were first incorporated in California on May 26, 1998. We merged with Excaliber Enterprises, Ltd., a Nevada corporation (Excaliber), a publicly held company, on May 11, 2011, and shortly thereafter changed our name to "VistaGen Therapeutics, Inc." 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. "VistaGen California" refer to VistaGen Therapeutics, Inc., a California corporation and our wholly owned subsidiary.

We are a stem cell company headquartered in South San Francisco, California focused on drug rescue and regenerative medicine. We believe better cells lead to better medicine™ and that the key to making better cells is precisely controlling the differentiation of human pluripotent stem cells, which are the building blocks of all cells of the human body. For over 15 years, our stem cell research, development teams and collaborators have focused on controlling the differentiation of pluripotent stem cells to produce multiple types of mature, functional, adult human cells, with emphasis on human heart and liver cells.

Our stem cell technology platform - Human Clinical Trials in a Test Tube™

Our stem cell technology platform, which we refer to as Human Clinical Trials in a Test Tube, is based on a combination of proprietary and exclusively licensed technologies for controlling the differentiation of human pluripotent stem cells into multiple types of mature, functional, adult human cells that we use, or plan to develop, to reproduce complex human biology and disease. We are currently producing human heart cells and liver cells for our drug rescue applications. However, we also intend to advance, internally and through collaborative research projects, production of pluripotent stem cell-derived blood, bone, cartilage, and pancreatic beta-islet cells and explore ways to leverage our stem cell technology platform for regenerative medicine purposes. Our interest in the regenerative medicine arena is on developing novel human disease models for discovery of small molecule drugs and biologics that activate the endogenous growth and healing processes enabling the body to repair tissue damage caused by certain degenerative diseases.

CardioSafe 3D™

Using mature cardiomyocytes (heart cells) differentiated from human pluripotent stem cells, we have developed CardioSafe 3D, as a novel, in vitro bioassay system used to assess new drug candidates for potential cardiac toxicity before they are tested in humans. We believe CardioSafe 3D is capable of predicting the in vivo cardiac effects, both toxic and non-toxic, of small molecule drug candidates with greater speed and precision than the long-established, surrogate safety models most often used in drug development, including models using animal cells or live animals, and cellular assays using cadaver, immortalized or transformed cells. Our pluripotent stem cell derived cardiomyocytes (heart cells) and CardioSafe 3D are key components of our Human Clinical Trials in a Test Tube platform and drug rescue programs.

LiverSafe 3D™

Using mature, functional adult hepatocytes (liver cells) derived from human pluripotent stem cells, we are correlating LiverSafe 3D, our second novel stem cell technology-based bioassay system, with reported clinical results. We believe LiverSafe 3D will enable us to assess, early in development, new drug candidates for potential drug-induced liver toxicity and particularly metabolism issues that can result in serious adverse drug-drug interactions, before animal or human testing. We plan to use LiverSafe 3D, and the clinically predictive liver biology insight we believe it will provide us, to expand the scope of our commercial opportunities related to drug rescue.

Drug Rescue

We believe drug rescue, using our novel in vitro bioassay systems, CardioSafe 3D and LiverSafe 3D, the foundation of our Human Clinical Trials in a Test Tube platform, is the highest-value near term commercial application of the human cells we produce. Detailed information is available to us in the public domain regarding the efficacy, pharmacology, formulation and toxicity of promising small molecule drug candidates developed by pharmaceutical and biotechnology companies which have failed due to unexpected heart or liver toxicity. These failed but still promising drug candidates, which we refer to as Drug Rescue Candidates™, have already been optimized and tested by a pharmaceutical or biotechnology company and assessed for efficacy and commercial potential.

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Failure of promising Drug Rescue Candidates due to unexpected human clinical toxicity highlights the need for new paradigms to evaluate potential heart and liver toxicity early in drug development. While efforts of pharmaceutical and biotechnology companies to improve their prediction of such human clinical toxicity for new drug candidates is ongoing, the existence of Drug Rescue Candidates™ offers us an opportunity to use our novel stem cell technology to take advantage of prior third-party investment in Drug Rescue Candidates with early signs of efficacy, by significantly reducing the toxicity that caused them to be terminated, and bring new, safer versions back into development protected by new intellectual property. We refer to the new, safer versions of Drug Rescue Candidates we intend to produce with our medicinal chemistry collaborator and validate internally in our bioassay systems as Drug Rescue Variants™.

Through stem cell technology-based drug rescue, our objective is to become a leading source of proprietary, small molecule drug candidates to the global pharmaceutical industry. We have designed our drug rescue model to leverage publicly available information and substantial prior investment by pharmaceutical companies and others in Drug Rescue Candidates. The key commercial objective of our drug rescue model is to generate revenue from license, development and commercialization arrangements involving Drug Rescue Variants. We anticipate that each validated lead Drug Rescue Variant will be suitable as a promising new drug development program, either internally or in collaboration with a strategic partner.

Our Drug Rescue Strategy

We believe the pre-existing public domain knowledge base supporting the therapeutic and commercial potential of our Drug Rescue Candidates will provide us with a valuable head start as we launch our drug rescue programs. Leveraging the substantial prior investments by global pharmaceutical companies and others in discovery, optimization and efficacy validation of Drug Rescue Candidates is an essential component of our drug rescue strategy.

Our current drug rescue emphasis is on Drug Rescue Candidates discontinued prior to FDA market approval due to unexpected cardiac safety concerns. By using our CardioSafe 3D assay platform to enhance our understanding of the cardiac liability profile of Drug Rescue Candidates, biological insight not previously available when the Drug Rescue Candidate was originally discovered and developed, we believe we can demonstrate in vitro proof-of-concept as to the efficacy and safety of Drug Rescue Variants earlier in development and with substantially less investment in discovery, efficacy optimization and development than was required of the pharmaceutical companies prior to their decision to terminate the Drug Rescue Candidates.

The key elements of our CardioSafe 3D drug rescue strategy are as follows:

- identify potential Drug Rescue Candidates with heart safety issues utilizing drug discovery and development information available in the public domain through open source, licensed databases, and published patents, as well as through our strategic relationships with our drug rescue and scientific advisors and consultants, including Synterys, Inc. and Cato Research Ltd., our preferred provider of contract medicinal chemistry and contract clinical development and regulatory services, respectively;

- leverage substantial prior research and development investments made by global pharmaceutical companies and others to analyze internally the therapeutic and commercial potential of Drug Rescue Candidates, as important criteria for selection of Drug Rescue Candidates and potential lead Drug Rescue Variants;

- use CardioSafe 3D to enhance our understanding of the cardiac liability profile of Drug Rescue Candidates, important and more comprehensive biological insights not available when the Drug Rescue Candidates were originally discovered and developed by pharmaceutical companies;

- leverage our internal knowledgebase about each Drug Rescue Candidate's specific chemistry to design and produce a portfolio of novel potential lead Drug Rescue Variants for each Drug Rescue Candidate;

- use CardioSafe 3D and pre-existing in vitro efficacy models to assess the efficacy and cardiac safety of potential Drug Rescue Variants and identify and validate a lead Drug Rescue Variant; and

- internally develop validated lead Drug Rescue Variants or out-license them to a global pharmaceutical company in revenue-generating agreements providing for the full development, market approval and commercial sale.

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We believe our exclusive focus on Drug Rescue Candidates with established therapeutic and commercial potential, and our ability to build on that valuable head start using our expertise in human biology, will help us to generate Drug Rescue Variants without incurring certain high costs and risks typically inherent in drug discovery and development. Although we plan to continue to identify Drug Rescue Candidates in the public domain, we may also seek to acquire rights to Drug Rescue Candidates not available to us in the public domain through in-licensing arrangements with third-parties.

Strategic Licensing of Drug Rescue Variants

We believe many pharmaceutical companies are experiencing, and will continue to experience, critical research and development productivity issues, as measured by their lack of, or very low number of, FDA-approved products each year during the past decade. For example, in 2013, the U.S. pharmaceutical industry invested over $51 billion in research and development and the Center for Drug Evaluation and Research (CDER) of the FDA approved a total of only 39 novel drugs, known as New Molecular Entities (NMEs). In 2013, CDER approved only 27 NMEs, thirteen of which NME approvals (48%) were received by only five pharmaceutical companies, including Bayer (two), GlaxoSmithKline (four), Johnson & Johnson (three), Roche (two) and Takeda (two). Despite remarkable levels of research and development investment by the global pharmaceutical industry as a whole, since 2003, the FDA has only approved an average of approximately 26 NMEs per year. In addition, we believe many pharmaceutical companies with established products that are no longer patent protected are also experiencing substantial market pressure from generic competition.

As a result of research and development productivity issues, diminishing product pipelines and generic competition, we believe there is and will continue to be a critical need among pharmaceutical companies to license or acquire the new, safer Drug Rescue Variants we are focused on developing, including companies that originally discovered, developed and ultimately discontinued the Drug Rescue Candidates we select for our drug rescue programs.

Once we achieve proof-of-concept (POC) in vitro as to the efficacy and safety of a lead Drug Rescue Variant, we intend to announce the results of our internal POC studies and, at that time, consider whether we will seek to license that Drug Rescue Variant to a pharmaceutical company, including the company that developed the Drug Rescue Candidate, or further develop it internally on our own. If we decide to license a lead Drug Rescue Variant to a pharmaceutical company, through a form of license arrangement we believe is generally accepted in the pharmaceutical industry, we anticipate that the pharmaceutical company will be responsible for all subsequent development, manufacturing, regulatory approval, marketing and sale of the Drug Rescue Variant and that we will receive licensing revenue through payments to us from the license upon signing the license agreement, achievement of development and regulatory milestones, and, if approved and marketed, upon commercial sales.

Regenerative Medicine and Drug Discovery

Although we believe the best and most valuable near term commercial application of our stem cell technology platform, Human Clinical Trials in a Test Tube, is for small molecule drug rescue, we also believe stem cell technology-based regenerative medicine has the potential to transform healthcare in the U.S. over the next decade by altering the fundamental mechanisms of disease. We are interested in exploring ways to leverage our stem cell technology platform for regenerative medicine purposes, with emphasis on developing novel human disease models for discovery of small molecule drugs with regenerative and therapeutic potential. Our regenerative medicine focus will be based on 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, liver and insulin-producing pancreatic beta-islet cells, and our expertise in formulating customized biological assays with the cells we produce. Among our key objectives will be to explore regenerative medicine opportunities through pilot nonclinical proof-of-concept studies, after which we intend to assess any potential opportunities for further

development and commercialization of therapeutically and commercially promising regenerative medicine programs and novel, customized, disease-specific biological assays, either on our own or with strategic partners.

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AV-101 for Neuropathic Pain, Epilepsy and Depression

With $8.8 million of grant funding awarded from the U.S. National Institutes of Health, we have successfully completed Phase 1 development of AV-101. AV-101, also known as "L-4-chlorokynurenine" and "4-Cl-KYN", is an orally-available, non-sedating, small molecule prodrug candidate aimed at the multi-billion dollar neurological disease and disorders market, including neuropathic pain, a serious and chronic condition causing pain after an injury or disease of the peripheral or central nervous system, epilepsy, depression and Parkinson's disease. Our AV-101 IND application, on file with the FDA, covers clinical development for neuropathic pain. However, we believe the Phase 1 AV-101 safety studies completed to date will support development of AV-101 for multiple indications, including epilepsy, depression and Parkinson's disease. We intend to seek potential opportunities for further clinical development and commercialization of AV-101 for neuropathic pain, epilepsy, depression and Parkinson's disease, on our own or with strategic partners. In the event that we successfully complete one or more strategic partnering arrangements for AV-101, we plan to use the net proceeds from such an arrangement(s) to expand our stem cell technology-based drug rescue and regenerative medicine programs.

Scientific Background

Stem Cell Basics

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 (hPSCs) can be differentiated into all of the more than 200 types of cells in the human body, 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 biological 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 these mature cells as the basis for formulating our novel, customized bioassay systems to test the safety and efficacy of new drug candidates in vitro. These cells also have potential for diverse regenerative medicine applications.

Human Embryonic Stem Cells

Human embryonic stem cells 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 embryonic stem cells have the greatest and most documented 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.

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Induced Pluripotent Stem Celli

It is also possible to obtain hPSC lines from individuals without the use of embryos. Induced pluripotent stem cells 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.

Although there are remaining questions in the field about the lifespan, clinical utility and safety of iPSCs, we believe the biology and differentiation capabilities of hESCs and iPSCs are likely to be comparable for drug rescue 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 and formulate novel, customized biological assays to model specific diseases and genetic conditions for drug discovery and drug rescue purposes.

Proprietary Stem Cell Differentiation Protocols

Over fifteen years of research, together with Dr. Gordon Keller, our co-founder and Chairman of our Scientific Advisory Board, we have developed proprietary differentiation protocols covering key conditions involved in the differentiation of hPSCs into multiple types of mature human cells. The human cells generated by following these proprietary differentiation protocols are integral to our Human Clinical Trials in a Test Tube platform. We believe they support more clinically-predictive in vitro bioassay systems than animal testing or cellular assays currently used in drug discovery and development. Our strategic technology licenses from National Jewish Health in Denver, the Icahn School of Medicine at Mount Sinai in New York and the University Health Network in Toronto (UHN) relate to proprietary stem cell differentiation protocols developed by Dr. Keller and involve precisely-coordinated temporal and quantitative conditions and interaction of biological molecules, including:

- specific growth and differentiation factors used in the tissue culture medium, applied in specific combinations, at critical concentrations, and at critical times unique to each desired human cell type;

- the experimentally controlled regulation of developmental genes, which is critical for determining what differentiation path a human cell will take; and

- biological markers characteristic of precursor cells, which are committed to becoming specific human cells and tissues, and which can be used to identify, enrich and purify the desired mature human cell type.

We believe our Human Clinical Trials in a Test Tube platform will allow us to assess the toxicity profile of Drug Rescue Variants and other new drug candidates for a wide range of diseases and conditions with greater speed and precision than nonclinical surrogate safety models most often currently used in drug development.

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Growth Factors that Direct and Stimulate the Differentiation Process

The proprietary and licensed technologies underlying our Human Clinical Trials in a Test Tube platform allow us to direct and stimulate the differentiation process of hPSCs. As an example, for hESCs, the epiblast is the first stage in differentiation. One biological factor that controls the first fate decision of the epiblast is the relative concentrations of serum growth factors and activin, a protein involved in early differentiation and many cell fate decisions. Substituting explicit amounts of defined growth factors in place of ill-defined animal serum, and adding the optimal amount of activin is an important step in inducing the reproducible development of functional cells and, in our view, is essential for the development of a robust, efficient, and reproducible model of human cellular differentiation suitable for drug rescue. The use of activin in these applications is core to many of the claims in the patent applications underlying our licensed hPSC technology. Replacing activin with continuous exposure to ill-defined and variable animal serum results in an inefficient and variable differentiation of the human heart, liver, blood and cells of other organs. See "Intellectual Property – Icahn School of Medicine at Mount Sinai Exclusive Licenses."

In addition to activin, Dr. Keller's studies have identified a number of other growth and developmental factors that play important roles in the differentiation of hESCs. Some of the patents and patent applications underlying our licensed hPSC technology are directed to the use of a variety of specific growth factors that increase the efficiency (yield) and reproducibility of the hPSC differentiation process. We have exclusive rights to certain patents and patent applications with claims relating to growth factor concentrations for hESC differentiation that we believe are core and essential for drug rescue and development. See "Intellectual Property – Icahn School of Medicine at Mount Sinai Exclusive Licenses" and "National Jewish Health Exclusive Licenses."

Developmental Genes That Direct and Stimulate the Stem Cell Differentiation Process

For the purpose of creating our Human Clinical Trials in a Test Tube platform, we further control the differentiation process by controlling regulation of key developmental genes. By studying natural organ and tissue development, researchers have identified many genes that are critical to the normal differentiation, growth and functioning of tissues of the body. We engineer hESCs in a way that enables us to regulate genes that have been identified as critical to control and direct the normal development of specific types of cells. We can then mimic human biology in a way that allows us to turn on and off the expression of a selected gene by the addition of a specific compound to a culture medium. By adding specific compounds, we have the ability to influence the expression of key genes that are critically important to the normal biology of the cell.

Cell Purification Approaches

The proprietary protocols we have licensed and developed for our Human Clinical Trials in a Test Tube platform also establish specific marker genes and proteins which can be used to identify, enrich, purify, and study important populations of intermediate precursor cells that have made specific fate decisions and are on a specific developmental pathway towards a certain type of functionally mature cell. These proprietary protocols enable a significant increase in the efficiency, reproducibility, and purity of final cell populations. For example, we are able to isolate millions of purified specific precursor cells which, together with a specific combination of growth factors, develop full culture wells of functional, beating human cardiomyocytes. Due to their functionality and purity, we believe these cell cultures are ideal for drug rescue.

3D "Micro-Organ" Culture Systems

In addition to standard two-dimensional (2D) cultures which work well for some cell types and cellular assays, the proprietary hPSC technologies underlying our Human Clinical Trials in a Test Tube platform enable us to grow large numbers of normal, non-transformed, human cells to produce novel in vitro 3D "micro-organ" culture systems. For

example, for CardioSafe 3D, we grow large numbers of normal, non-transformed, mature human heart cells in vitro in 3D micro-organ culture systems. The 3D micro-organ cultures induce the cells to grow, mature, and develop 3D cell networks and tissue structures. We believe these 3D cell networks and structures more accurately reflect the structures and biology inside the human body than traditional flat, 2D, single cell layers grown on plastic, that are widely used by pharmaceutical companies today. We believe that the more representative human biology afforded by the 3D system will yield responses to drug candidates that are more predictive of human drug responses.

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Medicinal Chemistry

Medicinal chemistry involves designing, synthesizing, or modifying a small molecule compound or drug suitable for clinical development. It is a highly interdisciplinary science combining organic chemistry, biochemistry, physical chemistry, computational chemistry, pharmacology, and statistics. The combination of medicinal chemistry with the proprietary and licensed hPSC technologies underlying our Human Clinical Trials in a Test Tube platform are core components of our drug rescue business model. Working with our strategic contract medicinal chemistry partner, Synterys, Inc., we are focused on using our stem cell biology to generate a pipeline of effective and safe Drug Rescue Variants of once-promising company drug candidates in a more efficient and cost-effective manner than the processes currently used for drug development.

CardioSafe 3D

The limitations of current preclinical drug testing systems used by pharmaceutical companies contribute to the high failure rate of drug candidates. Unexpected cardiotoxicity is one of the top two major safety-related reasons for failure of both drugs and drug candidates. Incorporating human pluripotent stem cell-derived cardiomyocyte (hPSC-CM) assays early in preclinical development offers the potential to improve clinical predictability, decrease rescue and development costs, and avoid adverse patient effects, late-stage clinical termination, and product recall from the market.

With our proprietary human pluripotent stem cell technology, we can generate fully-functional hPSC-CMs at a high level of purity (>95%), without genetic modification or antibiotic selection. This is important because genetic modification and antibiotic selection can distort the ratios of cardiac cell types and have a direct impact on the ultimate results and clinical predictivity of the assay. In addition to expressing all of the key ion channels of the human heart (calcium, potassium and sodium) and various cardiomyocytic markers of the human heart, our hPSC-CMs function reliably in all cardiac toxicity assays relevant to cardiac drug effects developed and tested to date.

Utilizing fully functional hPSC-CMs that underlie our Human Clinical Trials in a Test Tube platform, we have validated our CardioSafe 3D assay system to screen for both cardiomyopathy (or 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 Drug Rescue Variants and other new drug candidates than is possible with existing preclinical testing systems.

We have developed and validated two functional components of our CardioSafe 3D screening system to assess multiple different categories of cardiac toxicities. The first consists of a suite of five fluorescence or luminescence based high-throughput hPSC-CM assays. These five CardioSafe 3D assays measure drug-induced cardiomyopathy, including the following:

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 of 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. -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

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This suite of five CardioSafe 3D 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 our CardioSafe 3D assays provide valuable and more comprehensive bio-analytical tools for both assessing the effects of pharmaceutical compounds on cardiac cytotoxicity and for elucidating the specific mechanisms of cardiac toxicity, thereby laying a solid foundation for our drug rescue 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. We have validated (correlated with reported clinical results) this key component of our CardioSafe 3D assay system with twelve drugs, each with known toxic or non-toxic cardiac effects in humans. These twelve validation compounds are as follows:

1. One FDA-approved drug (aspirin) without cardiac liability to serve as a negative control;
2. Five FDA-approved drugs (astemizole, sotalol, cisapride, terfenadine and sertindole) that were withdrawn from the market due to heart toxicity concerns;
3. Five FDA-approved drugs (fexofenadine, nifedipine, verapamil, lidocaine and propranolol) that have certain measurable clinical non-toxic cardiac effects. Note: fexofenadine is a non-cardiotoxic drug variant of terfenadine; and
4. One research compound (E-4031) failed in Phase I human clinical study before being discontinued due to heart toxicity concerns.

We have validated that our CardioSafe 3D MEA assay was reproducible and consistent with the known human cardiac effects of all the twelve 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 cardiac effects of terfenadine (SeldaneTM), withdrawn by the FDA due to cardiotoxicity, and the cardiac effects of the close structurally related fexofenadine (AllegraTM), the non-cardiotoxic chemical variant of terfenadine, which remains on the market. Our validation data suggest that our CardioSafe 3D assay system provides valuable and more comprehensive bio-analytical tools for preclinical cardiac safety screening of drug candidates, which we believe will contribute to the efficient identification of novel, safer Drug Rescue Variants in our drug rescue programs.

To further evaluate the potential of our CardioSafe 3D assay system to predict cardiac toxicity of drug candidates, including Drug Rescue Variants, we have assessed cardiac effects induced by small molecule kinase inhibitors (KIs), which belong to a new category of drugs that have revolutionized cancer therapy due to decreased systemic toxicity and an increased tumor cell specific effect compared to classic cancer drugs. Since 1998, the FDA has approved approximately thirty small molecule KIs for cancer therapy. However, many KIs have been implicated in causing serious adverse cardiac events in patients which were not identified during preclinical drug development.

In our CardioSafe 3D validation studies, CardioSafe 3D detected cardiac toxicities of well-known anti-cancer KIs, all of which were cardiac toxicities not previously identified during the pre-FDA approval development process for each compound studied. This important validation set of compounds is as follows:

1. Inhibitors to growth factor receptors: sunitinib, axitinib, imatinib, dasatinib, sorafenib, erlotinib, Lapatinib, tyrphostin and AG1478;
2. Inhibitors to the mTOR pathway: everolimus, temsirolimus;
3. Inhibitors to cell cycle regulators: tozasertib, barasertib, alvocidib;
4. Inhibitors to the PI3K pathway : perifosine, LY294002, XL765;
5. Inhibitors to the MEK pathway: PD325901, AZD6264; and
6. Inhibitors to the JAK and other pathways: lestaurtinib.

Our validation data indicate that CardioSafe 3D successfully detected cardiotoxicity induced by all of the representative compounds, concordant with now-reported adverse cardiac events from each of the different KI categories. Our CardioSafe 3D assay system is able to distinguish between cardiotoxic and safe compounds, and even between those which inhibit the same kinase pathways. For instance, both sunitinib and axitinib are the inhibitors to VEGFR, PDGFR and c-Kit pathways, and our CardioSafe 3D assays indicate that sunitinib is cardiotoxic and axitinib is safe, outcomes which are consistent with the reported clinical outcomes.

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Furthermore, the cardiotoxicity profile of each KI studied provided clues as to the potential mechanism(s) causing the cardiac cytotoxicity of each compound. For example, cardiac cytotoxicity induced by perifosine showed apoptotic responses at lower concentrations, while imatinib was most active in the oxidative stress assays. In addition, no cardiac toxicity or alteration in electrophysiology was detected with drugs that do not have a cardiac liability, emphasizing the specificity of our CardioSafe 3D assay system. Having information on the pathways associated with the toxic effects of compounds provides important clues for novel medicinal chemistry approaches and compound modifications for our CardioSafe 3D drug rescue programs.

Our CardioSafe 3D assay system enables the sensitive measurement of drug effects with results that are consistent with reported clinical responses to the compounds. For example, our data indicated that sunitinib and dasatinib caused QT prolongation, arrhythmia, and/or altered contraction rates in hPSC-CMs, which are consistent with clinical observations.

We believe our CardioSafe 3D validation data demonstrate that CardioSafe 3D will improve clinical predictivity as an in vitro preclinical cardiac safety assay, helping not only to identify potential cardiac toxicities, but also to discover important potential mechanisms of cardiotoxicity. We believe the results of our CardioSafe 3D validation studies indicate that CardioSafe 3D may be effectively used to identify novel, Drug Rescue Variants, with reduced heart toxicity. By providing more accurate, comprehensive and timely indications of alterations in electrophysiological activity as well as direct heart toxicity of drug candidates than animal models or cellular assay systems currently used by pharmaceutical companies, we believe the results of our CardioSafe 3D validation studies support the central premise of our drug rescue business model: by using our hPSC-derived human heart and liver cell bioassay systems at the front end of the drug development process, we have the opportunity to leverage substantial prior investment by pharmaceutical companies and others in drug discovery and efficacy optimization of once-promising drug candidates that have been terminated prior to FDA approval due to unexpected heart or liver toxicity concerns.

LiverSafe 3D

LiverSafe 3D is a powerful new in vitro hepatotoxicity assay system that goes a step beyond the current commercially available gold standard primary (human cadaver) hepatocyte assays. By combining the flexibility of an in vitro, non-transformed human cell-based assay system with the renewable, reproducible sourcing of human pluripotent stem cells (hPSCs), the functional hPSC-derived hepatocytes we produce for LiverSafe 3D can be maintained in a healthy state for much longer than the current gold standard hepatocyte assays, greatly enhancing the reliability of hepatotoxicity testing for our drug rescue programs.

Until now, reliable human cell-based hepatotoxicity screening platforms have been difficult to establish for high throughput drug development with currently available primary hepatocyte systems. Primary hepatocytes have a short lifespan in culture, during which time they rapidly lose their drug metabolizing capabilities and develop signs of cellular stress. Furthermore, these commercially available primary hepatocytes have significant batch-to-batch genetic variation that alters the function of drug metabolism genes and their critical enzyme activity levels due to the use of hepatocytes from different sources. Additionally, primary hepatocytes are derived from individuals with significant differences in health status, with unknown effects on hepatocyte function. Consequently, it is difficult to maintain quantitative reproducibility using currently available primary hepatocyte assays, and this leads to limitations in the quality and clinical predictiviy of the results and conclusions drawn from these assays.

The foregoing limitations have led many in the field to believe that hPSC-derived hepatocyte assays offer a better alternative to the current gold standard primary hepatocyte assays. This belief is mainly due to the fact that hepatocytes derived from the same hPSC line are genetically identical, normal, non-transformed (that is, not tumor-derived) human cells derived from hPSCs. Importantly, hPSC-derived hepatocytes can be indefinitely propagated and frozen down into large, uniform, quality-controlled cell banks. The challenge to using hPSC-derived

hepatocytes has been differentiating the stem cells into mature hepatocytes that express a full complement of functional drug metabolizing enzymes, nuclear receptors, and transporters at least as well as primary hepatocytes. While many groups have taken on this challenge in recent years, published reports indicate that current hPSC differentiation protocols yield immature hepatocytes, especially with respect to extremely low expression of certain key drug metabolizing enzymes, such as CYP3A4. CYP3A4 is a critical liver enzyme responsible for metabolizing approximately 50% of the FDA-approved drugs currently available on the market. It is an important and well-accepted functional gene found almost exclusively in mature, adult hepatocytes. CYP3A4 is the key functional marker that we have used to optimize our hepatocyte differentiation cultures for LiverSafe 3D. We believe our optimized LiverSafe 3D assay system enables us to generate more mature hPSC-derived hepatocytes than are currently available from others in the field and that our LiverSafe 3D system provides the unique ability to specifically select for mature CYP3A4-expressing human hepatocytes.

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We developed LiverSafe 3D using hPSC differentiation protocols adapted from the laboratory of our co-founder, Dr. Gordon Keller, and our proprietary hPSC cell line, 3A4BLA. This 3A4BLA cell line is a human embryonic stem cell (hESC) line that contains a humanized BLA functional "reporter" that targets the CYP3A4 gene in a manner resulting in the expression of BLA only in cells that also express CYP3A4. This allows us to visualize by fluorescence cells that express CYP3A4 based on expression of the BLA reporter. By producing a cell line capable of tracking CYP3A4 expression, we have been able to optimize our hPSC differentiation protocols to increase expression of mature hepatocyte markers and drug metabolizing enzymes and to enrich for CYP3A4-expressing cells by cell sorting. However, even in the absence of cell sorting, our LiverSafe 3D hepatocyte populations contain greater than 80% ALBUMIN-positive cells and greater than 40% CYP3A4-positive cells, with CYP3A4 mRNA expression reaching levels nearly 60-fold higher than side-by-side 38-week human fetal liver controls. Our LiverSafe 3D hepatocytes secrete urea and ALBUMIN at levels that exceed commercially-available primary hepatocytes, and they also store both glycogen and lipids, characteristics that are required of functional, mature adult hepatocytes. Importantly, expression of fetal liver markers decreases over the time course of differentiation of our LiverSafe 3D hepatocytes. This decreased expression is expected and essential during maturation of hepatocytes, but it has rarely been reported by others in publications describing their hPSC-derived hepatocytes. With the addition of cell sorting, our LiverSafe 3D hepatocyte populations can be highly enriched for CYP3A4-BLA-positive cells, with CYP3A4 message in the positive cell population reaching greater than 30% that of an adult human liver pool control. To our knowledge, this level of CYP3A4 expression exceeds levels reported by others in the literature.

The most important capabilities of LiverSafe 3D relate to "Phase I" and "Phase II" drug metabolism, which are functional characteristics of mature adult hepatocytes. We have validated these capabilities of LiverSafe 3D by demonstrating its ability to metabolize known substrates, such as testosterone, and its ability to respond properly to known inducers of Phase I-mediated CYP3A4 metabolism, such as rifampicin. Moreover, our LiverSafe 3D hepatocytes demonstrate Phase II-mediated testosterone metabolism levels that exceed commercially available primary hepatocytes. These functional characteristics of mature adult hepatocytes are critical to the development of a reliable and clinically predictive hepatotoxicity screening platform for our drug rescue programs. We are currently focused on expanding our panel of validation assays and compounds to include more P450 substrates, inducers, and inhibitors, as well as adapting the cellular toxicity assays that have been developed for our CardioSafe 3D assay system to our LiverSafe 3D assay system and to apply specific hepatotoxic screening assays, such as ALBUMIN and urea secretion assays.

We believe LiverSafe 3D is a powerful, genetically identical, renewable, and reproducible hepatotoxicity assay system for drug rescue and development that provides great advantages over currently available primary hepatocyte assays. We have demonstrated that our LiverSafe 3D hepatocyte populations, even in the absence of cell sorting, secrete adult hepatocyte levels of ALBUMIN and urea and contain greater than 40% CYP3A4-positive cells, historically difficult to achieve in hPSC differentiation cultures. The proprietary 3A4BLA cell line component of LiverSafe 3D allows us the unique opportunity to enrich CYP3A4-positive cells, resulting in CYP3A4 expression reaching greater than 30% of an adult human liver pool, and to the best of our knowledge, a level higher than described in current literature. Most importantly for drug rescue and development purposes, our hPSC-derived hepatocytes for LiverSafe 3D metabolize known substrates and respond to known inducers in a manner expected only of mature adult hepatocytes, paving the way for our final validation of LiverSafe 3D system as a novel hepatotoxicity assay system that can improve clinical predictivity, decrease the cost of drug rescue and development, reduce use of live animal studies, and improve drug safety.

AV-101

We have successfully completed Phase I development of AV-101, also known as "L-4-chlorokynurenine" or "4-Cl-KYN". AV-101 is a prodrug candidate for the treatment of neuropathic pain, epilepsy and depression. Our AV-101 IND application, on file with the FDA, covers our Phase I clinical development for neuropathic pain. However, we believe the safety studies done in Phase I development of AV-101 will support development of AV-101 for other indications,

including epilepsy, depression and potentially other neurological diseases, such as Parkinson's disease.

The NIH awarded us $8.8 million of grant funding for our preclinical and Phase 1 clinical development of AV-101. During 2014, we plan to seek strategic partnering arrangements for further development and commercialization of AV-101 for neuropathic pain, epilepsy, depression and potentially neurodegenerative diseases related to aging.

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AV-101 is an orally-available, non-sedating, pro-drug that is converted in the brain into an active metabolite, 7-chlorokynurenic acid (7-Cl-KYNA), which regulates the N-methyl-D-aspartate (NMDA) receptors. 7-Cl-KYNA is a synthetic analogue of kynurenic acid, a naturally occurring neural regulatory compound, and is one of the most potent and selective blockers of the regulatory GlyB-site of the NMDA receptor. In preclinical studies, AV-101 has very good oral bioavailability, is rapidly and efficiently transported across the blood-brain barrier, and is converted into 7-Cl-KYNA in the brain and spinal cord, preferentially, at the site of seizures and potential neural damage.

The effect of AV-101 on chronic neuropathic pain due to inflammation and nerve damage was assessed in rats by using the Chung nerve ligation model. AV-101 effects were compared to either saline and MK-801, or gabapentin (NeurontinTM) as positive controls. Similar to the therapeutic effects seen in the acute formalin and thermal pain models, AV-101 had a positive effect on chronic neuropathic pain in the Chung model that were greater than two standard deviations of the control, with no adverse behavioral observations. As expected, MK-801 and gabapentin also demonstrated reduced pain readouts in the Chung model. The effects observed by AV-101 in both the acute and chronic neuropathic pain model systems was dose dependent, and was not associated with any side effects at the range of doses administered. Preclinical AV-101 data demonstrated the potential clinical utility of AV-101 as an analgesic.

Strategic Transactions and Relationships

Strategic collaborations are a cornerstone of our corporate development strategy. We believe that our strategic outsourcing and sponsorship of application-focused research gives us flexible access to medicinal chemistry, hPSC research and development, manufacturing, clinical development and regulatory expertise at a lower overall cost than developing and maintaining such expertise internally. In particular, we collaborate with the types of third parties identified below for the following functions:

- academic research institutions, such as Duke University and UHN, for hPSC technology research and development;

- contract medicinal chemistry companies, such as Synterys, Inc., to analyze Drug Rescue Candidates and design, produce and analyze Drug Rescue Variants; and

- contract clinical development and regulatory organizations (CROs), such as Cato Research, Ltd., for regulatory expertise and clinical development support.

McEwen Centre for Regenerative Medicine, University Health Network

The University Health Network (UHN) in Ontario, Canada is a major landmark in Canada's healthcare system. UHN is one of the world's largest research hospitals, with major research in transplantation, cardiology, neurosciences, oncology, surgical innovation, infectious diseases and genomic medicine. Providing care to the community for more than two centuries, UHN brings together the talent and resources needed to achieve global impact and provide exemplary patient care, research and education.

The McEwen Centre for Regenerative Medicine (McEwen Centre) is a world-renowned center for stem cell biology and regenerative medicine and a world-class stem cell research facility affiliated with UHN. Dr. Gordon Keller, our co-founder and Chairman of our Scientific Advisory Board, is Director of the McEwen Centre. Dr. Keller's lab is one of the world leaders in successfully applying principles from the study of developmental biology of many animal systems to the differentiation of pluripotent stem cell systems, resulting in reproducible, high-yield production of human heart, liver, blood and vascular cells. The results and procedures developed in Dr. Keller's lab are often quoted and used by academic scientists worldwide.

In September 2007, we entered into a long-term sponsored stem cell research and development collaboration with UHN. In December 2010, we extended the collaboration to September 2017. The primary goal of this ten-year collaboration is to leverage the stem cell research, technology and expertise of Dr. Gordon Keller to develop and commercialize industry-leading human pluripotent stem cell differentiation technology and bioassay systems for drug rescue and development and regenerative cell therapy applications. This sponsored research collaboration builds on our existing strategic licenses from National Jewish Health and the Icahn School of Medicine at Mount Sinai to certain pluripotent stem cell technologies developed by Dr. Keller, and is directed to diverse human pluripotent stem cell-based research projects, including, as expanded and amended, strategic projects related to drug rescue and regenerative medicine. See “Sponsored Research Collaborations and Intellectual Property Rights – University Health Network, McEwen Centre for Regenerative Medicine, Toronto, Ontario”, “Intellectual Property – National Jewish Health Exclusive Licenses” and “Intellectual Property – Icahn School of Medicine at Mount Sinai Exclusive Licenses.”

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Cardiac Safety Research Consortium

We have joined the Cardiac Safety Research Consortium (CERC) 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.

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 (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.

In December 2012, we formalized our membership in the CCRM's Industry Consortium. Other members of CCRM's Industry Consortium include such leading global companies as Pfizer, GE Healthcare and Lonza. 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 for Regenerative Medicine offers a solid foundation and unique opportunities for expanding the commercial applications of our Human Clinical Trials in a Test Tube 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.

Duke University

In November 2011, we entered into a strategic collaboration with Duke University, one of the premier academic research institutions in the U.S., aimed at combining our complementary expertise in cardiac stem cell technology, electrophysiology and tissue engineering. The initial goal of the collaboration is to explore the potential development

of novel, engineered, stem cell-derived cardiac tissues to expand the scope of our drug rescue capabilities focused on heart toxicity. We expect that this collaboration, employing our human stem cell-derived heart cells combined with Duke's technology relating to cardiac electrophysiology and cardiac tissue engineering, will permit us to use micro-patterned cardiac tissue to expand the approaches available to us in our drug rescue programs to quantify drug effects on functional human cardiac tissue.

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In May 2013, we announced that our scientists together with researchers at Duke University combined our human stem cell-derived heart cells with Duke's innovative tissue engineering and cardiac electrophysiology technologies to grow what is being called a "heart patch," which mimics the natural functions of native human heart tissue. We believe this is the closest man-made approximation of natural human heart muscle to date. This heart patch technology is being developed to aid in a better understanding of the biology critical to cardiac tissue engineering, for applications in regenerative cell therapy for heart disease, and as predictive in vitro assays for drug rescue and development. We believe the developed contractile forces and other functional properties of these cardiac tissues are remarkable and are significantly higher than any previous reports. The achievement of successfully growing a human heart muscle from cardiomyocytes derived from human pluripotent stem cells expands the scope of our drug rescue capabilities and reflects the advanced nature and potential of our collaboration with Duke University.

Achieving this capability represents a potentially significant breakthrough in heart cell-based therapies and in testing new medicines for potential heart toxicity and potential therapeutic benefits impacting heart disease.

The following are among several key development points from the study:

- The optimized 3D environment of a cardiac tissue patch yields advanced levels of structural and functional maturation of human cardiomyocytes that produce expected responses to drugs;

- Human cardiomyocyte maturation in an optimized 3D patch environment is enhanced relative to that found in industry standard 2D cultures;

- No genetic modifications were used to produce, purify, or mature cardiomyocytes, suggesting potential for future therapeutic applications;

- Cardiac tissue patches generated using VistaGen's cardiomyocytes exhibited 2.2-180 fold higher contractile force generation compared to previous studies;

- Based on a force per cardiomyocyte metric, cardiac tissue engineering methodology that used VistaGen's cardiomyocytes exhibited 4-700-fold higher efficiency than previously reported; and

- Cardiac tissue patches generated using VistaGen's cardiomyocytes exhibited velocities of electrical signal propagation 5-fold higher compared to previous reports in human engineered cardiac tissues.

Cato Research and Cato BioVentures

Cato Research

Cato Research is a contract research and development organization (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 has in-house capabilities to assist its sponsors with aspects of the drug development process including regulatory strategy, nonclinical and toxicology development, clinical development, data processing, data management, statistical analysis, regulatory applications, including INDs and NDAs, chemistry, manufacturing, and control programs, cGCP, cGLP and cGMP audit and compliance activities, and due diligence review of emerging technologies. Cato Research's senior management team, including co-founders Allen Cato, M.D., Ph.D. and Lynda Sutton, has over 25 years of experience interacting with the FDA and international regulatory agencies and a successful track record of product approvals. Should we elect to advance development of Drug Rescue Variants internally rather than license or sell them at an early-stage to pharmaceutical companies or others, we believe our long term strategic relationship with

Cato Research provides us with real time access to the global connections, insight and knowledge necessary to effectively plan, execute and manage successful nonclinical and clinical development programs throughout the world without incurring the substantial expenses typically associated with establishing and maintaining a wide range of drug development capabilities in-house.

Cato BioVentures

Cato Holding Company, doing business as Cato BioVentures (Cato BioVentures), is the venture capital affiliate of Cato Research. Through strategic CRO service agreements with Cato Research, Cato BioVentures invests in therapeutics and medical devices, as well as platform technologies such as our stem cell technology-based Human Clinical Trials in a Test Tube platform, which its principals believe, based on their experience as management of Cato Research, are capable of transforming the traditional drug development process and the research and development productivity of the biotechnology and pharmaceutical industries.

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Our Relationship with Cato Research and Cato BioVentures

Cato Research is our primary CRO for development of AV-101. Cato BioVentures is among our largest, long-term institutional investors.

As a result of the access Cato Research has to potential Drug Rescue Candidates from its biotechnology and pharmaceutical industry network, as well as Cato BioVentures' strategic long term equity interest in VistaGen, we believe that our relationships with Cato BioVentures and Cato Research may provide us with unique opportunities relating to our drug rescue efforts that will permit us to leverage both their industry connections and the CRO resources of Cato Research, either on a contract research basis or in exchange for economic participation rights, should we develop Drug Rescue Variants internally on our own rather than out-license them to strategic partners.

United States National Institutes of Health

Since our inception in 1998, the U.S. National Institutes of Health (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 Human Clinical Trials in a Test Tube platform and $8.8 million for nonclinical and Phase 1 clinical development of AV-101, our small molecule drug candidate which has successfully completed Phase 1 clinical development in the U.S. for neuropathic pain and other potential diseases and conditions, including epilepsy and depression.

California Institute for Regenerative Medicine

The California Institute for Regenerative Medicine (CIRM) funds stem cell research at academic research institutions and companies throughout California. CIRM was established in 2004 with the passage of Stem Cell Initiative (Proposition 71) by California voters. As a stem cell company based in California since 1998, we are eligible to apply for and receive grant funding under the Stem Cell Initiative. To date we have been awarded approximately $1.0 million of non-dilutive grant funding from CIRM for stem cell research and development related to stem cell-derived human liver cells. This funded research and development focused on the improvement of techniques and the production of engineered human ES Cell lines used to develop mature functional human liver cells as a biological system for testing drugs.

Celsis In Vitro Technologies

In March 2013, we entered into a strategic collaboration with Celsis In Vitro Technologies (Celsis IVT), a premier global provider of specialized in vitro products for drug metabolism, drug-drug interaction and toxicity screening, focused on characterizing and functionally benchmarking our human liver cell platform, LiverSafe 3D™ with Celsis IVT products for studying and predicting drug metabolism. We intend to utilize Celsis IVT's experience and expertise in in vitro drug metabolism to help validate LiverSafe 3DTM. We anticipate that Celsis IVT will not only validate our human liver cells in traditional pharmaceutical metabolism assays, but also will determine genetic variations in our human pluripotent stem cell lines that are important to drug development. In addition, we plan to utilize Celsis IVT's large inventory of cryopreserved primary human liver cells, currently used throughout the pharmaceutical industry for traditional and high-throughput liver toxicology and other bioassays, as reference controls with which to monitor and benchmark the functional properties of LiverSafe 3D.

Collaborating with Celsis IVT scientists, we are focused on the following four key objectives:

- Optimize techniques to handle and maintain primary human cryopreserved primary liver cells as reference controls for various drug development assays;

- Develop a stable supply of characterized and validated human cryopreserved primary liver cells to serve as internal controls and provide benchmark comparisons for the characterization of our pluripotent stem cell-derived liver cells;

- Characterize our human pluripotent stem cell-derived liver cells using many of the same industry-standardized assays used to characterize primary human liver cells; and

- Produce a joint publication of the characterization of our pluripotent stem cell-derived human liver cells.

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As an industry leader in the development of in vitro primary hepatocyte technology, we believe Celsis IVT has extensive resources to aid us in the benchmarking LiverSafe 3D to industry standards. We anticipate this collaboration will lead to the further validation of LiverSafe 3D for predicting liver toxicity and drug metabolism issues before costly human clinical trials.

Synterys, Inc.

In December 2011, we entered into a strategic medicinal chemistry collaboration agreement with Synterys, Inc. (Synterys), a leading medicinal chemistry and collaborative drug discovery company. We believe this important collaboration will further our drug rescue initiatives with the support of Synterys' medicinal chemistry expertise. In addition to providing flexible, real-time contract medicinal chemistry services in support of our drug rescue programs, we anticipate potential collaborative opportunities with Synterys wherein we may jointly identify and develop Drug Rescue Variants.

Intellectual Property

Intellectual Property Rights Underlying our Human Clinical Trials in a Test Tube Platform

We have established our intellectual property rights to the technology underlying our Human Clinical Trials in a Test Tube platform through a combination of exclusive and non-exclusive licenses, patent, and trade secret laws. To our knowledge, we are the first stem cell company focused primarily on stem cell technology-based drug rescue. We have assembled an intellectual property portfolio around the use of pluripotent stem cell technologies in drug discovery and development and with specific application to drug rescue. The differentiation protocols we have licensed direct the differentiation of pluripotent stem cells through:

- a combination of growth factors (molecules that stimulate the growth of cells);
- the experimentally controlled regulation of developmental genes, which is critical for determining what differentiation path a human cell will take; and
- precise selection of immature cell populations for further growth and development.

By influencing key branch points in the cellular differentiation process, our pluripotent stem cell technologies can produce fully-differentiated, non-transformed, highly functional human cells in vitro in an efficient, highly pure and reproducible process.

As of the date of this report, we either own or have licensed 43 issued U.S. patents and 12 U.S. patent applications and certain foreign counterparts relating to the stem cell technologies that underlie our Human Clinical Trials in a Test Tube platform. Our material rights and obligations with respect to these patents and patent applications are summarized below:

Licenses

National Jewish Health (NJH) Exclusive License

We have exclusive licenses to seven issued U.S. patents held by NJH, certain of which expire in November 2014. No foreign counterparts to these U.S. patents and patent application have been obtained. These U.S. patents contain claims covering composition of matter relating to specific populations of cells and precursors, methods to produce

such cells, and applications of such cells for ES Cell-derived immature pluripotent precursors of all the cells of the mesoderm and endoderm lineages. Among other cell types, this covers cells of the heart, liver, pancreas, blood, connective tissues, vascular system, gut and lung cells.

Under this license agreement, we may become required to pay to NJH 1% of our total revenues up to $30 million in each calendar year and 0.5% of all revenues for amounts greater than $30 million, with minimum annual payments of $25,000. Additionally, we may become obligated under the agreement to make certain royalty payments on sales of products based on NJH's patents or the sublicensing of such technology. The royalty payments are subject to anti-stacking provisions which would reduce our payments by a percentage of any royalty payments and fees paid to third parties who have licensed necessary intellectual property to us. This agreement remains in force for the life of the patents so long as neither party elects to terminate the agreement upon the other party's uncured breach or default of an obligation under the agreement. We also have the right to terminate the agreement at any time without cause.

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Icahn School of Medicine at Mount Sinai School (MSSM) Exclusive License

We have an exclusive, field restricted, license to two U.S. patents and two U.S. patent applications, and their foreign counterparts filed by MSSM. Foreign counterparts have been filed in Australia (two), Canada (two), Europe (two), Japan, Hong Kong and Singapore. Two of the U.S. applications have been issued and the foreign counterparts in Australia and Singapore have been issued, while a counterpart in Europe is pending. These patent applications have claims covering composition of matter relating to specific populations of cells and precursors, methods to produce such cells, and applications of such cells, including:

- the use of certain growth factors to generate mesoderm (that is, the precursors capable of developing into cells of the heart, blood system, connective tissues, and vascular system) from hESCs;

- the use of certain growth factors to generate endoderm (that is, the precursors capable of developing into cells of the liver, pancreas, lungs, gut, intestines, thymus, thyroid gland, bladder, and parts of the auditory system) from hESCs; and

- applications of cells derived from mesoderm and endoderm precursors, especially those relating to drug discovery and testing for applications in the field of in vitro drug discovery and development applications.

This license agreement requires us to pay annual license and patent prosecution and maintenance fees and royalty payments based on product sales and services that are covered by the MSSM patent applications, as well as for any revenues received from sublicensing. Any drug candidates that we develop, including any Drug Rescue Variants, will only require royalty payments to the extent they require the practice of the licensed technology. To the extent we incur royalty payment obligations from other business activities, the royalty payments are subject to anti-stacking provisions which reduce our payments by a percentage of any royalty payments or fees paid to third parties who have licensed necessary intellectual property to us. The license agreement will remain in force for the life of the patents so long as neither party terminates the agreement for cause (i) due to a material breach or default in performance of any provision of the agreement that is not cured within 60 days or (ii) in the case of failure to pay amounts due within 30 days.

Wisconsin Alumni Research Foundation (WARF) Non-Exclusive License

We have non-exclusive licenses to over 30 issued stem cell-related U.S. patents, 14 stem cell-related U.S. patent applications, and certain foreign counterparts held by WARF, for applications in the field of in vitro drug discovery and development. Foreign counterparts have been filed in Australia, Canada, Europe, China, India, Hong Kong, Israel, Brazil, South Korea, India, Mexico, and New Zealand. The subject matter of these patents includes specific hESC lines and composition of matter and use claims relating to hESCs important to drug discovery, and drug rescue screening. We have rights to:

- use the technology for internal research and drug development;

- provide discovery and screening services to third parties; and

- market and sell research products (that is, cellular assays incorporating the licensed technology).

This license agreement requires us to make royalty payments based on product sales and services that incorporate the licensed technology. We do not believe that any drug rescue candidates to be developed by us will incorporate the licensed technology and, therefore, no royalty payments will be payable. Nevertheless, there is a minimum royalty of $20,000 per calendar year. There are also milestone fees related to the discovery of therapeutic molecules, though no royalties are owed on such molecules. The royalty payments are subject to anti-stacking provisions which reduce our payments by a percentage of any royalty payments paid to third parties who have licensed necessary intellectual property to us. The agreement remains in force for the life of the patents so long as we pay all monies due and do not breach any covenants, and such breach or default is uncured for 90 days. We may also terminate the agreement at any time upon 60 days' notice. There are no reach through royalties on customer-owned small molecule or biologic drug products developed using the licensed technologies.

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Our Patents

We have filed two U.S. patent applications on liver stem cells and their applications in drug development relating to toxicity testing, both of which have issued. Of the related international filings, European, Canadian and Korean patents were issued. The European patent has been validated in 11 European countries. We have filed a U.S. patent application, with foreign counterpart filing in Canada and Europe, directed to methods for producing human pluripotent stem cell-derived endocrine cells of the pancreas, with a specific focus on beta-islet cells, the cells that produce insulin, and their uses in diabetes drug discovery and screening.

The material patents currently related to the generation of human heart and liver cells for use in connection with our drug rescue activities are set forth below:

Territory	Patent No.	General Subject Matter	Expiration
US	7,763,466	Method to produce endoderm cells	May 2025
US	7,955,849	Method of enriching population of mesoderm cells	May 2023
US	8,143,009	Toxicity typing using liver stem cells	June 2023
US	8,512,957	Toxicity typing using liver stem cells	June 2021

With respect to AV-101, we have filed three new U.S. patent applications.

Trade Secrets

We rely, in part, on trade secrets for protection of some of our intellectual property. We attempt to protect trade secrets by entering into confidentiality agreements with third parties, employees and consultants. Our employees and consultants also sign agreements requiring that they assign to us their interests in patents and copyrights arising from their work for us.

Sponsored Research Collaborations and Intellectual Property Rights

University Health Network, McEwen Centre for Regenerative Medicine, Toronto, Ontario

We have a long-term strategic stem cell research collaboration with our co-founder, Dr. Gordon Keller, Director of the UHN's McEwen Centre, focused on, among other things, developing improved methods for differentiation of cardiomyocytes (heart cells) from pluripotent stem cells, and their uses in biological assay systems for drug discovery and development. Pursuant to our sponsored research collaboration agreement with UHN, we have the right to acquire exclusive worldwide rights to any inventions arising from studies we sponsor, under pre-negotiated license terms. Such pre-negotiated terms provide for royalty payments based on product sales that incorporate the licensed technology and milestone payments based on the achievement of certain events. Any Drug Rescue Variants that we develop will not incorporate the licensed technology and, therefore, will not require any royalty payments. To the extent we incur royalty payment obligations from other business activities, the royalty payments will be subject to anti-stacking provisions, which reduce our payments by a percentage of any royalty payments paid to third parties who have licensed necessary intellectual property to us. These licenses will remain in force for so long as we have an obligation to make royalty or milestone payments to UHN, but may be terminated earlier upon mutual consent, by us at any time, or by UHN for our breach of any material provision of the license agreement that is not cured within 90 days. We also have the exclusive option to sponsor research for similar cartilage, liver, pancreas and blood cell projects with similar licensing rights.

The sponsored research collaboration agreement with UHN, as amended, has a term of ten years, ending on September 18, 2017. Our 2012/2013 sponsored research project budget under the agreement ended on September 30, 2013. We are currently in discussions with Dr. Keller and UHN regarding the scope of our future sponsored research project budget under the agreement, and we anticipate finalizing such budget within the near term. The ten-year term of the agreement is subject to renewal upon mutual agreement of the parties. The agreement may be terminated earlier upon a material breach by either party that is not cured within 30 days. UHN may elect to terminate the agreement if we become insolvent or if any license granted pursuant to the agreement is prematurely terminated. We have the option to terminate the agreement if Dr. Keller stops conducting his research or ceases to work for UHN.

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UHN License for Stem Cell Culture Technology

In April 2012, we licensed breakthrough stem cell culture technology from UHN's McEwen Centre. We intend to utilize the licensed technology to develop hematopoietic precursor stem cells from human pluripotent stem cells, with the goal of developing drug screening and cell therapy applications for human blood system disorders. The breakthrough technology is included in a new United States patent application. We believe this stem cell technology dramatically advances our ability to produce and purify this important blood stem cell precursor for both in vitro drug screening and in vivo cell therapy applications. In addition to defining new cell culture methods for our use, the technology describes the surface characteristics of stem cell-derived adult hematopoietic stem cells. Most groups study embryonic blood development from stem cells, but, for the first time, we are now able to not only purify the stem cell-derived precursor of all adult hematopoietic cells, but also pinpoint the precise timing when adult blood cell differentiation takes place in these cultures. We believe these early cells have the potential to be the precursors of the ultimate adult, bone marrow-repopulating hematopoietic stem cells to repopulate the blood and immune system when transplanted into patients prepared for bone marrow transplantation. These cells have important potential therapeutic applications for the restoration of healthy blood and immune systems in individuals undergoing transplantation therapies for cancer, organ grafts, HIV infections or for acquired or genetic blood and immune deficiencies.

AV-101-Related Intellectual Property

We have exclusive licenses to issued U.S. patents related to the use and function of AV-101, and various central nervous system (CNS)-active molecules related to AV-101. These patents are held by the University of Maryland, Baltimore, the Cornell Research Foundation, Inc. and Aventis, Inc. The principle U.S. method of use patent related to AV-101 expired in February 2011. Foreign counterparts to that U.S. patent expired in February 2012. However, in 2013 and through the date of this report, we have filed three new U.S. patent applications relating to AV-101. In addition, among the key components of our commercial protection strategy with respect to AV-101 is the 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 new chemical entities (NCEs) such as AV-101, which, by definition, are innovative and have not been approved previously by the FDA, either alone or in combination. The FDA's New Drug Product Exclusivity protection provides the holder of an FDA-approved new drug application (NDA) five (5) years of protection from new 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 certain abbreviated new drug applications (ANDAs), during the 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.

Under the terms of our license agreement, we may be obligated to make royalty payments on 2% of net sales of products using the unexpired patent rights, if any, including products containing compounds covered by the patent rights. Additionally, we may be required to pay a 1% royalty on net sales of combination products that use unexpired patent rights, if any, or contain compounds covered by the patent rights. Consequently, future sales of AV-101 may be subject to a 2% royalty obligation. There are no license, milestone or maintenance fees under the agreement. The agreement remains in force until the later of: (i) the expiration or invalidation of the last patent right; and (ii) 10 years after the first commercial sale of the first product that uses the patent rights or contains a compound covered by the patent rights. This agreement may also be terminated earlier at the election of the licensor upon our failure to pay any monies due, our failure to provide updates and reports to the licensor, our failure to provide the necessary financial and other resources required to develop the products, or our failure to cure within 90 days any breach of any provision of the agreement. We may also terminate the agreement at any time upon 90 days' written notice so long as we make all payments due through the effective date of termination.

Research and Development

Our research and development expense was approximately $2.5 million and $3.4 million for the years ended March 31, 2014 and 2013, respectively, or approximately 49% of our operating expenses for each of the years ended March 31, 2014 and 2013. Our research and development expense consists of both internal and external expenses incurred in sponsored stem cell research and drug development activities, costs associated with the development of AV-101 and costs related to the licensing, application and prosecution of our intellectual property.

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Competition

We believe that our human pluripotent stem cell (hPSC) technology platform, Human Clinical Trials in a Test Tube, 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 rapidly 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, development and rescue of new molecular entities (NMEs), 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 pluripotent stem cell technology includes the following: Acea Biosciences, Advanced Cell Technology, Athersys, BioTime, Cellectis Bioresearch, Cellular Dynamics, Cellerant Therapeutics, Cytori Therapeutics, HemoGenix, International Stem Cell, NeoStem, Neuralstem, Organovo Holdings, PluriStem Therapeutics, Stem Cells, and Stemina BioMarker Discovery. Pharmaceutical companies and other established corporations such as Bristol-Myers Squibb, GE Healthcare Life Sciences, GlaxoSmithKline, Life Technologies, Novartis, Pfizer, Roche Holdings and others have been and are expected to continue pursuing internally various stem cell-related research and development programs. 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.

We believe the best and most valuable near term commercial application of our Human Clinical Trials in a Test Tube platform is internal production of NMEs, which we refer to as Drug Rescue Variants, through small molecule drug rescue. We believe that the stem cell technologies underlying our Human Clinical Trials in a Test Tube platform and our primary focus on opportunities to produce small molecule NMEs through drug rescue provide us substantial competitive advantages associated with application of human biology at the front end of the drug development process, before animal and human testing. Although we believe that our model for the application of human pluripotent stem cell technology for drug rescue is novel, significant competition may arise or otherwise increase considerably as the acceptance and use of hPSC technology, the sale of hPSC-derived human heart and liver cells, and the availability of hPSC-related contract predictive toxicology screening services, for drug discovery, development and rescue, as well as cell therapy and regenerative medicine, continue to become more widespread throughout the academic research community and the pharmaceutical and biotechnology industries. In addition, significant competition may arise from those academic research institutions, contract research organizations, and biopharmaceutical companies currently producing or capable of producing, currently using or capable of using, hPSC-derived heart cells and liver cells for third-party sales, contract screening or cell therapy research and development, that elect or their customers elect to transform their current business operations to include internal drug rescue and development of small molecule NMEs in a manner similar to our drug rescue model.

With respect to AV-101, we believe that a range of pharmaceutical and biotechnology companies have programs to develop small molecule drug candidates for the treatment of neuropathic pain, epilepsy, depression, Parkinson's disease and other neurological conditions and diseases, including Abbott Laboratories, GlaxoSmithKline, Johnson & Johnson, Novartis, and Pfizer. We expect that AV-101 will have to compete with a variety of therapeutic products and procedures. With respect to each Drug Rescue Variant we are able to produce, we anticipate that a range of pharmaceutical and biotechnology companies will have programs to develop small molecule drug candidates or biologics for the treatment of the diseases or conditions targeted by each such Drug Rescue Variant.

Government Regulation

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.

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With respect to drug development, government authorities at the federal, state and local levels in the U.S. and other countries extensively regulate, among other things, the research, development, testing, manufacture, labeling, promotion, advertising, distribution, marketing, pricing and export and import of pharmaceutical products such as those we are developing. In the U.S., pharmaceuticals, biologics and medical devices are subject to rigorous FDA regulation. Federal and state statutes and regulations in the United States govern, among other things, the testing, manufacture, safety, efficacy, labeling, storage, export, record keeping, approval, marketing, advertising and promotion of our potential drug rescue variants. The information that must be submitted to the FDA in order to obtain approval to market a new drug varies depending on whether the drug is a new product whose safety and effectiveness has not previously been demonstrated in humans, or a drug whose active ingredient(s) and certain other properties are the same as those of a previously approved drug. Product development and approval within this regulatory framework takes a number of years and involves significant uncertainty combined with the expenditure of substantial resources.

Companies seeking FDA approval to sell a new prescription drug in the United States must test it in various ways. Currently, first are laboratory and animal tests. Next are tests in humans to see if the drug candidate is safe and effective when used to treat or diagnose a disease. After testing the drug candidate, the company developing it then sends the FDA an application called a New Drug Application (NDA). Some drug candidates are made out of biologic materials, including human cells, such as the human cells derived from human pluripotent stem cells. Instead of an NDA, new biologic drug candidates are approved using a Biologics License Application (BLA). Whether an NDA or a BLA, the application includes:

- the drug candidate’s test results;

- manufacturing information to demonstrate the company developing the drug candidate can properly manufacture it; and

- the proposed label for the drug candidate, which provides necessary information about the drug candidate, including uses for which it has been shown to be effective, possible risks, and how to use it.

If a review by FDA physicians and scientists shows the drug candidate's benefits outweigh its known risks and the drug candidate can be manufactured in a way that ensures a quality product, the drug candidate is approved and can be marketed in the United States.

New drug and biological product development and approval takes many years, involves the expenditure of substantial resources and is uncertain to succeed. Many new drug and biological candidates appear promising in early stages of development but ultimately do not reach the market because they cannot meet FDA or other regulatory requirements. In addition, the current regulatory framework may change through regulatory, legislative or judicial actions or that additional regulations will not arise during development that may affect approval, delay the submission or review of an application.

The activities required before a new drug or biological candidate may be approved for marketing in the U.S. begin with nonclinical testing, which includes laboratory evaluation and animal studies to assess the potential safety and efficacy of the product as formulated. Results of nonclinical studies are summarized in an Investigational New Drug (IND) application to the FDA. Human clinical trials may begin 30 days following submission of an IND application, unless the FDA requires additional time to review the application or raise questions.

Clinical testing involves the administration of the new drug or biological candidate to healthy human volunteers or to patients under the supervision of a qualified principal investigator, usually a physician, pursuant to an FDA-reviewed protocol. Each clinical study is conducted under the auspices of an institutional review board (IRB) at each of the institutions at which the study will be conducted. A clinical plan, or “protocol,” accompanied by the approval of an IRB,

must be submitted to the FDA as part of the IND application prior to commencement of each clinical trial. Human clinical trials are conducted typically in three sequential phases. Phase I trials primarily consist of testing the product's safety in a small number of patients or healthy volunteers. In Phase II trials, the safety and efficacy of the biological candidate is evaluated in a specific patient population. Phase III trials typically involve additional testing for safety and clinical efficacy in an expanded patient population at geographically dispersed sites. The FDA may order the temporary or permanent discontinuance of a nonclinical or clinical trial at any time for a variety of reasons, particularly if safety concerns exist.

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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 either 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 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.

Canada

In Canada, stem cell research and development is governed by two policy documents and by one legislative statute: the Guidelines for Human Pluripotent Stem Cell Research (the Guidelines) issued by the Canadian Institutes of Health Research; the Tri-Council Statement: Ethical Conduct for Research Involving Humans (the TCPS); and the Assisted Human Reproduction Act (the Act). The Guidelines and the TCPS govern stem cell research conducted by, or under the auspices of, institutions funded by the federal government. Should we seek funding from Canadian government agencies or should we conduct research under the auspices of an institution so funded, we may have to ensure the compliance of such research with the ethical rules prescribed by the Guidelines and the TCPS.

The Act subjects all research conducted in Canada involving the human embryo, including hESC derivation (but not the stem cells once derived), to a licensing process overseen by a federal licensing agency. However, as of the date of this report, the provisions of the Act regarding the licensing of hESC derivation were not in force.

We are not currently conducting stem cell research in Canada. We are, however, sponsoring pluripotent stem cell research by Dr. Gordon Keller at UHN's McEwen Centre. We anticipate conducting pluripotent stem cell research (with both hESCs and hiPSCs), in collaboration with Dr. Keller and his research team, at UHN during 2014 and beyond pursuant to our long term sponsored research collaboration with Dr. Keller and UHN. Should the provisions of the Act come into force, we may have to apply for a license for all hESC research we may sponsor or conduct in Canada and ensure compliance of such research with the provisions of the Act.

Foreign

In addition to regulations in the U.S., we may be subject to a variety of foreign regulations governing clinical trials and commercial sales and distribution of our products outside of the U.S. Whether or not we obtain FDA approval for a product, we must obtain approval of a product by the comparable regulatory authorities of foreign countries before we can commence clinical trials or marketing of the product in those countries. The approval process varies from country to country, and the time may be longer or shorter than that required for FDA approval. The requirements governing the conduct of clinical trials, product licensing, pricing and reimbursement vary greatly from country to country.

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Subsidiaries and Inter-Corporate Relationships

VistaGen Therapeutics. Inc., a California corporation, is our wholly-owned subsidiary and has the following two wholly-owned subsidiaries: VistaStem Canada Inc., a corporation incorporated pursuant to the laws of the Province of Ontario, intended to facilitate our stem cell-based research and development and drug rescue activities in Ontario, Canada including our collaboration with Dr. Keller and UHN should we elect to expand our U.S. operations into Canada; and Artemis Neuroscience, Inc., a corporation incorporated pursuant to the laws of the State of Maryland and focused on development of AV-101. The operations of VistaGen Therapeutics, Inc., a California corporation, and each of its two wholly-owned subsidiaries are managed by our senior management team based in South San Francisco, California.

Employees

We have ten full-time employees, four of whom have doctorate degrees. Seven full-time employees work in research and development and laboratory support services and three full-time employees work in general and administrative roles. Staffing for all other functional areas is achieved through strategic relationships with service providers and consultants, each of whom provides services on an as-needed basis, including human resources and payroll, accounting and public company reporting, information technology, facilities, legal, stock plan administration, investor relations and web site maintenance, regulatory affairs, and FDA program management. In addition, we currently conduct some of our research and development efforts through sponsored research relationships with stem cell scientists at academic research institutions in the U.S. and Canada, including Dr. Keller's laboratories at UHN. See "Business – Strategic Transactions and Relationships."

None of our employees is represented by a labor union or is subject to a collective bargaining agreement. We believe that our current relationship with all of our employees is good.

Environmental Regulation

Our business does not require us to comply with any particular environmental regulations.

Item 1A. Risk Factors

Investing in our common stock 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 Annual Report on Form 10-K, including our financial statements and related notes, before deciding whether to purchase shares of our common stock. 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 Our Business and Strategy

We are a development stage biotechnology company with no 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 biotechnology company. Since inception, we have generated approximately $16.4 million of revenues from strategic collaborations and grant awards. However, we currently have no approved products and 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 products we produce;
- maintain, leverage and expand our intellectual property portfolio;
- establish and maintain sales, distribution and marketing 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.

Moreover, we and any future strategic partner will need to receive regulatory approval for any new drug candidate, including each Drug Rescue Variant, biological candidate or regenerative medicine product before it may be marketed and distributed. Such regulatory approval will require, among numerous other things, completing carefully controlled and well-designed clinical trials demonstrating the safety and efficacy of each new product candidate. This process is lengthy, expensive and uncertain. As a company, we have limited experience developing new drug candidates, including Drug Rescue Variants, biological candidates or regenerative medicine products, including conducting clinical trials and in other areas required for the successful development and commercialization of therapeutic products. Such trials will require additional financial and management resources, third-party collaborators with the requisite clinical experience or reliance on third party clinical investigators, contract research organizations and independent consultants. Relying on third parties may force us to encounter delays that are outside of our control, which could materially harm our business.

If we are unsuccessful in accomplishing these fundamental objectives, or if we encounter delays in the regulatory approval process beyond our control, we may not be able to develop product candidates, raise capital, expand our business or continue our operations.

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Our future success is highly dependent upon our ability to produce product candidates, including Drug Rescue Variants, using stem cell technology, human cells derived from stem cells, our proprietary human cell-based bioassay systems and medicinal chemistry, and we cannot provide any assurance that we will successfully produce Drug Rescue Variants or other product candidates, or that, if produced, any of our Drug Rescue Variants or other product candidates will be developed and commercialized.

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

- our research methodology may not be successful in identifying potential Drug Rescue Candidates;

- competitors may develop alternatives that render our Drug Rescue Variants obsolete;

- a Drug Rescue Variant 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 Variant may not be capable of being produced in commercial quantities at an acceptable cost, or at all; or

- a Drug Rescue Variant may not be accepted as safe and effective by regulatory authorities, patients, the medical community or third-party payors.

Our future success depends heavily on our ability to use stem cell technology, human cells derived from stem cells, proprietary human cell-based bioassay systems, especially CardioSafe 3D, and medicinal chemistry to produce Drug Rescue Variants and, develop, obtain regulatory approval for, and commercialize lead Drug Rescue Variants, on our own or in strategic collaborations, which may never occur. We currently generate no revenues, and we may never be able to develop or commercialize a marketable drug.

We have limited operating history with respect to the identification and assessment of potential Drug Rescue Candidates and no operating history with respect to the production of Drug Rescue Variants, and we may never be able to produce a Drug Rescue Variant. If we are unable to identify suitable Drug Rescue Candidates for our drug rescue programs, including AV-101, or produce suitable lead Drug Rescue Variants for license to and preclinical and clinical development by pharmaceutical companies and others, we may not be able to obtain sufficient revenues in future periods, which likely would result in significant harm to our financial position and adversely impact our stock price. There are a number of factors, in addition to the utility of CardioSafe 3D, that may impact our ability to identify and assess Drug Rescue Candidates and produce, develop and commercialize Drug Rescue Variants, 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 assay 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 license or sell Drug Rescue Candidates to us on commercially reasonable terms;

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our medicinal chemistry collaborator's ability to design and produce proprietary Drug Rescue Variants based on the novel biology and structure-function insight we provide using CardioSafe 3D or LiverSafe 3D; and

· financial resources available to us to develop and commercialize lead Drug Rescue Variants internally, or, if we license them to strategic partners, the resources such partners choose to dedicate to development and commercialization of any Drug Rescue Variants licensed from us.

Even if we do produce a Drug Rescue Variant, we can give no assurance that we will be able to develop and commercialize it as a marketable drug, on our own or in a strategic collaboration. Before we generate any revenues from product sales, we must produce additional product candidates through drug rescue and we or our potential strategic collaborator must complete preclinical and clinical development of one or more of our product candidates, conduct human subject research, 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.

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We have not previously submitted a biologics license application, or BLA, or a new drug application or NDA, to the FDA, or similar drug approval filings to comparable foreign authorities, for any product candidate. We cannot be certain that any of our product candidates will be successful in clinical trials or receive regulatory approval. Further, our product candidates may not receive regulatory approval even if they are successful in clinical trials. If we do not receive regulatory approvals for our product candidates, we may not be able to continue our operations. Even if we successfully obtain regulatory approvals to market one or more of our product candidates, our revenues will be dependent, in part, upon the size of the markets in the territories for which we gain regulatory approval and have commercial rights. If the markets for patient subsets that we are targeting are not as significant as we estimate, we may not generate significant revenues from sales of such products, if approved.

We or our potential collaborator may also seek regulatory approval to commercialize our product candidates in the United States, the European Union and potentially in additional foreign countries. While the scope of regulatory approval is similar in other countries, to obtain separate regulatory approval in many other countries we must comply with numerous and varying regulatory requirements of such countries regarding safety and efficacy, clinical trials and commercial sales, pricing and distribution of our product candidates, and we cannot predict success in these jurisdictions.

Our CardioSafe 3D internal validation studies have not been subjected to extensive external peer review or validation.

Our proprietary internal studies conducted to validate the utility of CardioSafe 3D for drug rescue, including our ability to use it to predict the cardiac effects, both toxic and nontoxic, of Drug Rescue Candidates, have not been subjected to extensive external peer review or validation. It is possible, therefore, that the results we have obtained from our successful internal validation studies may not be replicable by external peer reviewers. We are currently focused on identifying and assessing Drug Rescue Candidates available in the public domain. However, should we seek to license or acquire Drug Rescue Candidates from third-parties, and such third-parties cannot replicate our results or do not have confidence in the capabilities of CardioSafe 3D, it may be difficult for us to acquire from them certain Drug Rescue Candidates which might be of interest to us. Even if such results can be replicated by external peer reviewers or other third-parties, they may nevertheless conclude that their current screening models are better than our CardioSafe 3D and that a license to the Drug Rescue Candidate we seek from them is not warranted. Our drug rescue business model is predicated on our ability to identify and, if information is not otherwise available in the public domain, obtain licenses from third-parties to Drug Rescue Candidates of interest to us. If third-party licenses are required, and if we cannot obtain such licenses to on reasonable terms, or at all, our business may be adversely affected.

If CardioSafe 3D fails to predict accurately and efficiently the cardiac effects, both toxic and nontoxic, of Drug Rescue Candidates and Drug Rescue Variants, then our drug rescue business will be adversely affected.

Our success is highly 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 Variants. 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.

We have not yet fully validated LiverSafe 3D for potential drug rescue applications, and we may never do so.

We have successfully developed proprietary protocols for controlling the differentiation of human pluripotent stem cells to produce functional, mature, adult liver cells. However, we have not yet fully validated our ability to use the human liver cells we produce for LiverSafe 3D to predict important biological effects, both toxic and nontoxic, of reference drugs, Drug Rescue Candidates or Drug Rescue Variants on the human liver, including drug-induced liver injury and adverse drug-drug interactions. Furthermore, we may never be able to do so, which could adversely affect

our business and the potential applications of LiverSafe 3D for drug rescue and regenerative medicine.

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CardioSafe 3D, and, when validated, LiverSafe 3D may not be meaningfully more predictive of the behavior of human cells than existing methods.

The success of our drug rescue business is highly dependent, in the first instance, upon CardioSafe 3D, and, in the second instance, when validated, LiverSafe 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, and, when validated, LiverSafe 3D, will be more efficient or accurate at predicting the heart or liver safety of new drug candidates than the testing models currently used. If CardioSafe 3D and LiverSafe 3D fail to provide a meaningful difference compared to existing or new models in predicting the behavior of human heart and liver cells, 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 Variants for which there proves to be no demand.

To generate revenue from our drug rescue activities, we must produce Drug Rescue Variants for which there proves to be demand within the healthcare marketplace, and, if we intend to out-license a particular Drug Rescue Variant for development and commercialization prior to market approval, then also among pharmaceutical companies and other potential strategic collaborators. However, we may produce Drug Rescue Variants 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.

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 new and technically complex, and the time and resources necessary to develop new cell types and customized bioassay systems are difficult to predict in advance. We intend 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 regenerative medicine, potential applications of our Human Clinical Trials in a Test Tube platform. In particular, we are planning to conduct development programs related to producing and using functional, mature adult liver cells to validate LiverSafe 3D as a novel bioassay system for drug rescue, as well as exploratory nonclinical regenerative medicine programs involving blood, bone, cartilage, heart, liver and insulin-producing pancreatic beta-islet cells. Although we and our collaborators have developed proprietary protocols for the production of multiple differentiated cell types, we may encounter difficulties in differentiating particular cell types, even when following these proprietary protocols. These difficulties may result in delays in production of certain cells, assessment of certain Drug Rescue Candidates and Drug Rescue Variants, and performance of certain exploratory nonclinical 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, liver and pancreatic 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, liver and insulin-producing pancreatic beta-islet cells could have a substantial adverse effect on our potential drug rescue and regenerative medicine business opportunities and results of operations.

If we are unable to keep up with rapid technological changes in our field, we will be unable to operate profitably.

We are engaged in activities in the life sciences field, which is characterized by rapid technological changes, frequent new product introductions, changing needs and preferences, emerging competition, and evolving industry standards. If

we fail to anticipate or respond adequately to technological developments, our business, revenue, financial condition and operating results could suffer materially. Although we believe we are the first stem cell technology company focused primarily on drug rescue, we anticipate that we will face increased competition in the future as competitors develop or access new or improved bioassay systems and explore and enter the drug rescue market with new technologies. Competitors may have significantly greater financial, manufacturing, sales and marketing resources and may be able to respond more quickly and effectively than we can to new opportunities. In light of these advantages, even if our technology is effective in producing Drug Rescue Variants, potential development partners might prefer new drug candidates available from others or develop their own new drug candidates in lieu of licensing or purchasing our Drug Rescue Variants. We may not be able to compete effectively against these organizations. Our failure to compete effectively could materially and adversely affect our business, financial condition and results of operations.

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We face substantial competition, which may result in others discovering, developing or commercializing product candidates before, or more successfully, than we do.

Our future success depends on our ability to demonstrate and maintain a competitive advantage with respect to the design, development and commercialization of Drug Rescue Variants. Our competitors may succeed in developing product candidates for the same indications we are pursuing before we do, obtaining regulatory approval for competing products or gaining acceptance of their products within the same markets that we are targeting for our Drug Rescue Variants. If, either on our own or in collaboration with a strategic partner, we are not "first to market" with one of our Drug Rescue Variants, our competitive position could be compromised because it may be more difficult for us or our partner to obtain marketing approval for our Drug Rescue Variant and successfully market it as a second competitor. We expect any Drug Rescue Variants that we commercialize, either independently or in collaboration, will compete with products from other companies in the biotechnology and pharmaceutical industries.

Many of our competitors have substantially greater research and development and commercial infrastructures and financial, technical and personnel resources than we have. We will not be able to compete successfully unless we:

- design, develop, produce and commercialize, either on our own or with collaborators, Drug Rescue Variants that are superior to other products in development or in the market;
- attract qualified scientific, medical, sales and marketing and commercial personnel or collaborators;
- obtain patent and/or other proprietary protection for our Drug Rescue Variants; and
- obtain, either on our own or in collaboration with strategic partners, required regulatory approvals for our Drug Rescue Variants.

Established competitors may invest heavily to quickly discover and develop novel compounds that could make our Drug Rescue Variants obsolete. In addition, any new product that competes with an approved product must demonstrate compelling advantages in efficacy, convenience, tolerability and safety in order to overcome price competition and to be commercially successful. If we are not able to compete effectively against our current and future competitors, our business will not grow and our financial condition and operations will suffer.

Other companies, academic institutions, government agencies and other public and private research organizations are conducting research, seeking patent protection and establishing collaborative arrangements for research, development and marketing of assays similar to ours and Drug Rescue Variants we may produce. These companies and institutions also compete with us in recruiting and retaining qualified scientific and management personnel, obtaining collaborators and licensees, as well as in acquiring technologies complementary to our programs.

As a result of the foregoing, our competitors may develop more effective or more affordable products, or achieve earlier patent protection or product commercialization than we will. Most significantly, competitive products may render any technologies and Drug Rescue Variants that we develop obsolete, which would negatively impact our business and ability to sustain operations.

With respect to drug rescue, the licensing and acquisition of proprietary small molecule compounds, even compounds that have failed in development due to heart or liver safety concerns, is a highly competitive area, and a number of more established companies may also pursue strategies to license, acquire, rescue and develop small molecule compounds that we may consider to be Drug Rescue Candidates. These established companies have a competitive advantage over us due to their size, cash resources and greater clinical development and commercialization capabilities. In addition, companies that perceive us to be a competitor may be unwilling to sell or license Drug

Rescue Candidate rights to us. We have limited experience in negotiating licenses to drug candidates and there can be no assurances that we will be able to acquire or obtain licenses to Drug Rescue Candidates in the future, on commercially reasonable terms, if at all, should we elect to pursue such third-party licenses. If we are unable to acquire or obtain licenses to Drug Rescue Candidates we seek, our business may be adversely affected.

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

Some of our most important ongoing and planned research and development programs involve the use 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 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.

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. These potential ethical concerns do not apply to induced pluripotent stem cells (iPSCs), or our plans to pursue pilot nonclinical regenerative medicine studies involving human cells derived from iPSCs, because their derivation does not involve the use of embryonic tissues.

We have assumed that the biological capabilities of induced pluripotent stem cells (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 Human Clinical Trials in a Test Tube platform could be harmed.

We use both hESCs and iPSCs for drug rescue purposes. However, we anticipate that our future exploratory research and development focused on potential regenerative medicine applications of our Human Clinical Trials in a Test Tube 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 applications of our Human Clinical Trials in a Test Tube platform, this would negatively affect our ability to explore expansion of our platform, including, in particular, where it would be preferable to use iPSCs to reproduce rather than approximate the effects of certain specific genetic variations.

If we fail to attract and retain senior management and key scientific personnel, we may be unable to successfully produce, develop trials and commercialize our Drug Rescue Variants.

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 senior management, as well as other employees, consultants and scientific collaborators. As of June 1, 2014, we had 10 full-time employees, which may make us more reliant on our individual employees than companies with a greater number of employees. Although none of our key scientific personnel or members of our senior management has informed us that he or she intends to resign or retire in the near future, the loss of services of any of these individuals could delay or prevent the successful development of potential expansions and applications of our Human Clinical Trials in a Test Tube platform and our production of

Drug Rescue Variants 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 activities. We may not be able to attract and retain quality personnel on acceptable terms.

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In addition, we rely on consultants and advisors, including scientific and clinical advisors, to assist us in formulating our research and development strategy, including our drug rescue strategy. 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.

We may encounter difficulties in managing our growth and expanding our operations successfully.

As we seek to advance our proposed CardioSafe 3D drug rescue programs, produce and develop Drug Rescue Variants, and develop and validate LiverSafe 3D, we will need to expand our research and development capabilities 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.

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 produce and develop Drug Rescue Variants or regenerative medicine products, either on our own or in collaboration with others, we will face an inherent risk 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 products. For example, we may be sued if any Drug Rescue Variant or regenerative medicine product 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 our Drug Rescue Variants or other 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;
- product recalls, withdrawals or labeling, marketing or promotional restrictions;
- loss of revenue;

- the inability to commercialize our product candidates; and
- a decline in our stock price.

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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.

To the extent we enter into licensing or collaboration agreements to develop and commercialize our product candidates, including Drug Rescue Variants, our dependence on such relationships may adversely affect our business.

We may enter into strategic partnerships in the future, including collaborations with other biotechnology or pharmaceutical companies, to enhance and accelerate the development and commercialization of our product candidates. Our strategy to produce, develop and commercialize our product candidates, including any Drug Rescue Variants, may depend on our ability to enter into such agreements with third-party collaborators. We face significant competition in seeking appropriate strategic partners. Supporting diligence activities conducted by potential collaborators and negotiating the financial and other terms of a collaboration agreement are long and complex processes with uncertain results. Moreover, we may not be successful in our efforts to establish a strategic partnership or other alternative arrangements for any future product candidates and programs because our research and development pipeline may be insufficient, our product candidates and programs may be deemed to be at too early of a stage of development for collaborative effort and/or third parties may not view our product candidates and programs as having the requisite potential to demonstrate safety and efficacy. Even if we are successful in entering into one or more strategic collaboration agreements with third-parties, such collaborations may involve greater uncertainty for us, as we may have less control over certain aspects of our collaborative programs than we do over our proprietary internal development and commercialization programs. We may determine that continuing a collaborative arrangement under the terms provided is not in our best interest, and we may terminate the collaboration. Our collaborators could also delay or terminate their agreements, and our products subject to collaborative arrangements may never be successfully commercialized.

Further, our future collaborators may develop alternative products or pursue alternative technologies either on their own or in collaboration with others, including our competitors, and the priorities or focus of our collaborators may shift such that our programs receive less attention or resources than we would like, or they may be terminated altogether. Any such actions by our collaborators may adversely affect our business prospects and ability to earn revenues. In addition, we could have disputes with our future collaborators, such as the interpretation of terms in our agreements. Any such disagreements could lead to delays in the development or commercialization of potential products or could result in time-consuming and expensive litigation or arbitration, which may not be resolved in our favor.

Even with respect to certain other products that we intend to commercialize ourselves, we may enter into agreements with collaborators to share in the burden of conducting preclinical studies, clinical trials, manufacturing and marketing our product candidates or products. In addition, our ability to apply our proprietary technologies to develop proprietary compounds will depend on our ability to establish and maintain licensing arrangements or other collaborative arrangements with the holders of proprietary rights to such compounds. We may not be able to establish such arrangements on favorable terms or at all, and our future collaborative arrangements may not be successful.

We cannot provide any assurance that our future collaborations will not terminate development before achievement of revenue-generating milestones or market approval, that our future collaborative arrangements will result in successful development and commercialization of Drug Rescue Variants, or that we will derive any revenues from such future

arrangements. The failure of any collaborator to conduct, successfully and diligently, their collaborative activities relating to the product candidate we license or sell to them would have a material adverse effect on us. Additionally, to the extent that we are unable to license or sell our Drug Rescue Variants to pharmaceutical companies or others, we would require substantial additional capital to undertake development and commercialization activities for any such product candidate on our own, and that substantial additional capital may not be available to us on a timely basis, on reasonable terms, or at all.

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Our and our collaborators' relationships with customers and third-party payors in the United States and elsewhere will be subject to applicable anti-kickback, fraud and abuse and other healthcare laws and regulations, which could expose us to criminal sanctions, civil penalties, contractual damages, reputational harm and diminished profits and future earnings.

Healthcare providers, physicians and third-party payors in the United States and elsewhere will play a primary role in the recommendation and prescription of any product candidates for which we may obtain marketing approval. Our or our future collaborator's arrangements with third-party payors and customers may 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 products for which we or they obtain marketing approval. Restrictions under applicable federal, state and foreign healthcare laws and regulations include the following:

- the federal healthcare 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 and state healthcare programs such as Medicare and Medicaid;

- the federal False Claims Act imposes criminal and civil penalties, 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 and also includes provisions allowing for private, civil whistleblower or "qui tam" actions;

- the federal Health Insurance Portability and Accountability Act of 1996 (HIPAA), as amended by the Health Information Technology for Economic and Clinical Health Act (HITECH), imposes criminal and civil liability for executing a scheme to defraud any healthcare benefit program. HIPAA and HITECH also regulate the use and disclosure of identifiable health information by health care providers, health plans and health care clearinghouses, and impose obligations, including mandatory contractual terms, with respect to safeguarding the privacy, security and transmission of identifiable health information as well as requiring notification of regulatory breaches. HIPAA and HITECH violations may prompt civil and criminal enforcement actions as well as enforcement by state attorneys general;

- 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 under the Health Care Reform Law requires manufacturers of drugs, devices, biologics and medical supplies 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, 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 health care providers or marketing expenditures; and

- analogous anti-kickback, fraud and abuse and healthcare laws and regulations in foreign countries.

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Efforts to ensure that our and our future collaborators' business arrangements with third parties will comply with applicable healthcare laws and regulations will involve substantial costs. It is possible that governmental authorities will conclude that our or their business practices may not comply with current or future statutes, regulations or case law involving applicable fraud and abuse or other healthcare laws and regulations. If our or their operations are found to be in violation of any of these laws or any other governmental regulations that may apply to us, we may be subject to significant civil, criminal and administrative penalties, damages, fines, exclusion from government funded healthcare programs, such as Medicare and Medicaid, and the curtailment or restructuring of our operations. If any of the physicians or other providers or entities with whom we or our collaborators expect to do business are found to be not in compliance with applicable laws, they may be subject to criminal, civil or administrative sanctions, including exclusions from government funded healthcare programs.

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.

To the extent our research and development activities involve using induced pluripotent stem cells, 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.

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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 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 cell-based 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.

Our human cells and human cell-based bioassay systems, including CardioSafe 3D and LiverSafe 3D, are not currently sold, for research 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 cells we derive from human pluripotent stem cells 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 cell therapy or for other 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.

We intend to rely on third-party contract manufacturers to produce our product candidate supplies and we intend to rely on such third-party manufacturers to produce commercial supplies of any approved product candidates we develop on our own. Any failure by a third-party manufacturer to produce for us supplies of product candidates we elect to develop on our own may delay or impair our ability to initiate or complete clinical trials, commercialize our product candidates, or continue to sell any products we commercialize.

We do not currently own or operate any manufacturing facilities, and we lack sufficient internal staff to produce product candidate supplies ourselves. As a result, we plan to work with third-party contract manufacturers to produce sufficient quantities of our product candidates for future preclinical and clinical testing and commercialization. If we are unable to arrange for such a third-party manufacturing source, or fail to do so on commercially reasonable terms or on a timely basis, we or our potential strategic partner may not be able to successfully produce, develop, and market our product candidates or may be delayed in doing so.

Reliance on third-party manufacturers entails risks to which we or our potential collaborators would not be subject if we or they manufactured product candidates ourselves or themselves, including reliance on the third party for regulatory compliance and quality assurance, the possibility of breach of the manufacturing agreement by the third party because of factors beyond our control (including a failure to synthesize and manufacture our product candidates in accordance with our product specifications), the possibility of termination or nonrenewal of the agreement by the

third party, based on its own business priorities, at a time that is costly or damaging to us, or misappropriation of proprietary formulas or protocols. We will be, and our potential strategic partners may be, dependent, on the ability of these third-party manufacturers to produce adequate supplies of drug product to support development programs and future commercialization of our product candidates. In addition, the FDA and other regulatory authorities require that all product candidates be manufactured according to cGMP and similar foreign standards. Any failure by our or our collaborators' third-party manufacturers to comply with cGMP or failure to scale up manufacturing processes, including any failure to deliver sufficient quantities of product candidates in a timely manner, could lead to a delay in, or failure to obtain, regulatory approval for trial initiation or marketing of any product candidates we may produce, including Drug Rescue Variants. In addition, such failure could be the basis for action by the FDA to withdraw approvals for product candidates previously granted and for other regulatory action, including recall or seizure, fines, imposition of operating restrictions, total or partial suspension of production or injunctions.

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We have limited staffing. We will, and our potential strategic partners may, rely on contract manufacturers to purchase from third-party suppliers the materials necessary to produce our product candidates for required studies. There may be a small number of suppliers for certain capital equipment and materials that we or our collaborators use to manufacture our product candidates. Such suppliers may not sell these materials to our manufacturers at the times we or they need them or on commercially reasonable terms. We will not have any control over the process or timing of the acquisition of these materials by our manufacturers. Although we and our collaborators generally will not begin a required study unless we or they believe a sufficient supply of a product candidate exists to complete the study, any significant delay in the supply of a product candidate or the material components thereof for an ongoing study due to the need to replace a third-party manufacturer could considerably delay completion of the studies, product testing and potential regulatory approval. If we or our manufacturers are unable to purchase these materials after regulatory approval has been obtained for our product candidates, the commercial launch of our product candidates could be delayed or there could be a shortage in supply, which would impair our ability to generate revenues from the sale of our product candidates.

In addition, we or our potential strategic partner may need to optimize the manufacturing processes for a particular drug substance and/or drug product so that certain product candidates may be produced in sufficient quantities of adequate quality, and at an acceptable cost, to support required development activities and commercialization. Contract manufacturers may not be able to adequately demonstrate that an optimized product candidate is comparable to a previously manufactured product candidate which could cause significant delays and increased costs to our or our collaborators' development programs. Our manufacturers may not be able to manufacture our product candidates at a cost or in quantities or in a timely manner necessary to develop and commercialize them. If we successfully commercialize any of our drugs, we may be required to establish or access large-scale commercial manufacturing capabilities. In addition, assuming that our drug development pipeline increases and matures, we will have a greater need for clinical trial and commercial manufacturing capacity. To meet our projected needs for commercial manufacturing, third party manufactures with whom we work will need to increase their scale of production or we will need to secure alternate suppliers.

If, in the future, we are unable to enter into licensing or collaboration agreements for the sales, marketing and distribution of our Drug Rescue Variants and other product candidates, such as AV-101, we may not be successful in commercializing our Drug Rescue Variants and other product candidates.

We currently have a relatively small number of employees and do not have a sales or marketing infrastructure, and we, including our executive officers, do not have any significant sales, marketing or distribution experience. We will be opportunistic in seeking to collaborate with others to develop and commercialize Drug Rescue Variants and future products if and when they are developed and approved. 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 our Drug Rescue Variants 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.

Our business is subject to the risks of earthquakes, fire, floods and other natural catastrophic events, and to interruption by man-made problems such as computer viruses or terrorism.

Our corporate headquarters are located in the San Francisco Bay Area, a region known for seismic activity. A significant natural disaster, such as an earthquake, fire or a flood, could harm our business. In addition, our servers are

vulnerable to computer viruses, break-ins and similar disruptions from unauthorized tampering with our computer systems. In addition, acts of terrorism or war could cause disruptions in our business or the economy as a whole.

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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 strategic 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 Securities and Exchange Commission (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 are rigorous and we may not be able to continue 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 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.

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Risks Related to Production, Development, and Regulatory Approval of Product Candidates

Even if we are able to begin clinical trails for a Drug Rescue Variant, we may encounter considerable delays and/or expend considerable resources without producing a marketable product capable of generating revenue.

We may never generate revenues from sales of a Drug Rescue Variant or any other product because of a variety of risks inherent in our business, including the following:

- clinical trials may not demonstrate the safety and efficacy of any Drug Rescue Variant, other new drug candidate, biological candidate or regenerative medicine product candidate;
- completion of nonclinical or clinical trials may be delayed, or costs of nonclinical or clinical trials may exceed anticipated amounts;
- we may not be able to obtain regulatory approval of any Drug Rescue Variant, other new drug candidate, biological candidate or regenerative medicine product candidate; or we may experience delays in obtaining any such approval;
- we may not be able to manufacture, or have manufactured for us, Drug Rescue Variants, other new drug candidates, biological candidates or regenerative medicine product candidates economically, timely and on a commercial scale;
- we and any licensees of ours may not be able to successfully market Drug Rescue Variants, other new drug candidates, biological candidates or regenerative medicine product candidates;
- physicians may not prescribe our products, or patients or third party payors may not accept our Drug Rescue Variants, other drug candidates, biological candidates or regenerative medicine product candidates;
- others may have proprietary rights which prevent us from marketing our Drug Rescue Variants, other new drug candidates, biological candidates or regenerative medicine product candidates; and
- competitors may sell similar, superior or lower-cost products.

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In the event we are able to begin a clinical trial of a Drug Rescue Variant, our or our collaborator's future clinical trials may be delayed or halted for many reasons, including:

- delays or failure reaching agreement on acceptable terms with prospective contract manufacturing organizations (CMOs), contract research organizations (CROs), and clinical trial sites, the terms of which can be subject to extensive negotiation and may vary significantly among different CROs and trial sites;

- failure of third-party contractors, such as CROs and CMOs, or investigators to comply with regulatory requirements or otherwise meet their contractual obligations in a timely manner;

- delays or failure in obtaining the necessary approvals from regulators or institutional review boards (IRBs) in order to commence a clinical trial at a prospective trial site;

- inability to manufacture, or obtain from third parties, a supply of drug product sufficient to complete preclinical studies and clinical trials;

- the FDA requiring alterations to study designs, preclinical strategy or manufacturing plans;

- delays in patient enrollment, and variability in the number and types of patients available for clinical trials, or high drop-out rates of patients;

- clinical trial sites deviating from trial protocols or dropping out of a trial and/or the inability to add new clinical trial sites;

- difficulty in maintaining contact with patients after treatment, resulting in incomplete data;

- poor effectiveness of our product candidates during clinical trials;

- safety issues, including serious adverse events associated with our product candidates and patients' exposure to unacceptable health risks;

- receipt by a competitor of marketing approval for a product targeting an indication that one of our product candidates targets, such that we are not "first to market" with our product candidate;

- governmental or regulatory delays and changes in regulatory requirements, policy and guidelines; or

- varying interpretations of data by the FDA and similar foreign regulatory agencies.

We or our collaborator could also encounter delays if a clinical trial is suspended or terminated by us, our collaborator, the IRBs of the institutions in which a trial is being conducted, by the Data Safety Monitoring Board (DSMB) for a trial, or by the FDA or other regulatory authorities. Such authorities may suspend or terminate a clinical trial due to a number of factors, including failure to conduct the clinical trial in accordance with regulatory requirements or clinical protocols, inspection of the clinical trial operations or trial site by the FDA or other regulatory authorities resulting in the imposition of a clinical hold, unforeseen safety issues or adverse side effects, failure to demonstrate a benefit from using a drug, changes in governmental regulations or administrative actions or lack of adequate funding to continue the clinical trial.

Moreover, if we or our collaborators are able to complete a clinical trial of a product candidate, the results of such trial may not be adequate to support marketing approval. For any such trial, if the FDA disagrees with the choice of

primary endpoint or the results for the primary endpoint are not robust or significant relative to control, are subject to confounding factors, or are not adequately supported by other study endpoints, including overall survival or complete response rate, the FDA may refuse to approve a Biologics License Application (BLA) or New Drug Application (NDA). The FDA may require additional clinical trials as a condition for approving our product candidates.

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Clinical testing involves the administration of the new drug or biological candidate to healthy human volunteers or to patients under the supervision of a qualified principal investigator, usually a physician, pursuant to an FDA-reviewed protocol. Each clinical study is conducted under the auspices of an institutional review board (IRB) at each of the institutions at which the study will be conducted. A clinical plan, or "protocol," accompanied by the approval of an IRB, must be submitted to the FDA as part of the IND application prior to commencement of each clinical trial. Human clinical trials are conducted typically in three sequential phases. Phase I trials primarily consist of testing the product's safety in a small number of patients or healthy volunteers. In Phase II trials, the safety and efficacy of the biological candidate is evaluated in a specific patient population. Phase III trials typically involve additional testing for safety and clinical efficacy in an expanded patient population at geographically dispersed sites. The FDA may order the temporary or permanent discontinuance of a nonclinical or clinical trial at any time for a variety of reasons, particularly if safety concerns exist.

Our or our collaborator's future clinical trials can be delayed or halted for many reasons, including:

- delays or failure reaching agreement on acceptable terms with prospective contract manufacturing organizations (CMOs), contract research organizations (CROs), and clinical trial sites, the terms of which can be subject to extensive negotiation and may vary significantly among different CROs and trial sites;
- failure of third-party contractors, such as CROs and CMOs, or investigators to comply with regulatory requirements or otherwise meet their contractual obligations in a timely manner;
- delays or failure in obtaining the necessary approvals from regulators or IRBs in order to commence a clinical trial at a prospective trial site;
- inability to manufacture, or obtain from third parties, a supply of drug product sufficient to complete preclinical studies and clinical trials;
- the FDA requiring alterations to study designs, preclinical strategy or manufacturing plans;
- delays in patient enrollment, and variability in the number and types of patients available for clinical trials, or high drop-out rates of patients;
- clinical trial sites deviating from trial protocols or dropping out of a trial and/or the inability to add new clinical trial sites;
- difficulty in maintaining contact with patients after treatment, resulting in incomplete data;
- poor effectiveness of our product candidates during clinical trials;
- safety issues, including serious adverse events associated with our product candidates and patients' exposure to unacceptable health risks;
- receipt by a competitor of marketing approval for a product targeting an indication that one of our product candidates targets, such that we are not "first to market" with our product candidate;
- governmental or regulatory delays and changes in regulatory requirements, policy and guidelines; or
- varying interpretations of data by the FDA and similar foreign regulatory agencies.

We or our collaborator could also encounter delays if a clinical trial is suspended or terminated by us, our collaborator, the IRBs of the institutions in which a trial is being conducted, by the Data Safety Monitoring Board (DSMB) for a trial, or by the FDA or other regulatory authorities. Such authorities may suspend or terminate a clinical trial due to a number of factors, including failure to conduct the clinical trial in accordance with regulatory requirements or clinical protocols, inspection of the clinical trial operations or trial site by the FDA or other regulatory authorities resulting in the imposition of a clinical hold, unforeseen safety issues or adverse side effects, failure to demonstrate a benefit from using a drug, changes in governmental regulations or administrative actions or lack of adequate funding to continue the clinical trial.

Moreover, if we or our collaborators are able to complete a clinical trial of a product candidate, the results of such trial may not be adequate to support marketing approval. For any such trial, if the FDA disagrees with the choice of primary endpoint or the results for the primary endpoint are not robust or significant relative to control, are subject to confounding factors, or are not adequately supported by other study endpoints, including overall survival or complete response rate, the FDA may refuse to approve a BLA or NDA. The FDA may require additional clinical trials as a condition for approving our product candidates.

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If we or our collaborator experience delays in the completion of, or termination of, any clinical trial of our product candidates, the commercial prospects of our product candidates will be harmed, and our ability to commence product sales and generate product revenues from any of our product candidates will be delayed. In addition, any delays in completing our clinical trials will increase our costs and slow our product candidate development and approval process. Delays in completing clinical trials could also allow our competitors to obtain marketing approval before we do or shorten the patent protection period during which we may have the exclusive right to commercialize our product candidates. Any of these occurrences may significantly harm our business, financial condition and prospects. In addition, many of the factors that cause, or lead to, a delay in the commencement or completion of clinical trials may also ultimately lead to the denial of regulatory approval of our product candidates.

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 product candidates may not be predictive of the results of later-stage clinical trials. 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.

This drug candidate development risk is heightened by any changes in planned 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 preclinical or clinical studies of our product candidates.

If we or our potential strategic partners experience delays in the enrollment of patients in clinical trials involving our product candidates, our receipt of necessary regulatory approvals could be delayed or prevented.

We or our potential strategic partners may not be able to initiate or continue clinical trials for our product candidates if we or they are unable to locate and enroll a sufficient number of eligible patients to participate in these trials as required by the FDA or other regulatory authorities. Patient enrollment, a significant factor in the timing of clinical trials, is affected by many factors including the size and nature of the patient population, the proximity of patients to clinical sites, the eligibility criteria for the trial, the design of the clinical trial, competing clinical trials and clinicians' and patients' perceptions as to the potential advantages of the drug being studied in relation to other available therapies, including any new drugs that may be approved for the indications we or our collaborators may be investigating. If we or they fail to enroll and maintain the number of patients for which the clinical trial was designed, the statistical power of that clinical trial may be reduced, which would make it harder to demonstrate that the product candidate being tested is safe and effective. Additionally, enrollment delays in clinical trials may result in increased development costs for our product candidates, which would cause the value of our common stock to decline and limit our ability to obtain additional financing. Our inability to enroll a sufficient number of patients for any of our current

or future clinical trials would result in significant delays or may require us to abandon one or more clinical trials, and, therefore, product candidates, altogether.

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Even if we receive regulatory approval for any of our Drug Rescue Variants or other product candidates, we and/or our potential strategic partners will be subject to ongoing FDA obligations and continued regulatory review, which may result in significant additional expense. Additionally, our product candidates, if approved, could be subject to labeling and other restrictions and market withdrawal and we may be subject to penalties if we fail to comply with regulatory requirements or experience unanticipated problems with our products.

Any regulatory approvals that we or our potential strategic partners receive for our Drug Rescue Variants or other product candidates may also be subject to limitations on the approved indicated uses for which the product may be marketed or to the conditions of approval, or contain requirements for potentially costly post-marketing testing, including Phase 4 clinical trials, and surveillance to monitor the safety and efficacy of the product candidate, all of which could adversely affect the product's commercial potential and our revenues. In addition, if the FDA approves any of our product candidates, the manufacturing processes, testing, packaging, labeling, storage, distribution, field alert or biological product deviation reporting, adverse event reporting, advertising, promotion and recordkeeping for the product will be subject to extensive and ongoing regulatory requirements. These requirements include submissions of safety and other post-marketing information and reports, as well as continued compliance with cGMP for commercial manufacturing and good clinical practices, or GCP, for any clinical trials that we conduct post-approval. Later discovery of previously unknown problems with a product, including adverse events of unanticipated severity or frequency, or with our third-party manufacturers or manufacturing processes, or failure to comply with regulatory requirements, may result in, among other things:

- restrictions on the marketing or manufacturing of the product, withdrawal of the product from the market, or voluntary or mandatory product recalls;
- warning letters or holds on clinical trials;
- refusal by the FDA to approve pending applications or supplements to approved applications filed by us or our strategic partners, or suspension or revocation of product license approvals;
- product seizure or detention, or refusal to permit the import or export of products; and
- injunctions, fines or the imposition of other civil or criminal penalties.

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Risks Related to Our Financial Position and Capital Requirements

We have incurred significant net losses since inception and anticipate that 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, including net losses of $3.0 million and $12.9 million during the fiscal years ending March 31, 2014 and 2013, respectively. As of March 31, 2014, we had an accumulated deficit of $70.6 million. We do not know whether or when we will become profitable. To date, although we have generated approximately $16.4 million in revenues, we have not commercialized any products or generated any revenues from product sales. Our losses have resulted principally from costs incurred in our research and development programs and from general and administrative expenses. We anticipate that our operating losses will substantially increase over the next several years as we execute our plan to expand our drug rescue, stem cell technology research and development, drug development and potential commercialization activities. Additionally, we expect that our general and administrative expenses will increase in the event we achieve our goal of obtaining a listing on a national securities exchange. The net losses we incur may fluctuate from quarter to quarter.

If we do not successfully develop, license, sell or obtain regulatory approval for our future product candidates and effectively manufacture, market and sell, or collaborate to accomplish such activities, any product candidates that are approved, we may never generate revenues from product sales, and even if we do generate product sales revenues, we may never achieve or sustain profitability on a quarterly or annual basis. Our failure to become and remain profitable would depress the market price of our common stock and could impair our ability to raise capital, expand our business, diversify our product offerings or continue our operations. A decline in the market price of our common stock also could cause you to lose all or a part of your investment.

We will require substantial additional financing to achieve our goals, and a failure to obtain this necessary capital when needed could force us to delay, limit, reduce or terminate our product development or commercialization efforts.

Since our inception, most of our resources have been dedicated to research and development of the drug rescue capabilities of our human pluripotent stem cell technology. In particular, we have expended substantial resources developing CardioSafe 3D and LiverSafe 3D, and we will continue to expend substantial resources for the foreseeable future developing LiverSafe 3D and CardioSafe 3D Drug Rescue Variants. These expenditures will include costs associated with general and administrative costs, facilities costs, research and development, acquiring new technologies, manufacturing product candidates, conducting preclinical experiments and clinical trials and obtaining regulatory approvals, as well as commercializing any products approved for sale. Furthermore, we expect to incur additional costs associated with operating as a public company.

We have no current source of revenue to sustain our present activities, and we do not expect to generate revenue until, and unless, we out-license a Drug Rescue Variant and/or AV-101 to a third party, 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 proposed drug rescue and AV-101 development 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, and may seek additional capital due to favorable market conditions or strategic considerations even if we believe we have sufficient funds for our current or future operating plans.

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Our future capital requirements depend on many factors, including:

- the number and characteristics of the product candidates we pursue, including Drug Rescue Candidates;
- 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 preparing, filing, prosecuting, maintaining, defending and enforcing patent claims necessary to preserve our freedom to operate in the stem cell industry, including litigation costs associated with any 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.

Additional funds may not be available when we need them, on terms that are acceptable to us, or at all. If adequate funds are not available to us on a timely basis, we may be required to delay, limit, reduce or terminate drug rescue programs, preclinical studies, clinical trials or other research and development activities for one or more of our product candidates, or cease or reduce our operating activities and/or sell or license to third parties some or all of our intellectual property, any of which could harm our operating results.

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

We will need to seek additional capital through a combination of private and public equity offerings, debt financings, strategic partnerships and alliances and licensing arrangements. To the extent that we raise additional capital through the sale of equity or convertible debt securities, the ownership interests of existing stockholders will be diluted, and the terms of the new capital may include liquidation or other preferences that adversely affect existing stockholder rights. Debt financing, if available, may involve agreements that include covenants limiting or restricting our ability to take certain actions, such as incurring debt, making capital expenditures or declaring dividends. If we raise additional funds through strategic partnerships and alliances and licensing arrangements with third parties, we may have to

relinquish valuable rights to our technologies or product candidates, or grant licenses on terms that are not favorable to us. If we are unable to raise additional funds through equity or debt financing when needed, we may be required to delay, limit, reduce or terminate our product development or commercialization efforts or grant rights to develop and market product candidates that we would otherwise prefer to develop and market ourselves.

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Some of our programs have been partially supported by government grants, 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 and the California Institute for Regenerative Medicine. 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.

Our independent auditors have expressed substantial doubt about our ability to continue as a going concern.

Our consolidated financial statements for the year ended March 31, 2014 included in Item 8 of this Annual Report on Form 10-K have been prepared assuming we will continue to operate as a going concern. However, due to our ongoing operating losses and our accumulated deficit, there is doubt about our ability to continue as a going concern. Because we continue to experience net operating losses, our ability to continue as a going concern is subject to our ability to generate a profit and/or obtain necessary funding from outside sources, including obtaining additional funding from the sale of our securities or obtaining loans and grants 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 its securities or from alternative sources, it may be required to reduce, defer, or discontinue certain of its research and development activities or it may not be able to continue as a going concern.

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

If we do not generate sufficient taxable income we may not be able to use a material portion, or any portion, of our existing net operating losses (NOLs). Furthermore, our existing NOLs may be subject to limitations under Section 382 of the Internal Revenue Code of 1986, as amended, which in general provides that a corporation that undergoes an "ownership change" is limited in its ability to utilize its pre- change NOLs to offset future taxable income. Our existing NOLs are subject to limitations arising from previous ownership changes, and if we undergo an ownership change, in connection with a future equity-based financing, series of equity-based financings or otherwise, our ability to utilize NOLs could be further limited by Section 382 of the Internal Revenue Code. Future changes in our stock ownership, some of which are outside of our control, could result in an ownership change under Section 382 of the Internal Revenue Code.

Risks Related to Intellectual Property

We utilize certain technologies that are licensed to us, including key aspects of our Human Clinical Trials in a Test Tube platform. If the licensors terminate the licenses or fail to maintain or enforce the underlying patents, our competitive position and market share will be harmed, and our business could be adversely affected.

We currently use certain licensed technologies to produce cells that are material to our research and development programs, including our drug rescue programs, and we may enter into additional license agreements in the future. Our

rights to use such licensed technologies are subject to the negotiation of, continuation of and compliance with the terms of the applicable licenses, including payment of any royalties and diligence, insurance, indemnification and other obligations. If a licensor believes that we have failed to meet our obligations under a license agreement for non-payment of license fees, non-reimbursement of patent expenses, or otherwise, the licensor could seek to limit or terminate our license rights, which could lead to costly and time-consuming litigation and, potentially, a loss of the licensed rights. During the period of any such litigation, our ability to carry out the development and commercialization of potential products could be significantly and negatively affected.

Our license rights are further subject to the validity of the owner's intellectual property rights. As such, we are dependent on our licensors to defend the viability of these patents and patent applications. We cannot be certain that drafting and/or prosecution of the licensed patents and patent applications by the licensors have been or will be conducted in compliance with applicable laws and regulations or will result in valid and enforceable patents and other intellectual property rights. Legal action could be initiated by or against the owners of the intellectual property that we license. Even if we are not a party to these legal actions, an adverse outcome could harm our business because it might prevent these other companies or institutions from continuing to license intellectual property that we may need to operate our business. In some cases, we do not control the prosecution, maintenance or filing of the patents to which we hold licenses, or the enforcement of these patents against third parties.

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Certain of our license agreements are subject to termination by the licensor in specific circumstances, including non-payment of license fees, royalties and patent-related expenses. Any such termination of these licenses could prevent us from producing cells for our research and development programs and future commercial activities, including selling or marketing products. Because of the complexity of our human pluripotent stem cell technology and the patents we have licensed, determining the scope of the license and related royalty obligation can be difficult and can lead to disputes between us and the licensor. An unfavorable resolution of such a dispute could lead to an increase in the royalties payable pursuant to the license. If a licensor believed we were not paying the royalties or other amounts due under the license or were otherwise not in compliance with the terms of the license, the licensor might attempt to revoke the license. If our license rights were restricted or ultimately lost, our ability to continue our business based on the affected technology would be severely adversely affected.

We may engage in discussions regarding possible commercial, licensing and cross-licensing agreements with third parties from time to time. There can be no assurance that these discussions will lead to the execution of commercial license or cross-license agreements or that such agreements will be on terms that are favorable to us. If these discussions are successful, we could be obligated to pay license fees and royalties to such third parties. If these discussions do not lead to the execution of mutually acceptable agreements, we may be limited or prevented from producing and selling our existing products and developing new products. One or more of the parties involved in such discussions could resort to litigation to protect or enforce its patents and proprietary rights or to determine the scope, coverage and validity of the proprietary rights of others. In addition, if we enter into cross-licensing agreements, there is no assurance that we will be able to effectively compete against others who are licensed under our patents.

If we seek to leverage prior discovery and development of Drug Rescue Candidates under in-license arrangements with academic laboratories, biotechnology companies, the NIH, pharmaceutical companies or other third parties, it is uncertain what ownership rights, if any, we will obtain over intellectual property we derive from such licenses to Drug Rescue Variants we may generate or develop in connection with any such third-party licenses.

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 Variants we may generate and develop. If we are unable to obtain ownership or substantial economic participation rights over intellectual property related to Drug Rescue Variants we generate, our business may be adversely affected.

Our ability to protect our intellectual property and proprietary technology through patents and other means is uncertain, and we could be unsuccessful in obtaining adequate patent protection for one or more of our product candidates.

Our commercial success will depend in part on our ability to protect our intellectual property and proprietary technologies. We rely on patents, where appropriate and available, as well as a combination of copyright, trade secret and trademark laws, license agreements and nondisclosure, confidentiality and other contractual restrictions to protect our proprietary technology. However, these legal means afford only limited protection and may not adequately protect our rights or permit us to gain or keep any competitive advantage. Pending patent applications of ours or our licensors may not issue as patents or may not issue in a form that will be sufficient to protect our proprietary technology and gain or maintain our competitive advantage. Any patents we have obtained or may obtain in the future, or the rights we have licensed, may be subject to re-examination, reissue, opposition or other administrative proceeding, or may be challenged in litigation, and such challenges could result in a determination that the patent is invalid or unenforceable. In addition, competitors may be able to design alternative methods or products that avoid infringement of these patents or technologies. To the extent our intellectual property, including licensed intellectual property, offers

inadequate protection, or is found to be invalid or unenforceable, we are exposed to a greater risk of direct competition. If our intellectual property does not provide adequate protection against our competitors' products, our competitive position could be adversely affected, as could our business. Both the patent application process and the process of managing patent disputes can be time consuming and expensive.

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The patent positions of companies in the life sciences industry can be highly uncertain and involve complex legal and factual questions for which important legal principles remain unresolved. A number of life sciences, biopharmaceutical and other companies, universities and research institutions have filed patent applications or have been issued patents relating to stem cells, use of stem cells and other modified cells to treat disease, disorder or injury, and other technologies potentially relevant to or required by our existing and planned products. We cannot be certain that patents we have filed or may file in the future will be issued or granted, or that issued or granted patents will not later be found to be invalid and/or unenforceable. The standards applied by the United States Patent and Trademark Office (US PTO) and foreign patent offices in granting patents are not always applied uniformly or predictably. For example, there is no uniform worldwide policy regarding patentable subject matter or the scope of claims allowable in biotechnology and pharmaceutical patents. Consequently, patents may not issue from our pending or future patent applications. As such, we do not know the degree of future protection that we will have on certain of our proprietary products and technology.

Our patents and patent applications may not be sufficient to protect our products, product candidates and technologies from commercial competition. Our inability to obtain adequate patent protection for our product candidates or platform technology could adversely affect our business.

Publication of discoveries in scientific or patent literature tends to lag behind actual discoveries by at least several months and sometimes several years. Therefore, the persons or entities that we or our licensors name as inventors in our patents and patent applications may not have been the first to invent the inventions disclosed in the patent applications or patents, or the first to file patent applications for these inventions. As a result, we may not be able to obtain patents for discoveries that we otherwise would consider patentable and that we consider to be extremely significant to our future success.

Where several parties seek U.S. patent protection for the same technology, the US PTO may declare an interference proceeding in order to ascertain the party to which the patent should be issued. Patent interferences are typically complex, highly contested legal proceedings, subject to appeal. They are usually expensive and prolonged, and can cause significant delay in the issuance of patents. Moreover, parties that receive an adverse decision in interference can lose patent rights. Our pending patent applications, or our issued patents, may be drawn into interference proceedings, which may delay or prevent the issuance of patents or result in the loss of issued patent rights. If more groups become engaged in scientific research related to hESCs, the number of patent filings by such groups and therefore the risk of our patents or applications being drawn into interference proceedings may increase. The interference process can also be used to challenge a patent that has been issued to another party.

Outside of the U.S., certain jurisdictions, such as Europe, Japan, New Zealand and Australia, permit oppositions to be filed against the granting of patents. Because we may seek to develop and commercialize our product candidates internationally, securing both proprietary protection and freedom to operate outside of the U.S. is important to our business. In addition, the European Patent Convention prohibits the granting of European patents for inventions that concern “uses of human embryos for industrial or commercial purposes”. The European Patent Office is presently interpreting this prohibition broadly, and is applying it to reject patent claims that pertain to hESCs. However, this broad interpretation is being challenged through the European Patent Office appeals system. As a result, we do not yet know whether or to what extent we will be able to obtain European patent protection for our proprietary hESC-based technology and systems.

Patent opposition proceedings are not currently available in the U.S. patent system, but legislation is pending to introduce them. However, issued U.S. patents can be re-examined by the US PTO at the request of a third party. Patents owned or licensed by us may therefore be subject to re-examination. As in any legal proceeding, the outcome of patent re-examinations is uncertain, and a decision adverse to our interests could result in the loss of valuable patent rights.

Successful challenges to our patents through interference, opposition or re-examination proceedings could result in a loss of patent rights in the relevant jurisdiction(s). As more groups become engaged in scientific research and product development areas of hESCs, the risk of our patents being challenged through patent interferences, oppositions, re-examinations or other means will likely increase. If we institute such proceedings against the patents of other parties and we are unsuccessful, we may be subject to litigation, or otherwise prevented from commercializing potential products in the relevant jurisdiction, or may be required to obtain licenses to those patents or develop or obtain alternative technologies, any of which could harm our business.

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Furthermore, if such challenges to our patent rights are not resolved promptly in our favor, our existing business relationships may be jeopardized and we could be delayed or prevented from entering into new collaborations or from commercializing certain products, which could materially harm our business.

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

If we were to initiate legal proceedings against a third party to enforce a patent covering one of our product candidates or technologies, the defendant could counterclaim that our patent is invalid and/or unenforceable. In patent litigation in the United States, defendant counterclaims alleging invalidity and/or unenforceability are commonplace. Grounds for a validity challenge could be an alleged failure to meet any of several statutory requirements, for example, lack of novelty, obviousness or non-enablement. Grounds for an unenforceability assertion could be an allegation that someone connected with prosecution of the patent withheld relevant information from the US PTO, or made a misleading statement, during prosecution. The outcome following legal assertions of invalidity and unenforceability during patent litigation is unpredictable. With respect to the patent validity, we cannot be certain, for example, 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 one or more of our products or certain aspects of our platform technology, Human Clinical Trials in a Test Tube. Such a loss of patent protection could have a material adverse impact on our business.

Claims that any of our product candidates, including our Human Clinical Trials in a Test Tube, or, if commercialized, the sale or use of our products infringe the patent rights of third parties could result in costly litigation or could require substantial time and money to resolve, even if litigation is avoided.

We cannot guarantee that our product candidates, the use of our product candidates, or our platform technology, do not or will not infringe third party patents. Third parties might allege that we are infringing their patent rights or that we have misappropriated their trade secrets. Such third parties might resort to litigation against us. The basis of such litigation could be existing patents or patents that issue in the future. Our failure to successfully defend against any claims that our product candidates or platform technology infringe the rights of third parties could also adversely affect our business. Failure to obtain any required licenses could restrict our ability to commercialize our products in certain territories or subject us to patent infringement litigation, which could result in us having to cease commercialization of our products and subject us to money damages in such territories.

It is also possible that we may fail to identify relevant patents or applications. For example, applications filed before November 29, 2000 and certain applications filed after that date that will not be filed outside the United States remain confidential until patents issue. Patent applications in the United States and elsewhere are published approximately 18 months after the earliest filing for which priority is claimed, with such earliest filing date being commonly referred to as the priority date. Therefore, patent applications covering our products or platform technology could have been filed by others without our knowledge. Additionally, pending patent applications which have been published can, subject to certain limitations, be later amended in a manner that could cover our platform technologies, our products or the use of our products.

To avoid or settle potential claims with respect to any patent rights of third parties, we may choose or be required to seek a license from a third party and be required to pay license fees or royalties or both. These licenses may not be available on acceptable terms, or at all. Even if we or any future strategic partners were able to obtain a license, the rights may be nonexclusive, which could result in our competitors gaining access to the same intellectual property. Ultimately, we could be prevented from commercializing one or more of our products, or be forced to cease some aspect of our business operations, if, as a result of actual or threatened patent infringement claims, we are unable to

enter into licenses on acceptable terms. This could harm our business significantly.

Defending against claims of patent infringement or misappropriation of trade secrets could be costly and time consuming, regardless of the outcome. Even if we were to ultimately prevail, or to settle at an early stage, such litigation could burden us with substantial unanticipated costs. In addition, litigation or threatened litigation could result in significant demands on the time and attention of our management team, distracting them from the pursuit of other business.

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Intellectual property litigation may lead to unfavorable publicity that harms our reputation, and could result in unfavorable outcomes that could limit our research and development activities and/or our ability to commercialize certain products.

During the course of any patent litigation, there could be public announcements of the results of hearings, rulings on motions, and other interim proceedings in the litigation. If securities analysts or investors regard these announcements as negative, the perceived value of our products, programs, or intellectual property could be diminished. Moreover, if third parties successfully assert intellectual property rights against us, we might be barred from using certain aspects of our platform technology, or barred from developing and commercializing certain products. Prohibitions against using certain technologies, or prohibitions against commercializing certain products, could be imposed by a court or by a settlement agreement between us and a plaintiff. In addition, if we are unsuccessful in defending against allegations of patent infringement or misappropriation of trade secrets, we may be forced to pay substantial damage awards to the plaintiff. There is inevitable uncertainty in any litigation, including intellectual property litigation. There can be no assurance that we would prevail in any intellectual property litigation, even if the case against us is weak or flawed. If litigation leads to an outcome unfavorable to us, we may be required to obtain a license from the patent owner to continue our research and development programs or to market our product(s). It is possible that the necessary license will not be available to us on commercially acceptable terms, or at all. This could limit our research and development activities, our ability to commercialize certain products, or both.

Most of our competitors are larger than we are and have substantially greater resources. They are, therefore, likely to be able to sustain the costs of complex patent litigation longer than we could. In addition, the uncertainties associated with litigation could have a material adverse effect on our ability to raise the funds necessary to continue our internal research programs, conduct clinical trials, continue to in-license needed technology, or enter into strategic partnerships that would help us bring our product candidates to market.

In addition, any future patent litigation, interference or other administrative proceedings will result in additional expense and distraction of our personnel. An adverse outcome in such litigation or proceedings may expose us or any future strategic partners to loss of our proprietary position, expose us to significant liabilities, or require us to seek licenses that may not be available on commercially acceptable terms, if at all.

Confidentiality agreements with employees and third parties may not prevent unauthorized disclosure of trade secrets and other proprietary information.

In addition to patents, we rely on trade secrets, technical know-how, and proprietary information concerning our business strategy in order to protect our competitive position in the field of stem cell research and product candidate development. In the course of our research and development activities and other business activities, we often rely on confidentiality agreements to protect our proprietary information. Such confidentiality agreements are used, for example, when we talk to vendors of laboratory or clinical development services or potential strategic partners. In addition, each of our employees is required to sign a confidentiality agreement upon joining the Company. We take steps to protect our proprietary information, and our confidentiality agreements are carefully drafted to protect our proprietary interests. Nevertheless, there can be no guarantee that an employee or an outside party will not make an unauthorized disclosure of our proprietary confidential information. This might happen intentionally or inadvertently. It is possible that a competitor will make use of such information, and that our competitive position will be compromised, in spite of any legal action we might take against persons making such unauthorized disclosures.

Trade secrets are difficult to protect. Although we use reasonable efforts to protect our trade secrets, our employees, consultants, contractors, or outside scientific collaborators might intentionally or inadvertently disclose our trade secret information to competitors. Enforcing a claim that a third party illegally obtained and is using any of our trade secrets is expensive and time consuming, and the outcome is unpredictable. In addition, courts outside the United

States sometimes are less willing than U.S. courts to protect trade secrets. Moreover, our competitors may independently develop equivalent knowledge, methods and know-how.

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Our research and development strategic partners may have rights to publish data and other information to which we have rights. In addition, we sometimes engage individuals or entities to conduct research relevant to our business. The ability of these individuals or entities to publish or otherwise publicly disclose data and other information generated during the course of their research is subject to certain contractual limitations. These contractual provisions may be insufficient or inadequate to protect our confidential information. If we do not apply for patent protection prior to such publication, or if we cannot otherwise maintain the confidentiality of our proprietary technology and other confidential information, then our ability to obtain patent protection or to protect our trade secret information may be jeopardized.

Intellectual property rights do not necessarily address all potential threats to our competitive advantage.

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, or permit us to maintain our competitive advantage. The following examples are illustrative:

- Others may be able to make compounds that are the same as or similar to our product candidates but that are not covered by the claims of the patents that we may own or have exclusively licensed;

- We or our licensors or any future strategic partners might not have been the first to make the inventions covered by the issued patent or pending patent application that we may own or have exclusively licensed;

- We or our licensors or any future strategic partners might not have been the first to file patent applications covering certain of our inventions;

- Others may independently develop similar or alternative technologies or duplicate any of our technologies without infringing our intellectual property rights;

- It is possible that our pending patent applications will not lead to issued patents;

- Issued patents that we own or have exclusively licensed may not provide us with any competitive advantages, or may be held invalid or unenforceable, as a result of legal challenges by our competitors;

- Our competitors might conduct research and development activities in countries where we do not have patent rights and then use the information learned from such activities to develop competitive products for sale in our major commercial markets;

- We may not develop additional proprietary technologies that are patentable; and

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

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 development stage biotechnology companies, our success is heavily dependent on intellectual property, particularly patents. Obtaining and enforcing patents in the biotechnology and pharmaceutical industries involve both technological and legal complexity. Therefore, obtaining and enforcing patents is costly, time-consuming and inherently uncertain. In addition, Congress has passed patent reform legislation which provides new limitations on attaining, maintaining and enforcing intellectual property. Further, the Supreme Court has ruled on several patent cases in recent years, either narrowing the scope of patent protection available in certain circumstances or weakening

the rights of patent owners in certain situations. In addition to increasing uncertainty with regard to our ability to obtain patents in the future, this combination of events has created uncertainty with respect to the value of patents, once obtained. Depending on decisions by the U.S. Congress, the federal courts, and the US PTO, the laws and regulations governing patents could change in unpredictable ways that would weaken our ability to obtain new patents or to enforce our existing patents and patents that we might obtain in the future.

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If we are not able to obtain and enforce patent protection or other commercial protection for AV-101, the value of AV-101 will be harmed.

Commercial protection of AV-101, our small molecule drug candidate for neuropathic pain and other neurological conditions is important to our business. Our success related to AV-101 will depend in part on our or a potential collaborator's ability to obtain and enforce potential patents and maintain our trade secrets and secure 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.

Additional patents may not be granted, and potential U.S. patents, if issued, might not provide us with commercial benefit or might be infringed upon, invalidated or circumvented by others. The principle U.S. method of use patent and its foreign counterparts for AV-101 have expired. Although we have recently filed three new U.S. patent applications relating to AV-101, we or others with whom we may collaborate for the development and commercialization of AV-101 may choose not to seek, or may be unable to obtain, patent protection in a country that could potentially be an important market for AV-101.

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

Our ability to execute on our business plan will depend on the talents and efforts of highly skilled individuals with specialized training in the field of stem cell research and bioassay development, as well as medicinal chemistry and in vitro drug candidate screening and nonclinical and clinical development. Our future success depends on our ability to identify, hire and retain these highly skilled personnel during our development stage. We may hire additional highly skilled scientific and technical employees, including employees who may have been previously employed at biopharmaceutical companies, including our competitors or potential competitors, and who may have executed invention assignments, nondisclosure agreements and/or non-competition agreements in connection with such previous employment. As to such future employees, we may become subject to claims that we, or these future employees, have inadvertently or otherwise used or disclosed trade secrets or other proprietary information of their former employers. Litigation may be necessary to defend against these claims. If we fail in defending such claims, in addition to paying monetary damages, we may lose valuable intellectual property or personnel. A loss of key research personnel or their work product could hamper or prevent our ability to commercialize certain potential products, which could severely harm our business. Even if we are successful in defending against these claims, litigation could result in substantial costs and be a distraction to management.

Risks Related to our Common Stock

There is no assurance that an active, liquid and orderly trading market will develop for our common stock or what the market price of our common stock will be and, as a result, it may be difficult for you to sell your shares of our common stock.

Since we became a publicly-traded company in May 2011, there has been a limited public market for shares of our common stock on the OTCQB Marketplaces (OTCQB). We do not yet meet the initial listing standards of the New York Stock Exchange, the NASDAQ Capital Market, or other similar national securities exchanges. Until our common stock is listed on a broader exchange, we anticipate that it will remain quoted on the OTCQB, another over-the-counter quotation system, or in the "pink sheets." In those venues, investors may find it difficult to obtain accurate quotations as to the market value of our common stock. In addition, if we fail to meet the criteria set forth in SEC regulations, various requirements would be imposed by law on broker-dealers who sell our securities to persons other than established customers and accredited investors. Consequently, such regulations may deter broker-dealers from recommending or selling our common stock, which may further affect liquidity. This could also make it more

difficult to raise additional capital.

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We cannot predict the extent to which investor interest in our company will lead to the development of a more active trading market on the OTCQB, whether we will meet the initial listing standards of the New York Stock Exchange, the NASDAQ Capital Market, or other similar national securities exchanges, or how liquid that market might become. If an active trading market does not develop, you may have difficulty selling any of the shares of our common stock that you buy. In addition, the trading price of our common stock may be highly volatile and could be subject to wide fluctuations in response to various factors, some of which are beyond our control. These factors include:

- actual or anticipated quarterly variation in our results of operations or the results of our competitors;
- announcements by us or our competitors of new commercial products, significant contracts, commercial relationships or capital commitments;
- financial projections we may provide to the public, any changes to those projections, or our failure to meet those projections;
- issuance of new or changed securities analysts' reports or recommendations for our stock;
- developments or disputes concerning our intellectual property or other proprietary rights;
- commencement of, or our involvement in, litigation;
- market conditions in the biopharmaceutical and life sciences sectors;
- failure to complete significant sales;
- changes in legislation and government regulation;
- public concern regarding the safety, efficacy or other aspects of our products;
- entering into, changing or terminating collaborative relationships;
- any shares of our common stock or other securities eligible for future sale;
- any major change to the composition of our board of directors or management; and
- general economic conditions and slow or negative growth of our markets.

The stock market in general, and biotechnology-based companies like ours in particular, has from time to time experienced 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 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. Prior to this date of this report, there has been a limited public market for shares of our common stock on the OTCQB. Future sales of substantial amounts of shares of our common stock, including shares issued upon the exchange of our Series A Preferred Stock, conversion of convertible promissory notes and exercise of outstanding options and warrants for common stock, in the public market, or the possibility of these sales occurring, could cause the prevailing market price for our common stock to fall or impair our ability to raise equity capital in the future.

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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 and their respective affiliates beneficially own approximately 46% of our outstanding capital stock, as beneficial ownership is defined by SEC rules and regulations. Accordingly, these stockholders may continue to have substantial influence over the outcome of corporate actions requiring stockholder approval, including the election of directors, 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. For information regarding the ownership of our outstanding stock by such stockholders, refer to Item 12, "Security Ownership of Certain Beneficial Owners and Management and Related Stockholder Matters" in this Annual Report on Form 10-K.

If securities or industry analysts do not publish research or publish inaccurate or unfavorable research about our business, our stock price and trading volume could decline.

The trading market for our common stock may depend in part on the research and reports that securities or industry analysts publish about us and our business. Securities and industry analysts do not currently, and may never, publish research on our company. If no or too few securities or industry analysts commence coverage of our company, the trading price for our stock would likely be negatively impacted. In the event securities or industry analysts initiate coverage, if one or more of the analysts who cover us downgrade our stock or publish inaccurate or unfavorable research about our business, our stock price would likely decline. In the event we obtain analyst coverage, we will not have any control of the analysts or the content and opinions included in their reports. If one or more equity research analysts cease coverage of our company or fail to publish reports on us regularly, demand for our stock could decrease, which in turn could cause our stock price or trading volume to decline.

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

Following approval by our stockholders in October 2011, our Articles of Incorporation permit us to issue up to 10.0 million shares of preferred stock and our Board has authorized the issuance of 500,000 shares of Series A Preferred, all of which shares are currently issued and outstanding. Our board of directors 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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Internal control over financial reporting is a process designed to provide reasonable assurance regarding the reliability of financial reporting and the preparation of financial statements in accordance with GAAP. Our management is currently required to assess the effectiveness of our controls and we are required to disclose changes made in our internal control over financial reporting on a quarterly basis. As a "smaller reporting company," however, our independent registered public accounting firm is not required to attest to the effectiveness of our internal control over financial reporting pursuant to Section 404 of the Sarbanes-Oxley Act of 2002. If we cannot continue to favorably assess the effectiveness of our internal control over financial reporting, or if our independent registered public accounting firm is unable to provide an unqualified attestation report on our internal controls whenever required in the future, investors could lose confidence in our financial information and the price of our common stock could decline. Additionally, should we cease to be a "smaller reporting company," we will incur additional expense and management effort to facilitate the required attestation of the effectiveness of our internal control over financial reporting by our independent registered public accounting firm.

Our common stock may be considered a "penny stock."

Since we became a publicly-traded company in May 2011, our common stock has traded on the OTCQB at a price of less than $5.00 per share. The SEC has adopted regulations which generally define a "penny stock" as an equity security that has a market price of less than $5.00 per share, subject to specific exemptions. To the extent that the market price of our common stock is less than $5.00 per share and, therefore, may be considered a "penny stock," brokers and dealers effecting transactions in our common stock must disclose certain information concerning the transaction, obtain a written agreement from the purchaser and determine that the purchaser is reasonably suitable to purchase the securities. These rules may restrict the ability of brokers or dealers to sell our common stock and may affect your ability to sell shares of our common stock. In addition, as long as our common stock remains quoted only on the OTCQB, investors may find it difficult to obtain accurate quotations of the stock, and may find few buyers to purchase such stock and few market makers to support its price.

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

We have paid no cash dividends on any of our classes of capital stock to date and currently intend to retain our future earnings, if any, to fund the development and growth of our business. As a result, capital appreciation, if any, of our common stock will be your sole source of gain for the foreseeable future. Any payment of cash dividends will depend upon our financial condition, contractual restrictions, financing agreement covenants, solvency tests imposed by corporate law, results of operations, anticipated cash requirements and other factors and will be at the discretion of our board of directors. Furthermore, we may incur indebtedness that may severely restrict or prohibit the payment of dividends.

Item 1B. Unresolved Staff Comments

The disclosures in this section are not required since we qualify as a smaller reporting company.

Item 2. Properties

Our principal executive offices and laboratories are located at 343 Allerton Avenue, South San Francisco, California 94080, where we occupy approximately 10,900 square feet of office and lab space under a lease expiring on July 31, 2017. We believe that our facilities are suitable and adequate for our current and foreseeable needs.

Item 3. Legal Proceeding

From time to time, we may become involved in claims and other legal matters arising in the ordinary course of business. We are not presently involved in any legal proceeding nor do we know of any legal proceeding which is threatened or contemplated.

Item 4. Mine Safety Disclosures

Not applicable.

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PART II

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

Market Information

On June 21, 2011 our common stock began trading on the OTC Marketplace (OTCQB), under the symbol "VSTA". There was no established trading market for our common stock prior to that date.

Shown below is the range of high and low sales prices for our common stock for the periods indicated as reported by the OTCQB. The market quotations reflect inter-dealer prices, without retail mark-up, mark-down or commissions and may not necessarily represent actual transactions.

	High	Low
Year Ending March 31, 2014		
First quarter ending June 30, 2013	$0.90	$0.60
Second quarter ending September 30, 2013	$0.89	$0.55
Third quarter ending December 31, 2013	$0.61	$0.26
Fourth quarter ending March 31, 2014	$0.50	$0.28
Year Ending March 31, 2013		
First quarter ending June 30, 2012	$2.80	$0.50
Second quarter ending September 30, 2012	$1.50	$0.51
Third quarter ending December 31, 2012	$0.95	$0.55
Fourth quarter ending March 31, 2013	$0.90	$0.60

On June 19, 2014 the closing price of our common stock on the OTCQB was $0.65 per share.

As of June 19, 2014, we had 25,451,877 shares of common stock outstanding and approximately 300 stockholders of record. On the same date, one stockholder held all 500,000 outstanding restricted shares of our Series A Preferred.

Dividend Policy

We have not paid any dividends in the past and we do not anticipate that we will pay dividends in the foreseeable future. Covenants in certain of our debt agreements prohibit us from paying dividends while the debt remains outstanding.

Issuer Purchase of Equity Securities

There were no repurchases of our common stock during the fiscal year ended March 31, 2014

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Recent Sales of Unregistered Securities

During the three years preceding the date of this report, we issued the following securities in private placement transactions which were not registered under the Securities Act of 1933, as amended (Securities Act) and that have not been previously reported in a Quarterly Report on Form 10-Q or a Current Report on Form 8-K:

2013/2014 Unit Private Placement

On March 11, 2014, we entered into a securities purchase agreement with an accredited investor pursuant to which we sold Units consisting of an aggregate of (i) a 10% convertible note in the face amount of $37,500 maturing on July 30, 2014 (2013/2014 Unit Note); (ii) 75,000 shares of our restricted common stock; and (iii) a warrant exercisable through July 30, 2016 to purchase 75,000 shares of our restricted common stock at an exercise price of $1.00 per share (Unit Warrant). We received cash proceeds of $37,500 which we used for general corporate purposes. The Unit Note and related accrued interest are convertible into shares of our restricted common stock at a conversion price of $0.50 per share at or prior to maturity at the option of the investor. The Units were offered and sold in a transaction exempt from registration under the Securities Act, in reliance on Section 4(2) thereof.

Securities Issued in Satisfaction of Technology License and Maintenance Fees and Patent Expenses

On April 10, 2014, we issued (i) a promissory note in the face amount of $300,000 due on the earlier of December 31, 2014 or the completion of a qualified financing, as defined, (ii) 300,000 restricted shares of our common stock and (iii) a warrant exercisable through March 31, 2019 to purchase 300,000 restricted shares of our common stock at an exercise price of $0.50 per share to Icahn School of Medicine at Mount Sinai in satisfaction of $288,400 of license maintenance fees and reimbursable patent prosecution costs. The securities were issued in a private placement transaction exempt from registration under the Securities Act, in reliance on Section 4(2) thereof.

Securities Issued for Consulting Services

On May 21, 2014, we issued to an accredited investor 200,000 restricted shares of our common stock as partial compensation under the terms of a strategic consulting agreement. The securities were issued in a private placement transaction exempt from registration under the Securities Act, in reliance on Section 4(2) thereof.

Item 6. Selected Financial Data

The disclosures in this section are not required since we qualify as a smaller reporting company.

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

Special Note Regarding Forward-Looking Statements

This Annual Report on Form 10-K includes forward-looking statements. All statements contained in this Annual Report on Form 10-K other than statements of historical fact, including statements regarding our future results of operations and financial position, our business strategy and plans, and our objectives for future operations, are forward- looking statements. The words "believe," "may," "estimate," "continue," "anticipate," "intend," "expect" and similar expressions are intended to identify forward-looking statements. 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 financial condition, results of operations, business strategy, short-term and long-term business operations and objectives, and financial needs. These forward-looking statements are subject to a number of risks, uncertainties and assumptions, including those described in the "Risk Factors" section. Moreover, we operate in a very competitive and rapidly changing environment. New risks emerge from time to time. It is not possible for our management to predict all risks, nor can we assess the impact of all factors on our business or the extent to which any factor, or combination of factors, may cause actual results to differ materially from those contained in any forward-looking statements we may make. In light of these risks, uncertainties and assumptions, the future events and trends discussed in this Annual Report on Form 10-K may not occur and actual results could differ materially and adversely from those anticipated or implied in the forward-looking statements.

You should not rely upon forward-looking statements as predictions of future events. The events and circumstances reflected in the forward-looking statements may not be achieved or occur. Although we believe that the expectations reflected in the forward-looking statements are reasonable, we cannot guarantee future results, levels of activity, performance or achievements. We are under no duty to update any of these forward-looking statements after the date of this Annual Report on Form 10-K or to conform these statements to actual results or revised expectations. If we do update one or more forward-looking statements, no inference should be drawn that we will make additional updates with respect to those or other forward- looking statements.

Business Overview

We are a stem cell company headquartered in South San Francisco, California and focused on drug rescue and regenerative medicine. We believe better cells lead to better medicine™ and that the key to making better cells is precisely controlling the differentiation of human pluripotent stem cells, which are the building blocks of all cells of the human body. For over 15 years, our stem cell research and development teams and collaborators have focused on controlling the differentiation of pluripotent stem cells to produce multiple types of mature, functional, adult human cells, with emphasis on human heart and liver cells for drug rescue applications.

Our Stem Cell Technology Platform - Human Clinical Trials in a Test Tube™

Our stem cell technology platform, which we refer to as Human Clinical Trials in a Test Tube, is based on a combination of proprietary and exclusively licensed technologies for controlling the differentiation of human pluripotent stem cells into multiple types of mature, functional, adult human cells that we use, or plan to develop, to reproduce complex human biology and disease. We are currently producing human heart cells and liver cells for our drug rescue applications. However, we also intend to advance, internally and through collaborative research projects, production of pluripotent stem cell-derived blood, bone, cartilage, and pancreatic beta-islet cells and explore ways to leverage our stem cell technology platform for regenerative medicine purposes. Our interest in the regenerative medicine arena is on developing novel human disease models for discovery of small molecule drugs and biologics that activate the endogenous growth and healing processes enabling the body to repair tissue damage caused by certain degenerative diseases.

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CardioSafe 3D™

Using mature cardiomyocytes (heart cells) differentiated from human pluripotent stem cells, we have developed CardioSafe 3D, as a novel, in vitro bioassay system used to assess new drug candidates for potential cardiac toxicity before they are tested in animals or humans. We believe CardioSafe 3D is capable of predicting the in vivo cardiac effects, both toxic and non-toxic, of small molecule drug candidates with greater speed and precision than the long-established, surrogate safety models most often used in drug development, including models using animal cells or live animals, and cellular assays using cadaver, immortalized or transformed cells. Our pluripotent stem cell derived cardiomyocytes (heart cells) and CardioSafe 3D are key components of our Human Clinical Trials in a Test Tube platform and drug rescue programs.

LiverSafe 3D™

Using mature, functional adult hepatocytes (liver cells) derived from human pluripotent stem cells, we are correlating LiverSafe 3D, our second novel stem cell technology-based bioassay system, with reported clinical results. We believe LiverSafe 3D will enable us to assess, early in development, new drug candidates for potential drug-induced liver toxicity and particularly metabolism issues that can result in serious adverse drug-drug interactions, before animal or human testing. We plan to use LiverSafe 3D, and the clinically predictive liver biology insight we believe it will provide us, to expand the scope of our commercial opportunities related to drug rescue.

Drug Rescue

We believe drug rescue using our novel in vitro bioassay systems, CardioSafe 3D and LiverSafe 3D, the foundation of our Human Clinical Trials in a Test Tube platform, is the highest-value near term commercial application of the human cells we produce. Detailed information is available to us in the public domain regarding the efficacy, pharmacology, formulation and toxicity of promising small molecule drug candidates developed by pharmaceutical and biotechnology companies which have failed due to unexpected heart or liver toxicity. These failed but still promising drug candidates, which we refer to as Drug Rescue Candidates™, have already been optimized and tested by a pharmaceutical or biotechnology company and assessed for efficacy and commercial potential.

Failure of promising Drug Rescue Candidates due to unexpected human clinical toxicity highlights the need for new paradigms to evaluate potential heart and liver toxicity early in drug development. While efforts of pharmaceutical and biotechnology companies to improve their prediction of such human clinical toxicity for new drug candidates is ongoing, the existence of Drug Rescue Candidates™ offers us an opportunity to use our novel stem cell technology to take advantage of prior third-party investment in Drug Rescue Candidates with early signs of efficacy, by significantly reducing the toxicity that caused them to be terminated, and bring new, safer versions back into development protected by new intellectual property. We refer to the new, safer versions of Drug Rescue Candidates we intend to produce with our medicinal chemistry collaborator and validate internally in our bioassay systems as Drug Rescue Variants™.

Through stem cell technology-based drug rescue, our objective is to become a leading source of proprietary, small molecule drug candidates to the global pharmaceutical industry. We have designed our drug rescue model to leverage publicly available information and substantial prior investment by pharmaceutical companies and others in Drug Rescue Candidates. The key commercial objective of our drug rescue model is to generate revenue from license, development and commercialization arrangements involving Drug Rescue Variants. We anticipate that each validated lead Drug Rescue Variant will be suitable as a promising new drug development program, either internally or in collaboration with a strategic partner.

Our Drug Rescue Strategy

We believe the pre-existing public domain knowledge base supporting the therapeutic and commercial potential of our Drug Rescue Candidates will provide us with a valuable head start as we launch our drug rescue programs. Leveraging the substantial prior investments by global pharmaceutical companies and others in discovery, optimization and efficacy validation of Drug Rescue Candidates is an essential component of our drug rescue strategy.

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Our current drug rescue emphasis is on Drug Rescue Candidates discontinued prior to FDA market approval due to unexpected cardiac safety concerns. By using our CardioSafe 3D assay platform to enhance our understanding of the cardiac liability profile of Drug Rescue Candidates, biological insight not previously available when the Drug Rescue Candidate was originally discovered and developed, we believe we can demonstrate in vitro proof-of-concept as to the efficacy and safety of Drug Rescue Variants earlier in development and with substantially less investment in discovery, efficacy optimization and development than was required of the pharmaceutical companies prior to their decision to terminate the Drug Rescue Candidates.

The key elements of our CardioSafe 3D drug rescue strategy are as follows:

- identify potential Drug Rescue Candidates with heart safety issues utilizing drug discovery and development information available in the public domain through open source, licensed databases, and published patents, as well as through our strategic relationships with our drug rescue and scientific advisors and consultants, including Synterys, Inc. and Cato Research Ltd., our preferred provider of contract medicinal chemistry and contract clinical development and regulatory services, respectively;

- leverage substantial prior research and development investments made by global pharmaceutical companies and others to analyse internally the therapeutic and commercial potential of Drug Rescue Candidates, as important criteria for selection of Drug Rescue Candidates and potential lead Drug Rescue Variants;

- use CardioSafe 3D to enhance our understanding of the cardiac liability profile of Drug Rescue Candidates, important and more comprehensive biological insights not available when the Drug Rescue Candidates were originally discovered and developed by pharmaceutical companies;

- leverage our internal knowledge base about each Drug Rescue Candidate's specific chemistry to design and produce a portfolio of novel potential lead Drug Rescue Variants for each Drug Rescue Candidate;

- use CardioSafe 3D and pre-existing in vitro efficacy models to assess the efficacy and cardiac safety of potential Drug Rescue Variants and identify and validate a lead Drug Rescue Variant; and

- internally develop validated lead Drug Rescue Variants or out-license them to a global pharmaceutical company in revenue-generating agreements providing for the full development, market approval and commercial sale.

We believe our exclusive focus on Drug Rescue Candidates with established therapeutic and commercial potential, and our ability to build on that valuable head start using our expertise in human biology, will help us to generate Drug Rescue Variants without incurring certain high costs and risks typically inherent in drug discovery and development. Although we plan to continue to identify Drug Rescue Candidates in the public domain, we may also seek to acquire rights to Drug Rescue Candidates not available to us in the public domain through in-licensing arrangements with third-parties.

Strategic Licensing of Drug Rescue Variants

We believe many pharmaceutical companies are experiencing, and will continue to experience, critical research and development productivity issues, as measured by their lack of, or very low number of, FDA-approved products each year during the past decade. For example, in 2013, the U.S. pharmaceutical industry invested over $51 billion in research and development and the Center for Drug Evaluation and Research (CDER) of the FDA approved a total of only 39 novel drugs, known as New Molecular Entities (NMEs). In 2013, CDER approved only 27 NMEs, thirteen of which NME approvals (48%) were received by only five pharmaceutical companies, including Bayer (two), GlaxoSmithKline (four), Johnson & Johnson (three), Roche (two) and Takeda (two). Despite remarkable levels of

research and development investment by the global pharmaceutical industry as a whole, since 2003, the FDA has only approved an average of approximately 26 NMEs per year. In addition, we believe many pharmaceutical companies with established products that are no longer patent protected are also experiencing substantial market pressure from generic competition.

As a result of research and development productivity issues, diminishing product pipelines and generic competition, we believe there is and will continue to be a critical need among pharmaceutical companies to license or acquire the new, safer Drug Rescue Variants we are focused on developing, including companies that originally discovered, developed and ultimately discontinued the Drug Rescue Candidates we select for our drug rescue programs.

Once we achieve proof-of-concept (POC) in vitro as to the efficacy and safety of a lead Drug Rescue Variant, we intend to announce the results of our internal POC studies and, at that time, consider whether we will seek to license that Drug Rescue Variant to a pharmaceutical company, including the company that developed the Drug Rescue Candidate, or further develop it internally on our own. If we decide to license a lead Drug Rescue Variant to a pharmaceutical company, through a form of license arrangement we believe is generally accepted in the pharmaceutical industry, we anticipate that the pharmaceutical company will be responsible for all subsequent development, manufacturing, regulatory approval, marketing and sale of the Drug Rescue Variant and that we will receive licensing revenue through payments to us from the license upon signing the license agreement, achievement of development and regulatory milestones, and, if approved and marketed, upon commercial sales.

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Regenerative Medicine and Drug Discovery

Although we believe the best and most valuable near term commercial application of our stem cell technology platform, Human Clinical Trials in a Test Tube, is for small molecule drug rescue, we also believe stem cell technology-based regenerative medicine has the potential to transform healthcare in the U.S. over the next decade by altering the fundamental mechanisms of disease. We are interested in exploring ways to leverage our stem cell technology platform for regenerative medicine purposes, with emphasis on developing novel human disease models for discovery of small molecule drugs with regenerative and therapeutic potential. Our regenerative medicine focus will be based on 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, liver and insulin-producing pancreatic beta-islet cells, and our expertise in formulating customized biological assays with the cells we produce. Among our key objectives will be to explore regenerative medicine opportunities through pilot nonclinical proof-of-concept studies, after which we intend to assess any potential opportunities for further development and commercialization of therapeutically and commercially promising regenerative medicine programs and novel, customized, disease-specific biological assays, either on our own or with strategic partners.

AV-101 for Neuropathic Pain, Epilepsy and Depression

With $8.8 million of grant funding awarded from the U.S. National Institutes of Health, we have successfully completed Phase 1 development of AV-101. AV-101, also known as “L-4-chlorokynurenine” and “4-Cl-KYN”, is an orally available non-sedating, small molecule prodrug candidate aimed at the multi-billion dollar neurological disease and disorders market, including neuropathic pain, a serious and chronic condition causing pain after an injury or disease of the peripheral or central nervous system, epilepsy, depression and Parkinson’s disease. Our AV-101 IND application, on file with the FDA, covers clinical development for neuropathic pain. However, we believe the Phase 1 AV-101 safety studies completed to date will support development of AV-101 for multiple indications, including epilepsy, depression and Parkinson’s disease. We intend to seek potential opportunities for further clinical development and commercialization of AV-101 for neuropathic pain, epilepsy, depression and Parkinson’s disease, on our own or with strategic partners. In the event that we successfully complete one or more strategic partnering arrangements for AV-101, we plan to use the net proceeds from such an arrangement(s) to expand our stem cell technology-based drug rescue and regenerative medicine programs.

The Merger

VistaGen Therapeutics, Inc., a California corporation incorporated on May 26, 1998 (VistaGen California), is our wholly-owned subsidiary. Excaliber Enterprises, Ltd. (Excaliber), a publicly-held company (formerly OTCBB: EXCA) was incorporated under the laws of the State of Nevada on October 6, 2005. Pursuant to a strategic merger transaction on May 11, 2011, Excaliber acquired all outstanding shares of VistaGen California in exchange for 6,836,452 shares of our common stock and assumed all of VistaGen California’s pre-Merger obligations (the Merger). Shortly after the Merger, Excaliber’s name was changed to “VistaGen Therapeutics, Inc.” (a Nevada corporation).

VistaGen California, as the accounting acquirer in the Merger, recorded the Merger as the issuance of common stock for the net monetary assets of Excaliber, accompanied by a recapitalization. The accounting treatment for the Merger was identical to that resulting from a reverse acquisition, except that we recorded no goodwill or other intangible assets. A total of 1,569,000 shares of our common stock, representing the shares held by stockholders of Excaliber immediately prior to the Merger and effected for a post-Merger two-for-one (2:1) stock split, have been retroactively reflected as outstanding for all periods presented in the Consolidated Financial Statements of the Company included in Item 8 of this Annual Report on Form 10-K. Additionally, the Consolidated Balance Sheets retroactively reflect the $0.001 par value of Excaliber’s common stock.

In October 2011, our stockholders amended our Articles of Incorporation to authorize us to issue up to 200 million shares of common stock and up to 10 million shares of preferred stock and to authorize our Board of Directors to prescribe the classes, series and the number of each class or series of preferred stock and the voting powers, designations, preferences, limitations, restrictions and relative rights of each class or series of preferred stock. In December 2011, our Board of Directors authorized the creation of a series of up to 500,000 shares of Series A Preferred Stock, par value $0.001 (Series A Preferred). Pursuant to the Note Exchange and Purchase Agreement of October 11, 2012 (the October 2012 Agreement), as amended, between us and Platinum Long Term Growth VII, LLC (Platinum), currently our largest institutional security holder, Platinum has the right and option to exchange the 500,000 shares of our Series A Preferred Stock that it holds for (i) 15,000,000 restricted shares of our common stock, and (ii) a five-year warrant to purchase 7,500,000 restricted shares of our common stock at an exercise price of $0.50 per share.

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The Consolidated Financial Statements included in Item 8 of this Annual Report on Form 10-K represent the activity of VistaGen California from May 26, 1998, and the consolidated activity of VistaGen California and Excaliber (now VistaGen Therapeutics, Inc., a Nevada corporation), from May 11, 2011 (the date of the Merger). The Consolidated Financial Statements also include the accounts of VistaGen California's two wholly-owned subsidiaries, Artemis Neuroscience, Inc., a Maryland corporation (Artemis), and VistaStem Canada, Inc., a corporation organized under the laws of Ontario, Canada (VistaStem Canada).

Financial Operations Overview

Net Loss

We are in the development stage and, since inception, have devoted substantially all of our time and efforts to hPSC research and bioassay development, small molecule drug development, creating, protecting and patenting intellectual property, recruiting personnel and raising working capital. As of March 31, 2014, we had an accumulated deficit of $70.6 million. Our net loss for the years ended March 31, 2014 and 2013 was $3.0 million and $12.9 million, respectively. We expect these conditions to continue for the foreseeable future as we expand our drug rescue activities and the capabilities of our Human Clinical Trials in a Test Tube™ platform.

Summary of Fiscal Year 2014

During fiscal 2014, our scientific personnel have continued to expand the drug rescue capabilities of CardioSafe 3D and further develop LiverSafe 3D. Our internal scientific operations were curtailed somewhat during our second fiscal quarter as we decommissioned our former lab space in preparation for the move to expanded lab and office facilities at the end of July 2013, and completed the corresponding relocation, recalibration and recertification of our laboratories and equipment following the move. Limited cash resources following the move, resulting in part from the failure to close the financing described below, continued to restrict certain scientific activities and collaborations for the remainder of the fiscal year. Nevertheless, we have continued to advance the capabilities of our heart and liver cells and pursue our internal evaluation of prospective drug rescue candidates. We successfully completed Phase 1 clinical development of AV-101 during our fiscal year ended March 31, 2013 and directed effort during the first quarter of fiscal 2014 to finalizing AV-101 Phase 1b clinical study reports, as required under the terms of our NIH grant awards and to facilitate further collaborative development of AV-101.

Throughout fiscal 2014, our executive management has been significantly focused on providing sufficient operating capital to advance our research and development objectives while meeting our continuing operational needs. To that end, in April 2013, we entered into a Securities Purchase Agreement (as amended, Securities Purchase Agreement) with Autilion AG, a company organized and existing under the laws of Switzerland (Autilion), under which Autilion is contractually obligated to purchase an aggregate of 72.0 million restricted shares of our common stock at a purchase price of $0.50 per share for aggregate cash proceeds to us of $36.0 million (the Autilion Financing). To date, Autilion has completed only a nominal closing under the Securities Purchase Agreement. Therefore, Autilion is in default under the Securities Purchase Agreement and we can give no assurance that Autilion will complete a material closing under the Securities Purchase Agreement.

To meet our working capital needs as a result of Autilion's default under the Securities Purchase Agreement, during June and July 2013, we offered certain warrant holders the opportunity to exercise outstanding warrants having an exercise price of $1.50 per share to purchase shares of our restricted common stock at a reduced exercise price of $0.50 per share. Participating warrant holders exercised modified warrants to purchase an aggregate of 528,370 restricted shares of our common stock and we received cash proceeds of $264,200. In addition, certain long-term warrant holders exercised modified warrants to purchase 16,646 restricted shares of our common stock in lieu of payment by us in satisfaction of amounts due for professional services in the aggregate amount of $8,300.

Additionally, in July 2013, we issued to Platinum a senior secured convertible note in the face amount of $250,000 (the July 2013 Note) and a five-year warrant to purchase 250,000 restricted shares of our common stock at an exercise price of $0.50 per share. Between August 2013 and March 14, 2014, we entered into securities purchase agreements with certain accredited investors pursuant to which we sold units of our securities (Units) consisting, in aggregate, of: (i) 10% convertible notes maturing on July 30, 2014 in the aggregate face amount of $1,007,500; (ii) an aggregate of 2,015,000 restricted shares of our common stock; and (iii) warrants exercisable through July 30, 2016 to purchase an aggregate of 2,015,000 restricted shares of our common stock at an exercise price of $1.00 per share. We received cash proceeds of $1,007,500 from the sale of the Units, including $50,000 in lieu of repayment of previous advances made to us by one of our executive officers.

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Between late-March 2014 and the date of this report, we have entered into subscription agreements with accredited investors, including Platinum, which has purchased $750,000 of such securities through June 19, 2014, pursuant to which we sold Units of our securities consisting, in aggregate, of: (i) 10% convertible notes maturing on March 31, 2015 in the aggregate face amount of $1,515,000; (ii) an aggregate of 1,515,000 restricted shares of our common stock; and (iii) warrants exercisable through December 31, 2016 to purchase an aggregate of 1,515,000 restricted shares of our common stock at an exercise price of $0.50 per share.

Given our working capital constraints during fiscal 2014, we attempted to minimize cash commitments and expenditures for external research and development and general and administrative services to the greatest extent possible, particularly during the later portion of the fiscal year. The following table summarizes the results of our operations for the fiscal years ended March 31, 2014 and 2013 (amounts in $000):

	Fiscal Years Ended March 31,	
	2014	2013
Revenues:		
Grant revenue	$-	$200
Operating expenses:		
Research and development	2,481	3,431
General and administrative	2,548	3,562
Total operating expenses	5,029	6,993
Loss from operations	(5,029)	(6,793)
Other expenses, net:		
Interest expense, net	(1,503)	(921)
Change in warrant liabilities	3,567	(1,636)
Loss on early extinguishment of debt	-	(3,568)
Other income	-	35
Loss before income taxes	(2,965)	(12,883)
Income taxes	(3)	(4)
Net loss	$(2,968)	$(12,887)
Deemed dividend on Series A Preferred Stock	-	(10,193)
Net loss attributable to common stockholders	$(2,968)	$(23,080)

Revenue

We have successfully completed our Phase I development of AV-101, our pro-drug candidate for the treatment of neuropathic pain and, potentially, depression and other neurological conditions. Our NIH grant related to AV-101 expired in its normal course on June 30, 2012. We had drawn the maximum amount available under the grant prior to its expiration. Revenue associated with our earlier subcontract research arrangement terminated in May 2012. We had no other grant or contract revenue sources during the fiscal year ended March 31, 2014.

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Research and Development Expense

Research and development expense represented approximately 49% of our operating expenses for each of the years ended March 31, 2014 and 2013. Research and development costs are expensed as incurred. Research and development expense consists of both internal and external expenses incurred in sponsored stem cell research and drug development activities, costs associated with the development of AV-101 and costs related to the licensing, application and prosecution of our intellectual property. These expenses primarily consist of the following:

- salaries, benefits, including stock-based compensation costs, travel and related expense for personnel associated with research and development activities;
- fees to contract research organizations and other professional service providers for services related to the conduct and analysis of clinical trials and other development activities;
- fees to third parties for access to licensed technology and costs associated with securing and maintaining patents related to our internally generated inventions:
- laboratory supplies and materials;
- leasing and depreciation of laboratory equipment; and
- allocated costs of facilities and infrastructure.

General and Administrative Expense

General and administrative expense consists primarily of salaries and related expense, including stock-based compensation expense, for personnel in executive, finance and accounting, and other support functions. Other costs include professional fees for legal, investor relations and accounting services and other strategic consulting and public company expenses as well as facility costs not otherwise included in research and development expense.

Other Expenses, Net

In both fiscal 2014 and 2013, we incurred interest expense, including discount amortization with respect to certain notes, on the outstanding balances of our Senior Secured Convertible Promissory Notes issued to Platinum during fiscal 2013 and in July 2013, on the new and modified notes issued to Morrison &Foerster, Cato Research Ltd. and University Health Network during August and September 2012, and on various notes issued to certain service providers during fiscal years 2011 and 2012. Additionally, in fiscal 2014, we incurred interest expense and related discount amortization attributable to the convertible notes issued in connection with the sale of Units between August 2013 and March 2014. In fiscal 2013, we incurred non-cash losses on extinguishment of debt resulting from the modification of indebtedness to Platinum, Morrison & Foerster, Cato Research Ltd., and University Health Network, as well as the conversion by the holders of our 12% Convertible Promissory Notes issued in February 2012 into restricted shares of our common stock and warrants in November 2012. In fiscal 2014 and 2013, we recorded income and expense, respectively, related to the changes in the fair values of the warrants issued or issuable in connection with the various Senior Secured Convertible Promissory Notes issued to Platinum during fiscal 2013 and 2014.

In fiscal 2013, we also recorded a non-cash deemed dividend related to the modification of the exchange rights of our Series A Preferred Stock held by Platinum and the impact of the prospective issuance of a five-year warrant to purchase restricted shares of our common stock upon Platinum’s exercise of its Series A Preferred Stock exchange

rights.

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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, 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.

Revenue Recognition

Although we do not currently have any such arrangements, we have historically generated revenue principally from collaborative research and development arrangements, 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 milestone and future product 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.

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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 statements of operations.

Research and Development Expenses

Research and development expenses include 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 of sponsored stem cell research and development costs, costs associated with clinical and non-clinical development of AV-101, our lead drug candidate, and costs related to application and prosecution of patents related to our stem cell technology platform, Human Clinical Trials in a Test Tube™, and AV-101. All such costs are charged to expense as incurred.

Stock-Based Compensation

We account for stock-based payment arrangements in accordance with ASC 718, Compensation-Stock Compensation and ASC 505-50, Equity-Equity Based Payments to Non-Employees which requires the recognition of compensation expense, using a fair-value based method, for all costs related to stock-based payments including stock options and restricted stock awards. We recognize compensation cost for all share-based awards to employees based on their grant date fair value. Share-based compensation expense is recognized over the period during which the employee is required to perform service in exchange for the award, which generally represents the scheduled vesting period. We have no 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.

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 terms 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, 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.

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

See Note 3 to the consolidated financial statements included in Item 8 in this Annual Report on Form 10-K for information on recent accounting pronouncements.

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Results of Operations

Comparison of Years Ended March 31, 2014 and 2013

Revenue

The following table compares our primary revenue sources between the periods (in $000):

	Fiscal Years Ended March 31,	
	2014	2013
NIH - AV-101 grant	$-	$187
Subcontract revenue	-	13
Total Revenue	$-	$200

We have successfully completed our Phase I development of AV-101, our prodrug candidate for the treatment of neuropathic pain and, potentially, depression and other neurological conditions. Our NIH grant related to AV-101 expired in its normal course on June 30, 2012. We had drawn the maximum amount available under the grant prior to its expiration. Revenue associated with our earlier subcontract research arrangement terminated in May 2012.

Research and Development Expense

Research and development expense decreased by 28% to $2.5 million in fiscal 2014 compared to $3.4 million in fiscal 2013. The following table compares the primary components of research and development expense between the periods (in $000):

	Fiscal Years Ended March 31,	
	2014	2013
Salaries and benefits	$902	$792
Stock-based compensation	453	510
UHN research under SRCA	160	466
Consulting services	53	14
Technology licenses and royalties	484	136
Project-related third-party research and supplies:		
AV-101	51	1,079
All other including CardioSafe and LiverSafe	145	293
	196	1,372
Rent	185	115
Depreciation	44	26
All other	4	-
Total Research and Development Expense	$2,481	$3,431

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The increase in research and development salaries and benefits expense reflects the impact of (i) the addition of a research technician in April 2013; (ii) the partial restoration in April 2013 of an earlier voluntary salary reduction to below his contractual pay rates taken by our President and Chief Scientific Officer; and (iii) general annual increases in employee benefits costs. In addition to the ratable amortization of stock-based compensation expense over the requisite service period of the respective grants made in fiscal 2014 and in prior years, stock-based compensation expense for fiscal 2014 includes approximately $82,000 as the impact of October 2013 and December 2013 modifications to reduce the exercise price of certain outstanding option grants to $0.40 per share or $0.50 per share, as well as approximately $157,000 attributable to the expense resulting from the March 2014 and March 2013 grants of warrants to our President and Chief Scientific Officer that vest over three years, subject to certain vesting acceleration events. Stock-based compensation expense for fiscal 2013 includes approximately $89,000 from the impact of October 2012 modifications reducing the exercise price to $0.75 per share and reducing any remaining vesting period to two years for certain option grants having exercise prices between $1.13 per share and $2.58 per share made to certain scientific employees and consultants in prior years and approximately $268,000 attributable to the expense resulting from the March 2013 grant of a warrant to our President and Chief Scientific Officer. Our 2012/2013 sponsored research project budget under the collaboration agreement with Dr. Gordon Keller's laboratory at UHN ended on September 30, 2013. We are currently in discussions with Dr. Keller and UHN regarding the scope of our 2013/2014 sponsored research project budget under the agreement, and we anticipate finalizing such budget in the near term. The expense recorded in fiscal 2013 reflects our stem cell research collaboration in accordance with our agreements with UHN made in the third and fourth quarters of our fiscal year ended March 31, 2012 and in a further modification effective beginning in October 2012. Technology license expense increased significantly in fiscal 2014 as a result of costs for patent prosecution and protection that we are required to fund under the terms of certain of our license agreements. 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. We began Phase 1b clinical trials of AV-101 early in calendar 2012, completing them by mid-year 2012. We recorded significant expense related to the trials during fiscal 2013. AV-101 expenses in fiscal 2014 reflect the costs associated with finalizing the AV-101 clinical trial results, preparing the final clinical trial and other reports required under the terms of the NIH grant and monitoring for feedback related to the reports, activities performed primarily through our contract research collaborator, Cato Research Ltd. We do not track internal research and development expenses, including compensation costs, by project as we do not currently believe that such project accounting is necessary given the level and overlap of project resources, including staffing, that are dedicated to our internal research and development projects. The increase in rent expense and depreciation in fiscal 2014 reflects increased rental costs and the amortization of tenant improvements related to our relocation to expanded facilities in late-July 2013.

General and Administrative Expense

General and administrative expense decreased by 29% to $2.5 million in fiscal 2014 compared to $3.6 million in fiscal 2013. The following table compares the primary components of general and administrative expense between the periods (in $000):

	Fiscal Years Ended March 31,	
	2014	2013
Salaries and benefits	$675	$617
Stock-based compensation	684	731
Consulting Services	94	157
Legal, accounting and other professional fees	340	554
Investor relations	120	622
Insurance	130	122

Travel and entertainment	18	37
Rent and utilities	139	85
Warrant modification expense	205	507
All other expenses	143	130
Total General and Administrative Expense	$2,548	$3,562

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The increase in administrative salaries and benefits expense reflects the impact of (i) the partial restoration in April 2013 of an earlier voluntary salary reduction to below his contractual pay rate taken by our Chief Executive Officer; (ii) the September 2012 conversion of our Chief Financial Officer from part-time consultant to full-time employee status; (iii) the April 2013 conversion of an administrative assistant from consultant to employee status, and (iv) general annual increases in employee benefits costs; all offset by the impact of voluntary resignations of certain administrative personnel. In addition to the ratable amortization of stock-based compensation expense over the requisite service period of the respective grants made in fiscal 2014 and in prior years, stock-based compensation expense for fiscal 2014 includes approximately $170,000 as the impact of October 2013 and December 2013 modifications to reduce the exercise price of certain outstanding option grants to $0.40 per share or $0.50 per share, as well as approximately $299,000 attributable to the expense resulting from the March 2014 and March 2013 grants of warrants vesting over three years, subject to certain vesting acceleration events, to certain members of our senior management and to the independent members of our Board of Directors. Stock-based compensation expense for fiscal 2013 includes approximately $44,000 reflecting the impact of October 2012 modifications reducing the exercise price to $0.75 per share and reducing any remaining vesting period to two years for certain option grants having exercise prices between $1.13 per share and $2.58 per share made to certain administrative employees and consultants in prior years, and approximately $535,000 attributable to the expense resulting from the March 2013 grants of warrants to certain members of our senior management and to the independent members of our Board of Directors. The reduction in legal, accounting and other professional fees reflects the impact of converting our Chief Financial Officer from part-time consultant to full-time employee status, as noted above, a reduction in legal expenses and the absence in fiscal 2014 of non-cash expense related to the granting of warrants to certain administrative consultants and service providers. During fiscal 2013, we engaged third parties to provide us with investor relations services and to conduct market awareness initiatives, however, for strategic purposes, we significantly scaled back those initiatives during fiscal 2014. The fiscal 2014 increase in rent and utilities expenses reflects higher costs related to our relocation to expanded facilities in late-July 2013. Warrant modification expense for fiscal 2014 reflects the impact of October 2013, December 2013 and February 2014 strategic reductions in the exercise price of certain outstanding warrants, generally from $1.75 per share or $1.50 per share, to $0.50 per share, and in certain cases, the extension of the term of outstanding warrants by approximately one year. In fiscal 2013, we recorded warrant modification expense also related to the reduction of the exercise price of certain outstanding warrants. The increase in other expenses for 2013 includes one-time costs associated with our late-July 2013 relocation to new facilities.

Other Expenses, Net

In both fiscal 2014 and 2013, other expenses, net includes interest expense, including non-cash discount amortization, on our outstanding promissory notes, net of interest income, as well as the non-cash impact of changes in the fair value of the warrant liabilities related to warrants issued or issuable to Platinum as a result of the October 2012 Agreement with Platinum, as amended, and, in fiscal 2014, the warrant issued to Platinum in July 2013. In fiscal 2013, other expenses, net additionally includes the non-cash loss on extinguishment of debt resulting from the modification of indebtedness to Platinum, Morrison & Foerster, Cato Research Ltd., and University Health Network, as well as the conversion by the holders of our 12% Convertible Promissory Notes issued in February 2012 into restricted shares of our common stock and warrants in November 2012.

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The following table compares the primary components of net interest expense between the periods (in $000):

	Fiscal Years Ended March 31,	
	2014	2013
Interest expense on promissory notes, including discount amortization	$1,547	$796
Charge for fair value of replacement warrants issued in connection with exercise of modified warrants	-	36
Charge related to losses on accounts payable settled by issuance of common stock or notes payable	-	80
Charge for investment banker warrants related to February 2012 Convertible promissory notes	-	28
Charge for legal fees related to issuance of Senior Secured Promissory Notes to Platinum under June and October 2012 agreements	-	59
Other interest expense, including on capital leases and premium financing	15	5
	1,562	1,004
Effect of foreign currency fluctuations on notes payable	(49)	(53)
Interest Income	(10)	(30)
Interest Expense, net	$1,503	$921

The increase in interest expense is primarily attributable to the accrued interest and discount amortization recorded for the July 2012 through July 2013 issuances and restructuring of an aggregate of $3.5 million of 10% senior secured convertible notes to Platinum, including the $250,000 convertible note issued in July 2013, as well as the restructuring in September and October 2012 of an additional $3.9 million of debt into new convertible notes to other service providers, including Morrison & Foerster, Cato Research Ltd., and University Health Network. These transactions are described in greater detail in Note 9, Convertible Promissory Notes and Other Notes Payable, in the Consolidated Financial Statements included in Item 8 of this Annual Report on Form 10-K.

In conjunction with the issuance to Platinum, pursuant to the October 2012 Note Exchange and Purchase Agreement, of certain Senior Secured Convertible Promissory Notes and the related Exchange Warrant and Investment Warrants in October 2012, February 2013 and March 2013, and in connection with the similar senior secured convertible promissory note and related warrant issued to Platinum in July 2013, (as described more completely in Note 9, Convertible Promissory Notes and Other Notes Payable, in the Consolidated Financial Statements included in Item 8 of this Annual Report on Form 10-K), and the contingent issuance of the Series A Exchange Warrant to Platinum upon Platinum's exchange of shares of our Series A Preferred Stock held by Platinum into shares of our common stock, we determined that the warrants included certain exercise price adjustment features requiring the warrants to be treated as liabilities. Accordingly, we recorded a non-cash warrant liability at its estimated fair value as of the date of warrant issuance or contract execution. During fiscal 2014, we recognized non-cash income of $3,566,900 related to the net decrease in the estimated fair value of these liabilities since March 31, 2013, or issuance in the case of the warrant issued in July 2013, which resulted from a combination of both the decrease in the market price of our common stock during that period and an agreement with Platinum in May 2013 pursuant to which the stated exercise price of the warrants was reduced from $1.50 per share to $0.50 per share, and (ii). During fiscal 2013, we recognized non-cash expense of $1,635,800 attributable to the net increase in the fair value of these liabilities between the issuance date of the warrants and March 31, 2013, primarily as the result of the increase in the market price of our common stock during that period.

During fiscal 2013, we recognized non-cash losses on the early extinguishment of debt in the aggregate amount of $3.6 million primarily as a result of the restructuring of notes payable to Platinum and Cato Holding Company, and the restructuring of accounts payable to Cato Research, Ltd. and University Health Network that were converted in to notes payable, as well as upon the conversion by the holders of our 12% Convertible Promissory Notes issued in February 2012 into restricted shares of our common stock and warrants, all of which were treated as extinguishment of debt for accounting purposes, all as described more completely in Note 9, Convertible Promissory Notes and Other Notes Payable, in the Consolidated Financial Statements included in Item 8 of this Annual Report on Form 10-K.

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In fiscal 2013, in connection with the October 2012 Note Exchange and Purchase Agreement we entered with Platinum, as described in Note 9, Convertible Promissory Notes and Other Notes Payable, and Note 10, Capital Stock, in the Consolidated Financial Statements included in Item 8 of this Annual Report on Form 10-K, we recorded a non-cash deemed dividend of $10.2 million as a result of the modification of the exchange rights for the Series A Preferred Stock held by Platinum and the related contingent issuance of a five-year warrant to purchase shares of our common stock upon Platinum's exercise of its Series A Preferred Stock exchange rights.

Liquidity and Capital Resources

Since our inception in May 1998 through March 31, 2014, we have financed our operations and technology acquisitions primarily through the issuance and sale of equity and debt securities, including convertible promissory notes and short-term promissory notes, for aggregate cash proceeds of approximately $26.0 million, as well as from an aggregate of approximately $16.4 million of government research grant awards, strategic collaboration payments and other revenues. Additionally, we have issued equity securities with an approximate aggregate value at issuance of $12.6 million, primarily as compensation for professional services rendered to us since inception. At March 31, 2014, we had negligible cash and cash equivalents. To meet our cash needs and fund our working capital requirements after March 31, 2014 and prior to the expected completion of the Autilion Financing (described below) or an alternate debt- or equity-based financing, through June 19, 2014, we entered into securities purchase agreements with accredited investors pursuant to which we sold to such accredited investors certain Units for aggregate cash proceeds of $1,465,000, consisting of: (i) 10% subordinate convertible promissory notes in the aggregate face amount of $1,465,000 maturing on March 31, 2015; (ii) an aggregate of 1,465,000 restricted shares of our common stock; and (iii) warrants exercisable through December 31, 2016 to purchase an aggregate of 1,465,000 restricted shares of our common stock at an exercise price of $0.50 per share. We anticipate that our cash expenditures during the next twelve months will be approximately $4.0 to $6.0 million.

In April 2013, we entered into the Securities Purchase Agreement with Autilion, under which Autilion is contractually obligated to purchase an aggregate of 72.0 million restricted shares of our common stock at a purchase price of $0.50 per share for aggregate cash proceeds to us of $36.0 million. To date, Autilion has completed only a nominal closing under the Securities Purchase Agreement. Therefore, Autilion is in default under the Securities Purchase Agreement, and we can provide no assurance that Autilion will complete a material closing under the Securities Purchase Agreement. In the event that Autilion does not complete a material closing under the Securities Purchase Agreement in the near term, we will need to obtain from $4.0 million to $6.0 million from alternative financing sources to execute our current business plan. Substantial additional financing may not be available to us on a timely basis, on terms acceptable to us, or at all. In the event we are unable to obtain substantial additional financing on a timely basis, our business, financial condition, and results of operations may be harmed, the price of our stock may decline, and we may not be able to continue as a going concern.

In the event Autilion completes a closing under the Securities Purchase Agreement in an amount exceeding $13.0 million, and we issue to Autilion over 26 million shares of our restricted common stock in connection with such closing, Autilion will control in excess of 50% of our issued and outstanding common stock, resulting in a change in control of the Company. In addition, substantial dilution to existing stockholders will occur upon completion of a material portion of the Autilion Financing, or completion of an alternate equity-based financing.

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If and as necessary, we may seek to complete a combination of additional private placements or public offerings of our securities, which may include both debt and equity securities, stem cell technology-based research and development collaborations, stem cell technology and drug candidate license fees and government grant awards. Although we have been successful since May 1998 with raising sufficient capital for our operations, and we will continue to pursue additional financing opportunities as necessary to meet our business objectives, there can be no assurance that substantial additional capital will be available to us in sufficient amounts, on terms favorable to us, and without substantial dilution to our current stockholders, if at all. If we are unable to complete one or more private placements or public offerings, or otherwise obtain sufficient financing through strategic collaborations or government grant awards, we may be required to delay, scale back or discontinue certain drug rescue and/or research and development activities, and this may adversely affect our ability to continue as a going concern. If we obtain additional financing by selling our equity or debt securities, we anticipate that substantial dilution to our existing stockholders will result. Our future working capital requirements will depend on many factors, including, without limitation, the scope and nature of strategic opportunities related to our stem cell technology platform, including drug rescue and cell therapy research and development efforts, the success of such programs, our ability to obtain government grant awards and our ability to enter into strategic collaborations with institutions on terms acceptable to us. To further advance drug rescue applications of our stem cell technology platform, as well as support our operating activities, we plan to continue to carefully manage our monthly operating costs associated with salaries and benefits, regulatory and public company consulting, contract research and development, legal, accounting and other working capital costs.

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

	Fiscal Years Ended March 31,	
	2014	2013
Net cash used in operating activities	$(2,126)	$(3,463)
Net cash used in investing activities	(10)	(135)
Net cash provided by financing activities	1,498	4,155
Net increase (decrease) in cash and cash equivalents	(638)	557
Cash and cash equivalents at beginning of period	638	81
Cash and cash equivalents at end of period	$-	$638

Off-Balance Sheet Arrangements

Other than contractual obligations incurred in the normal course of business, we do not have any off-balance sheet financing arrangements or liabilities, guarantee contracts, retained or contingent interests in transferred assets or any obligation arising out of a material variable interest in an unconsolidated entity. VistaGen California has two inactive, wholly-owned subsidiaries, Artemis Neuroscience, Inc., a Maryland corporation, and VistaStem Canada, Inc., an Ontario corporation.

Item 7A. Quantitative and Qualitative Disclosures About Market Risk

The disclosures in this section are not required since we qualify as a smaller reporting company.

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Item 8. Financial Statements and Supplementary Data

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REPORT OF INDEPENDENT REGISTERED PUBLIC ACCOUNTING FIRM

To the Board of Directors and Stockholders
VistaGen Therapeutics, Inc.
(a development stage company)

We have audited the accompanying consolidated balance sheets of VistaGen Therapeutics, Inc. (a development stage company) as of March 31, 2014 and 2013 and the related consolidated statements of operations and comprehensive loss, cash flows, preferred stock, and stockholders' deficit for the years then ended, and for the period from May 26, 1998 (inception) through March 31, 2014. 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. We were not engaged to perform an audit of the Company's 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, and 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. (a development stage company) at March 31, 2014 and 2013, and the consolidated results of its operations and its cash flows for the years then ended, and for the period from May 26, 1998 (inception) through March 31, 2014, 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 is a development stage company, has not yet generated sustainable revenues, has suffered recurring losses from operations and has a stockholders' deficit, 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 23, 2014

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VISTAGEN THERAPEUTICS, INC.

(a development stage company)

CONSOLIDATED BALANCE SHEETS

(Amounts in dollars, except share amounts)

	March 31, 2014	March 31, 2013
ASSETS		
Current assets:		
Cash and cash equivalents	$-	$638,100
Prepaid expenses and other current assets	40,500	33,700
Total current assets	40,500	671,800
Property and equipment, net	176,300	180,700
Security deposits and other assets	46,900	29,000
Total assets	$263,700	$881,500
LIABILITIES AND STOCKHOLDERS' DEFICIT		
Current liabilities:		
Accounts payable	$2,443,900	$1,353,600
Accrued expenses	625,600	342,900
Advance from officer	3,600	-
Current portion of notes payable and accrued interest	1,442,300	617,200
Current portion of notes payable to related parties and accrued interest	290,400	93,000
Convertible promissory notes and accrued interest, net of discount of $697,400 at March 31, 2014	396,000	-
Capital lease obligations	3,900	7,600
Total current liabilities	5,205,700	2,414,300
Non-current liabilities:		
Senior secured convertible promissory notes, net of discount of $2,085,900 at March 31, 2014 and $1,963,100 at March 31, 2013 and accrued interest	1,929,800	1,425,700
Notes payable, net of discount of $848,100 at March 31, 2014 and $1,142,600 at March 31, 2013 and accrued interest	1,797,600	2,091,800
Notes payable to related parties, net of discount of $103,200 at March 31, 2014 and $147,200 at March 31, 2013 and accrued interest	1,057,100	1,106,000
Warrant liability	2,973,900	6,394,000
Deferred rent liability	97,400	-
Capital lease obligations	2,100	6,100
Total non-current liabilities	7,857,900	11,023,600
Total liabilities	13,063,600	13,437,900
Commitments and contingencies		
Stockholders' deficit:		
Preferred stock, $0.001 par value; 10,000,000 shares, including 500,000 Series A shares, authorized at March 31, 2014 and 2013; 500,000 Series A shares issued and outstanding at March 31, 2014 and 2013	500	500
Common stock, $0.001 par value; 200,000,000 shares authorized at March 31, 2014 and 2013; 26,200,185 and 23,480,169 shares issued at March 31, 2014 and March 31, 2013, respectively	26,200	23,500
Additional paid-in capital	61,976,500	59,266,000
	(3,968,100)	(3,968,100)

Treasury stock, at cost, 2,713,308 shares of common stock held at March 31, 2014 and 2013		
Note receivable from sale of common stock	(198,100)	(209,100)
Deficit accumulated during development stage	(70,636,900)	(67,669,200)
Total stockholders' deficit	(12,799,900)	(12,556,400)
Total liabilities and stockholders' deficit	$263,700	$881,500

See accompanying notes to consolidated financial statements.

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VISTAGEN THERAPEUTICS, INC.
(a development stage company)
CONSOLIDATED STATEMENTS OF OPERATIONS AND COMPREHENSIVE LOSS
(Amounts in dollars, except share amounts)

	Fiscal Years Ended March 31,		May 26, 1998 (Inception) Through March 31,
	2014	2013	2014
Revenues:			
Grant revenue	$-	$200,400	$12,963,100
Collaboration revenue	-	-	2,283,600
Other	-	-	1,123,500
Total revenues	-	200,400	16,370,200
Operating expenses:			
Research and development	2,480,600	3,430,800	32,036,300
Acquired in-process research and development	-	-	7,523,200
General and administrative	2,548,300	3,562,700	33,229,400
Total operating expenses	5,028,900	6,993,500	72,788,900
Loss from operations	(5,028,900)	(6,793,100)	(56,418,700)
Other expenses, net:			
Interest expense, net	(1,503,000)	(920,700)	(11,865,200)
Change in warrant and put and note extension option liabilities	3,566,900	(1,635,800)	2,349,600
Loss on early extinguishment of debt	-	(3,567,800)	(4,761,300)
Other income	-	34,400	81,900
Loss before income taxes	(2,965,000)	(12,883,000)	(70,613,700)
Income taxes	(2,700)	(3,700)	(23,200)
Net loss	(2,967,700)	(12,886,700)	(70,636,900)
Deemed dividend on Series A Preferred stock	-	(10,193,200)	(10,193,200)
Net loss attributable to common stockholders	$(2,967,700)	$(23,079,900)	$(80,830,100)
Basic net loss attributable to common stockholders per common share	$(0.14)	$(1.27)	
Diluted net loss attributable to common stockholders per common share	$(0.19)	$(1.27)	
Weighted average shares used in computing:			
Basic net loss attributable to common stockholders per common share	21,973,149	18,108,444	
Diluted net loss attributable to common stockholders per common share	21,973,149	18,108,444	
Comprehensive loss	$(2,967,700)	$(12,886,700)	$(70,636,900)

See accompanying notes to consolidated financial statements.

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VISTAGEN THERAPEUTICS, INC.
(a development stage company)
CONSOLIDATED STATEMENTS OF CASH FLOWS
(Amounts in dollars)

	Fiscal Years Ended March 31, 2014	2013	Period From May 26, 1998 (Inception) Through March 31, 2014
Cash flows from operating activities:			
Net loss	$ (2,967,700)	$ (12,886,700)	$ (70,636,900)
Adjustments to reconcile net loss to net cash used in operating activities:			
Depreciation and amortization	54,600	33,800	832,100
Amortization of discounts on convertible and promissory notes	640,000	254,800	5,315,500
Change in warrant liability and put and note term extension option liabilities	(3,566,900)	1,635,800	(2,349,700)
Stock-based compensation	1,137,300	1,241,300	6,732,900
Expense related to modification of warrants	204,300	508,200	1,454,200
Non-cash rent and relocation expense	56,800	-	56,800
Interest income on note receivable for stock purchase	(1,200)	(27,600)	(28,800)
Fair value of common stock granted for services following the Merger	-	340,000	852,700
Fair value of warrants granted for services and interest following the Merger	60,700	183,800	748,300
Gain on currency fluctuation	(48,600)	(53,000)	(101,600)
Fair value of additional warrants granted pursuant to exercises of modified warrants	-	35,900	174,000
Loss on settlements of accounts payable	-	78,300	78,300
Acquired in-process research and development	-	-	7,523,200
Loss on early extinguishment of debt	-	3,567,800	4,761,300
Fair value of Series C preferred stock, common stock, and warrants granted for services prior to the Merger	-	-	3,150,900
Fair value of common stock issued for note term modification	-	-	22,400
Consulting services by related parties settled by issuing promissory notes	-	-	44,600
Gain on sale of assets	-	-	(16,800)
Changes in operating assets and liabilities:			
Unbilled contract payments receivable	-	106,200	-
Prepaid expenses and other current assets	92,700	46,200	134,400
Security deposits and other assets	(17,900)	-	(46,900)
Accounts payable and accrued expenses, including accrued interest	2,229,900	1,485,200	18,201,400
Deferred revenues	-	(13,200)	-
Net cash used in operating activities	(2,126,000)	(3,463,200)	(23,097,700)

Cash flows from investing activities:			
Purchases of equipment, net	(9,600)	(135,400)	(825,800)
Net cash used in investing activities	(9,600)	(135,400)	(825,800)
Cash flows from financing activities:			
Net proceeds from issuance of common stock and warrants, including Units	1,075,500	1,185,100	5,060,600
Proceeds from exercise of modified warrants	264,200	262,100	1,692,600
Net proceeds from issuance of Platinum notes and warrants	250,000	3,222,100	7,172,100
Advance from officer	64,000	-	64,000
Proceeds from issuance of notes under line of credit	-	-	200,000
Proceeds from issuance of 7% note payable to founding stockholder	-	-	90,000
Net proceeds from issuance of 7% convertible notes	-	-	575,000
Net proceeds from issuance of 10% convertible notes and warrants	-	-	1,655,000
Net proceeds from issuance of preferred stock and warrants	-	-	4,198,600
Net proceeds from issuance of notes and warrants from 2006 to 2010	-	-	4,851,800
Net proceeds from issuance of February 2012 12% convertible notes and warrants	-	-	466,500
Repayment of capital lease obligations	(7,600)	(16,900)	(125,000)
Repayment of notes	(148,600)	(496,700)	(1,977,700)
Net cash provided by financing activities	1,497,500	4,155,700	23,923,500
Net (decrease) increase in cash and cash equivalents	(638,100)	557,100	-
Cash and cash equivalents at beginning of period	638,100	81,000	-
Cash and cash equivalents at end of period	$ -	$ 638,100	$ -
Supplemental disclosure of cash flow activities:			
Cash paid for interest	$ 21,000	$ 225,900	$ 686,600
Cash paid for income taxes	$ 2,700	$ 3,700	$ 23,200

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VISTAGEN THERAPEUTICS, INC.
(a development stage company)
CONSOLIDATED STATEMENTS OF CASH FLOWS (continued)
(Amounts in dollars, except share amounts)

	Twelve Months Ended March 31,		Period From May 26, 1998 (Inception) Through March 31,
	2014	2013	2014
Supplemental disclosure of noncash activities:			
Forgiveness of accrued compensation and accrued interest payable to officers transferred to equity	$-	$-	$800,000
Exercise of warrants and options in exchange for debt cancellation	$-	$-	$112,800
Settlement of accrued and prepaid interest by issuance of Series C Preferred Stock	$-	$-	$35,300
Conversion of 10% notes payable, net of discount, and related accrued interest of $408,600 into Series C Preferred stock	$-	$-	$2,050,300
Issuance of Series B-1 Preferred stock for acquired in-process research and development	$-	$-	$7,523,200
Conversion of 7% notes payable, net of discount, and related accrued interest of $3,800 into Series B Preferred stock	$-	$-	$508,000
Conversion of accounts payable into convertible promissory notes	$-	$-	$893,700
Conversion of accounts payable into note payable	$-	$1,558,500	$4,368,800
Conversion of accounts payable into common stock	$-	$103,200	$1,927,300
Conversion of accrued interest on convertible promissory notes into common stock	$-	$-	$921,400
Notes receivable from sale of common stock to related parties upon exercise of options and warrants	$-	$-	$149,800
Capital lease obligations	$-	$-	$139,700
Recognition of put option and note term extension option liabilities upon issuance of Original Platinum Notes	$-	$-	$141,200
Incremental fair value of put option and note term extension option liabilities from debt modifications	$-	$-	$479,400
Incremental fair value of note conversion option from debt modification	$-	$-	$1,891,200
Incremental fair value of warrant from debt modifications	$-	$-	$276,700
Recognition of warrant liability upon adoption of new accounting standard	$-	$-	$151,300
Fair value of warrants issued with August 2010 short term notes	$-	$-	$130,900
Note discount upon issuance of August 2010 short term notes	$-	$-	$320,000
Fair value of warrants issued with February 2012 12 % convertible notes	$-	$-	$542,000
Note discount upon issuance of February 2012 12% convertible notes	$-	$-	$495,200
	$-	$-	$6,174,800

Conversion of 2006/2007 and 2008/2010 Notes into Units, including accrued interest of $1,365,600			
Conversion of all series of pre-Merger preferred stock into Units	$-	$-	$14,534,800
Conversion of 2011 Platinum Note into Series A Preferred Stock, including accrued interest of $611,100 and conversion premium	$-	$-	$5,763,900
Conversion of 7% note payable and accrued interest of $11,500 into common stock and warrants	$-	$-	$19,500
Conversion of accounts payable to Morrison & Foerster, McCarthy Tetrault and Desjardins into notes payable	$-	$-	$1,603,400
Accounts payable and cancellation premium converted into 2011 Private Placement Units	$-	$-	$169,000
Accrued interest on Cato Holding Company note converted to note payable	$-	$-	$90,800
Accounts payable settled in December 2011 and May/June 2012 warrant exercises	$-	$12,500	$280,100
Insurance premiums settled by issuing note payable	$98,300	$110,100	$296,900
Conversion of accrued interest and fees on February 2012 Notes into 2012 Private Placement Units	$-	$92,900	$92,900
Accrued interest on July and August 2012 Notes to Platinum converted into Exchange Note	$-	$22,600	$22,600
Accounts payable settled by issuance of stock or notes payable and stock	$-	$104,900	$104,900
Accounts payable converted into 2012 Private Placement Units	$-	$50,000	$50,000
Recognition of warrant liability upon issuance to Platinum of October 2012 Exchange Note and October 2012, February 2013 and March 2013 Investment Notes and July 2013 Convertible Note	$146,800	$1,690,000	$1,836,800
Recognition of warrant liability for potential issuance to Platinum of Series A Exchange Warrant under the terms of the October 2012 Agreement	$-	$3,068,200	$3,068,200

See accompanying notes to consolidated financial statements.

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VISTAGEN THERAPEUTICS, INC.
(a development stage company)
CONSOLIDATED STATEMENTS OF PREFERRED STOCK
Period from May 26, 1998 (inception) through March 31, 2014
(Amounts in dollars, except share amounts)

	Preferred Stock (Shares)	Series A Preferred Stock	Series B Preferred Stock	Series B-1 Preferred Stock	Series C Preferred Stock	Total Preferred Stock
Balances at May 26, 1998 (inception)	-	$-	$-	$-	$-	$-
Issuance of Series A preferred stock at $2.302 per share for cash, net of issuance costs of $29,500	431,930	964,700	-	-	-	964,700
Issuance of Series B preferred stock at $5.545 per share for cash, including conversion of $575,000 face value of 7% convertible notes plus accrued accrued interest of $3,800, net of unamortized note discount of $70,800 and issuance costs of $137,000	515,568	-	2,651,100	-	-	2,651,100
Issuance of Series B-1 preferred stock at $5.545 per share for acquired in-process research and development	1,356,750	-	-	7,523,200	-	7,523,200
Issuance of Series C preferred stock at $6.00 per share for cash, including conversion of $1,655,000 face value of 10% convertible notes plus accrued interest of $408,600, net of unamortized note discount of $13,200 and issuance costs of $47,900	533,658	-	-	-	3,140,800	3,140,800
Issuance of Series C preferred stock at $6.00 per share for services and in payment of interest on line of credit	46,749	-	-	-	280,500	280,500
Proceeds allocated to warrants issued in connection with Series C preferred stock	-	-	-	-	(25,500)	(25,500)

Balances at March 31, 2006 through March 31, 2011	2,884,655	964,700	2,651,100	7,523,200	3,395,800	14,534,800
Conversion of all series of VistaGen California preferred stock into common stock at May 11, 2011 in connection with the Merger	(2,884,655)	(964,700)	(2,651,100)	(7,523,200)	(3,395,800)	(14,534,800)
Balances at May 11, 2011 through March 31, 2014	-	$-	$-	$-	$-	$-

See accompanying notes to consolidated financial statements.

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VISTAGEN THERAPEUTICS, INC.

(a development stage company)

CONSOLIDATED STATEMENTS OF STOCKHOLDERS' DEFICIT

Period from May 26, 1998 (inception) through March 31, 2014

(Amounts in dollars, except share amounts)

	Series A Preferred Stock		Common Stock						
	Shares	Amount	Shares	Amount	Additional Paid-in Capital	Treasury Stock	Notes Receivable from Sale of Stock	Deficit Accumulated During the Development Stage	Total Stock Defic
Balances at May 26, 1998(inception)	-	$-	-	$-	$-	$-	$-	$-	$-
Sale of common stock for cash	-	-	1,211,086	1,200	24,900	-	-	-	26,1
Fair value of common stock issued for services	-	-	403,375	400	359,400	-	-	-	359
Fair value of warrants issued for services	-	-	-	-	481,700	-	-	-	481
Common stock issued upon exercise of options from 1999 and 2008 Stock Incentive Plans and SAB Plan	-	-	410,863	400	314,900	-	(149,800)	-	165
Common stock issued for cancellation of accounts payable and accrued interest (FY 2010)	-	-	1,646,792	1,600	2,468,600	-	-	-	2,47
Accrued interest on notes receivable	-	-	-	-	-	-	(34,300)	-	(34,
Proceeds allocated to warrants issued in connection with convertible and other notes issued in fiscal years 2001 through 2011, including Original Platinum Notes, and Series C preferred stock	-	-	-	-	1,059,100	-	-	-	1,05
Share-based compensation expense	-	-	-	-	2,763,000	-	-	-	2,76
	-	-	-	-	1,891,200	-	-	-	1,89

Incremental fair value of note conversion options from debt modification (FY 2010 and 2011)									
Forgiveness of accrued compensation and accrued interest payable to officers (FY 2007)	-	-	-	-	799,900	-	-	-	799
Effect of reverse stock split (FY 2009)	-	-	(6)	-	-	-	-	-	-
Effect of the Merger			1,569,000	1,600	(1,600)	-	-	-	-
Cumulative effect of adopting new accounting standard	-	-	-	-	(293,700)	-	-	142,300	(15
Net loss for fiscal years 1999 through 2011	-	-	-	-	-	-	-	(42,715,300)	(42,
Balances at March 31, 2011	-	$-	5,241,110	$5,200	$9,867,400	$-	$(184,100)	$(42,573,000)	$(32,
Share-based compensation expense	-	-	-	-	1,591,300	-	-	-	1,59
Accrued interest on notes receivable	-	-	-	-		-	(1,000)	-	(1,0
Reclassification of warrant liability to equity	-	-	-	-	424,100	-	-	-	424
Incremental value of Platinum note modification	-	-	-	-	1,070,600	-	-	-	1,07
Incremental value of Morrison & Foerster warrant modification	-	-	-	-	58,700	-	-	-	58,7
Stock issued in May 2011 Private Placement, net of $202,000 placement fees	-	-	2,216,106	2,200	3,674,000	-	(500,000)	-	3,17
Payments on note receivable for sale of stock	-	-					250,000		250
Stock issued upon conversion of	-	-	3,528,290	3,500	6,171,300	-	-	-	6,17

convertible promissory notes									
Stock issued upon conversion of all series of VistaGen California preferred stock	-	-	2,884,655	2,900	14,531,900	-	-	-	14,5
Fair value of stock issued for services prior to the Merger	-	-	1,371,743	1,400	2,224,100	-	-	-	2,22
Forgiveness of notes at the Merger	-	-	-	-	-	-	185,100	-	185
Stock issued upon exercise of modified warrants (including Platinum exercises)	-	-	3,121,259	3,100	3,426,200	-	-	-	3,42
Incremental value of warrant modifications (including modification of Platinum warrants)	-	-	-	-	1,028,900	-	-	-	1,02
Fair value of bonus warrants under FY 2012 Discounted Warrant Exercise Program	-	-	-	-	138,100	-	-	-	138
Stock issued in Fall 2011 Follow-on Offering	-	-	63,570	100	111,200	-	-	-	111
Stock issued upon exercise of options from the 1999 Stock Incentive Plan	-	-	113,979	100	102,100	-	-	-	102
Fair value of stock issued for services following the Merger	-	-	155,555	200	451,800	-	-	-	452
Fair value of warrants issued for services	-	-	-	-	564,500	-	-	-	564
Proceeds allocated to warrants issued and beneficial conversion feature in connection with 12% convertible notes	-	-	-	-	461,700	-	-	-	461
Stock issued in connection with note term extension	-	-	8,000	-	22,400	-	-	-	22,4

Stock issued upon conversion of Platinum Note to equity (net of Platinum warrant exercise reflected above)	231,090	200	-	-	3,387,700	-	-	-	3,38
Common stock exchanged for Series A Preferred under agreements with Platinum: Common Stock Exchange Agreement	45,980	-	-	-	750,600	(750,600)	-	-	-
Note and Warrant Exchange Agreement	159,985	200	-		2,480,900	(2,481,100)			-
Net loss for fiscal year 2012	-	-	-	-	-	-	-	(12,209,500)	(12,
Balances at March 31, 2012	437,055	$400	18,704,267	$18,700	$52,539,500	$(3,231,700)	$(250,000)	$(54,782,500)	$(5,7

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VISTAGEN THERAPEUTICS, INC.
(a development stage company)
CONSOLIDATED STATEMENTS OF STOCKHOLDERS' DEFICIT (continued)
Period from May 26, 1998 (inception) through March 31, 2013
(Amounts in $100s, except share and per share amounts)

	Series A Preferred Stock		Common Stock		Additional Paid-in Capital	Treasury Stock	Notes Receivable from Sale of Stock	Deficit Accumulated During the Development Stage	Total Stockholde Deficit
	Shares	Amount	Shares	Amount					
Balances at March 31, 2012	437,055	$400	18,704,267	$18,700	$52,539,500	$(3,231,700)	$(250,000)	$(54,782,500)	$(5,705,600
Share-based compensation expense	-	-	-	-	1,241,300	-	-	-	1,241,300
Fair value of common stock issued for services	-	-	400,000	400	339,600	-	-	-	340,000
Fair value of warrants issued for services	-	-	-	-	106,200	-	-	-	106,200
Shares issued upon exercise of modified warrants	-	-	549,056	500	274,000	-	-	-	274,500
Incremental fair value of modified warrants	-	-	-	-	440,700	-	-	-	440,700
Fair value of warrants issued upon exercise of moddified warrants	-	-	-	-	35,900	-	-	-	35,900
Fair value of shares issued in settlement of accounts payable	-	-	103,235	100	103,100	-	-	-	103,200
Common stock exchanged for	62,945	100	-	-	736,300	(736,400)	-	-	-

Series A Preferred under 2012 Exchange Agreement with Platinum									
Payment on note receivable from sale of stock	-	-	-	-	-	-	66,900	-	66,900
Modification of note receivable from sale of stock	-	-	-	-	-	-	(26,000)	-	(26,000
Incremental fair value of modified warrant and fair value of warrant issued in connection with Morrison & Foerster note payable restructuring	-	-	-	-	998,500	-	-	-	998,500
Fair value of warrant issued to Cato Holding Company in connection with note payable restructuring	-	-	-	-	120,500	-	-	-	120,500
Fair value of warrant issued to Cato Research, Ltd. in connection accounts payable restructuring	-	-	-	-	486,200	-	-	-	486,200
Fair value of warrant issued to University Health Network in connection with accounts	-	-	-	-	264,800	-	-	-	264,800

payable restructure									
Fair value of warrants issued to Morrison & Foerster, Cato Research Ltd. and University Health Network in connection with accrued interest on underlying notes	-	-	-	-	49,400	-	-	-	49,400
Sale of Units in Winter 2012 Private Placement, net	-	-	2,366,330	2,400	1,246,600	-	-	-	1,249,000
Exchange of February 2012 convertible notes for Units	-	-	1,357,281	1,400	1,214,200	-	-	-	1,215,600
Fair value of warrants issued to banker in connection with exchange of February 2012 convertible notes	-	-	-	-	28,200	-	-	-	28,200
Premium of fair value over face value of Exchange Note issued to Platinum in October 2012	-	-	-	-	1,083,200	-	-	-	1,083,200
Fair value of Series A Exchange Warrant issuable to Platinum recorded as a Warrant Liability	-	-	-	-	(3,068,200)	-	-	-	(3,068,200

Proceeds allocated to beneficial conversion feature of Investment Notes issued to Platinum in October 2012, February 2013 and March 2013	-	-	-	-	958,500	-	-	-	958,500
Incremental fair value of warrant modifications in February 2013	-	-	-	-	67,500	-	-	-	67,500
Net loss for fiscal year 2013	-	-	-	-	-	-	-	(12,886,700)	(12,886,70
Balances at March 31, 2013	500,000	$500	23,480,169	$23,500	$59,266,000	$(3,968,100)	$(209,100)	$(67,669,200)	$(12,556,40
Share-based compensation expense	-	-	-	-	1,137,300	-	-	-	1,137,300
Proceeds from sale of common stock for cash, including exercises of warrants under Discount Warrant Exercise Program	-	-	655,016	700	335,200	-	-	-	335,900
Beneficial conversion feature on note issued to Platinum in July 2013	-	-	-	-	100,700	-	-	-	100,700
Payment on note receivable from sale of	-	-	-	-	-	-	11,000	-	11,000

stock									
Allocated proceeds from sale of Units for cash under Winter 2013/2014 Private Placement, including beneficial conversion feature	-	-	2,015,000	2,000	836,200	-	-	-	838,200
Allocated proceeds from sale of Units for cash under Spring 2014 Private Placement, including beneficial conversion feature	-	-	50,000	-	36,000				36,000
Incremental fair value of warrant modifications	-	-	-	-	204,300	-	-	-	204,300
Fair value of warrants issued to Morrison & Foerster, Cato Research Ltd. and University Health Network in connection with accrued interest on underlying notes	-	-	-	-	60,800	-	-	-	60,800
Net loss for fiscal year 2014	-	-	-	-	-	-	-	(2,967,700)	(2,967,700
Balances at March 31, 2014	500,000	$500	26,200,185	$26,200	$61,976,500	$(3,968,100)	$(198,100)	$(70,636,900)	$(12,799,90

See accompanying notes to consolidated financial statements.

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VISTAGEN THERAPEUTICS, INC.
(a development stage company)

NOTES TO CONSOLIDATED FINANCIAL STATEMENTS

1. Description of Business

VistaGen Therapeutics, Inc., a Nevada corporation ("VistaGen" or the "Company"), is a biotechnology company with expertise in human pluripotent stem cell technology. The Company is applying and developing its stem cell technology for drug rescue and regenerative medicine. The Company's primary focus is on leveraging its stem cell technology platform, which it refers to as Human Clinical Trials in a Test Tube™, the human cells it produces, its novel, human cell-based bioassay systems, and medicinal chemistry to produce small molecule Drug Rescue Variants. These are new, safer variants of promising small molecule drug candidates previously discovered, developed and ultimately discontinued by pharmaceutical companies and others, after substantial investment and prior to market approval, due to unexpected heart or liver safety concerns. The Company refers to these promising drug candidates that are now potentially suitable for drug rescue as Drug Rescue Candidates These Drug Rescue Candidates have already been tested extensively and validated by a pharmaceutical or biotechnology company for their therapeutic (efficacy) and commercial potential. The key commercial objective of the Company's drug rescue strategy is to generate revenue from license, development and commercialization arrangements involving new, safer and proprietary Drug Rescue Variants that it produces with its contract medicinal chemistry collaborator and validates internally in its human cell-based bioassay systems prior to license. The Company anticipates that each validated lead Drug Rescue Variant will be suitable as a promising drug development program, either internally or in collaboration with a strategic partner. Through stem cell technology-based drug rescue, the Company intends to become a leading source of proprietary, small molecule drug candidates to the global pharmaceutical industry.
In parallel with its drug rescue activities, the Company is also interested in exploring ways to leverage its stem cell technology platform for regenerative medicine purposes, with emphasis on developing novel human disease models for discovery of small molecule drugs and biologics with regenerative and therapeutic potential. The Company's regenerative medicine focus would be based on its expertise in human biology and differentiation of human pluripotent stem cells to develop functional adult human cells and tissues involved in human disease, including blood, bone, cartilage, heart, liver and insulin-producing pancreatic beta-islet cells. Among its key objectives will be to explore regenerative medicine opportunities through pilot nonclinical proof-of-concept studies, after which the Company intends to assess any potential opportunities for further development and commercialization of therapeutically and commercially promising regenerative medicine programs, either on its own or with strategic partners.

AV-101 is VistaGen's orally-available, small molecule prodrug candidate which has successfully completed Phase 1 clinical development in the Unites States for treatment of neuropathic pain, a serious and chronic condition causing pain after an injury or disease of the peripheral or central nervous system that affects millions of people worldwide. The NIH awarded VistaGen approximately $8.8 million for preclinical and Phase 1 clinical development of AV-101. The Company intends to pursue potential opportunities for further clinical development and commercialization of AV-101 for neuropathic pain, epilepsy and depression, on its own and with strategic partners. In the event that it successfully completes a strategic partnering arrangement for AV-101, the Company plans to use the net proceeds from such an arrangement to expand its stem cell technology-based drug rescue and regenerative medicine programs.

VistaGen is in the development stage and, since inception, has devoted substantially all of its time and efforts to human pluripotent stem cell technology research and development, including, among other things, bioassay system development, small molecule drug development, creating, protecting and patenting intellectual property, recruiting personnel and raising working capital.

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The Merger

VistaGen Therapeutics, Inc., a California corporation incorporated on May 26, 1998 ("VistaGen California"), is a wholly-owned subsidiary of the Company. Excaliber Enterprises, Ltd. ("Excaliber"), a publicly-held company (formerly OTCBB: EXCA) was incorporated under the laws of the State of Nevada on October 6, 2005. Pursuant to a strategic merger transaction on May 11, 2011, Excaliber acquired all outstanding shares of VistaGen California in exchange for 6,836,452 shares of the Company's common stock and assumed all of VistaGen California's pre-Merger obligations (the "Merger"). Shortly after the Merger, Excaliber's name was changed to "VistaGen Therapeutics, Inc." (a Nevada corporation).

VistaGen California, as the accounting acquirer in the Merger, recorded the Merger as the issuance of common stock for the net monetary assets of Excaliber, accompanied by a recapitalization. The accounting treatment for the Merger was identical to that resulting from a reverse acquisition, except that the Company recorded no goodwill or other intangible assets. A total of 1,569,000 shares of common stock, representing the shares held by stockholders of Excaliber immediately prior to the Merger and effected for a post-Merger two-for-one (2:1) stock split, have been retroactively reflected as outstanding for all periods presented in the accompanying Consolidated Financial Statements of the Company. Additionally, the accompanying Consolidated Balance Sheets of the Company retroactively reflect the $0.001 par value of Excaliber's common stock.

In October 2011, the Company's stockholders amended the Company's Articles of Incorporation to authorize the Company to issue up to 200 million shares of common stock and up to 10 million shares of preferred stock and to authorize the Company's Board of Directors to prescribe the classes, series and the number of each class or series of preferred stock and the voting powers, designations, preferences, limitations, restrictions and relative rights of each class or series of preferred stock. In December 2011, the Company's Board of Directors authorized the creation of a series of up to 500,000 shares of Series A Preferred Stock, par value $0.001 ("Series A Preferred"), all of which are held by Platinum Long Term Growth VII, LLC ("Platinum"), currently the Company's largest institutional security holder. Pursuant to the Note Exchange and Purchase Agreement of October 11, 2012, as amended, between the Company and Platinum, Platinum has the right and option to exchange the 500,000 shares of the Company's Series A Preferred it holds for (i) 15,000,000 restricted shares of the Company's common stock, and (ii) a five-year warrant to purchase 7,500,000 restricted shares of the Company's common stock at an exercise price of $0.50 per share (see Note 10, Capital Stock).

The Consolidated Financial Statements of the Company in this Report represent the activity of VistaGen California from May 26, 1998, and the consolidated activity of VistaGen California and Excaliber (now VistaGen Therapeutics, Inc., a Nevada corporation), from May 11, 2011 (the date of the Merger). The Consolidated Financial Statements of the Company included in this Report also include the accounts of VistaGen California's two wholly-owned subsidiaries, Artemis Neuroscience, Inc., a Maryland corporation ("Artemis"), and VistaStem Canada, Inc., a corporation organized under the laws of Ontario, Canada ("VistaStem Canada").

2. Basis of Presentation and Going Concern

The accompanying Consolidated Financial Statements of the Company have been prepared assuming the Company will continue as a going concern. As a development stage company without sustainable revenues, VistaGen has experienced recurring losses and negative cash flows from operations. From inception through March 31, 2014, VistaGen has a deficit accumulated during its development stage of $70.6 million. The Company expects these conditions to continue for the foreseeable future as it expands its Human Clinical Trials in a Test Tube™ platform and executes its drug rescue programs and, potentially, regenerative medicine programs.

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Since its inception in May 1998 and through March 2014, the Company has financed its operations and technology acquisitions primarily through the issuance and sale of equity and debt securities, including convertible promissory notes and short-term promissory notes, for aggregate cash proceeds of approximately $26.0 million, as well as from an aggregate of approximately $16.4 million of government research grant awards, strategic collaboration payments and other revenues. Additionally, during the same period, the Company has issued equity securities with an approximate aggregate value at issuance of $12.6 million in non-cash settlements of certain liabilities, including liabilities for professional services rendered to the Company or as compensation for such services. At March 31, 2014, the Company did not have sufficient cash or cash equivalents to enable it to fund its operations, including expected cash expenditures of approximately $5 million, through the next twelve months. To meet its cash needs and fund its working capital requirements after March 31, 2014 and prior to a debt- or equity-based financing, through June 19, 2014, the Company entered into securities purchase agreements with accredited investors and institutions pursuant to which it sold to such accredited investors units of our securities ("Units"), for aggregate proceeds of $1,465,000, consisting of: (i) 10% subordinate convertible promissory notes in the aggregate face amount of $1,465,000 maturing on March 31, 2015; (ii) an aggregate of 1,465,000 restricted shares of its common stock; and (iii) warrants exercisable through December 31, 2016 to purchase an aggregate of 1,465,000 restricted shares of its common stock at an exercise price of $0.50 per share.

In April 2013, the Company entered into a Securities Purchase Agreement (as amended, "Securities Purchase Agreement") with Autilion AG, a company organized and existing under the laws of Switzerland ("Autilion"), under which Autilion is contractually obligated to purchase an aggregate of 72.0 million restricted shares of the Company's common stock at a purchase price of $0.50 per share for aggregate cash proceeds to the Company of $36.0 million (the "Autilion Financing"). To date, Autilion has completed only a nominal closing under the Securities Purchase Agreement. Therefore, Autilion is in default under the Securities Purchase Agreement, and the Company can provide no assurance that Autilion will complete a material closing under the Securities Purchase Agreement. In the event that Autilion does not complete a material portion of the Autilion Financing pursuant to the Securities Purchase Agreement in the near term, the Company will need to obtain from $4.0 million to $6.0 million from alternative financing sources to execute its business plan over the next twelve to fifteen months. Substantial additional financing may not be available to the Company on a timely basis, on acceptable terms, or at all. In the event the Company is unable to obtain substantial additional financing on a timely basis, its business, financial condition, and results of operations may be harmed, the price of its stock may decline, and it may not be able to continue as a going concern.

To meet its working capital needs during the fiscal year ended March 31, 2014, the Company issued an additional Senior Secured Convertible Promissory Note to Platinum, and sold Units consisting of convertible promissory notes, shares of its restricted common stock and warrants to purchase restricted shares of its common stock, to accredited investors in private placements as described more completely in Note 9, Convertible Promissory Notes and Other Notes Payable, and Note 10, Capital Stock. To provide working capital for operations from March 31, 2014 through the date of this report, the Company completed private placements of its securities to Platinum and other accredited investors resulting in aggregate cash proceeds of $1,465,000, as described in Note 17, Subsequent Events.

To the extent necessary, the Company may also seek to meet its future cash needs and fund its working capital requirements through a combination of additional private placements of its securities, which may include both debt and equity securities, research and development collaborations, license fees, and government grant awards. Alternatively, the Company may seek to raise additional capital through a registered public offering of its securities. In May 2014, the Company filed a Registration Statement on Form S-1 with the Securities and Exchange Commission covering the potential sale of shares of its common stock in a registered public offering. Additionally, the Company believes that its participation in strategic collaborations, including licensing transactions, may provide additional cash in support of its future working capital requirements. If the Company is unable to obtain sufficient financing from the Autilion Financing or alternative sources, it may be required to reduce, defer, or discontinue certain of its research and development activities or it may not be able to continue as a going concern. The

consolidated financial statements do not include any adjustments that might result from the outcome of this uncertainty.

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3. Summary of Significant Accounting Policies

Use of Estimates

The preparation of financial statements in conformity with U.S. generally accepted accounting principles ("U.S. GAAP") requires management to make estimates and assumptions that affect the reported amounts of assets and liabilities, the disclosure of contingent assets and liabilities at the date of the financial statements, and the reported amounts of revenues and expenses during the reporting period. Actual results could differ from those estimates. Significant estimates include, but are not limited to, those relating to stock-based compensation, revenue recognition, and the assumptions used to value warrants, warrant modifications and warrant liabilities.

Principles of Consolidation

The accompanying consolidated financial statements include the Company's accounts, and the accounts of VistaGen California's wholly-owned inactive subsidiaries, Artemis Neurosciences and VistaStem Canada.

Cash and Cash Equivalents

Cash and cash equivalents are considered to be highly liquid investments with maturities of three months or less at the date of purchase.

Property and Equipment

Property and equipment is stated at cost. Repairs and maintenance costs are expensed in the period incurred. Depreciation is calculated using the straight-line method over the estimated useful lives of the assets. The estimated useful lives of property and equipment range from five to seven years.

Impairment or Disposal of Long-Lived Assets

The Company evaluates its long-lived assets, primarily property and equipment, for impairment whenever events or changes in circumstances indicate that their carrying value may not be recoverable from the estimated future cash flows expected to result from their use or eventual disposition. If the estimates of future undiscounted net cash flows are insufficient to recover the carrying value of the assets, the Company records an impairment loss in the amount by which the carrying value of the assets exceeds their fair value. If the assets are determined to be recoverable, but the useful lives are shorter than originally estimated, the Company depreciates or amortizes the net book value of the assets over the newly determined remaining useful lives. The Company has not recorded any impairment charges to date.

Revenue Recognition

Although the Company does not currently have any such arrangements, it has historically generated revenue principally from collaborative research and development arrangements, technology transfer agreements, including strategic licenses, and government grants. 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.

The Company recognizes 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, the Company complies with the above revenue recognition criteria in the following manner:

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Collaborative arrangements typically consist of non-refundable and/or exclusive up front technology access fees, cost reimbursements for specific research and development spending, and various milestone and future product 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 the Company has continuing performance obligations and has no objective and reliable evidence of the fair value of those obligations. The Company recognizes non-refundable upfront technology access fees under agreements in which it has a continuing performance obligation ratably, on a straight-line basis, over the period in which the Company is 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, development and/or regulatory milestone payments 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 the Company's continuing involvement, and, in the case of development and/or regulatory milestone payments, when the applicable event triggering such a payment has occurred.

Government grants, which support the Company's research efforts on specific projects, generally provide for reimbursement of approved costs as defined in the terms of grant awards. Grant revenue is recognized when associated project costs are incurred.

Research and Development Expenses

Research and development expenses include internal and external costs. Internal costs include salaries and employment related expenses of the Company's internal scientific personnel and direct project costs. External research and development expenses consist of sponsored stem cell research and development costs, costs associated with non-clinical and clinical drug rescue and development activities, including development of AV-101, the Company's drug development candidate which has successfully completed Phase 1 development, and costs related to protection of the Company's intellectual property, including, but not limited to, application and prosecution of patents related to the Company's stem cell technology platform, Human Clinical Trials in a Test Tube, and AV-101. All such research and development costs are charged to expense as incurred.

Stock-Based Compensation

The Company recognizes compensation cost for all stock-based awards to employees in its financial statements based on their grant date fair value. Stock-based compensation expense is recognized over the period during which the employee is required to perform service in exchange for the award, which generally represents the scheduled vesting period of options and warrants to purchase shares of the Company's common stock. The Company has no awards with market or performance conditions. For stock-based awards to non-employees, the Company re-measures the fair value of such awards as they vest and the resulting value is recognized as an expense during the period over which applicable services are performed by the recipient.

Income Taxes

The Company accounts for income taxes using the asset and liability approach for financial reporting purposes. Deferred tax assets and liabilities are recognized 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.

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Concentrations of Credit Risk

Financial instruments, which potentially subject the Company to concentrations of credit risk, consist principally of cash and cash equivalents. The Company's investment policies limit any such investments to short-term, low-risk investments. The Company deposits cash and cash equivalents with quality financial institutions and is insured to the maximum of federal limitations. Balances in these accounts may exceed federally insured limits at times.

Warrant Liability

The Company has issued certain warrants to Platinum and, subject to Platinum's exercise of its rights to exchange shares of the Company's Series A Preferred that it holds, the Company is also obligated to issue an additional warrant to Platinum, that contain an exercise price adjustment feature in the event the Company subsequently issues additional equity instruments at a price lower than the exercise price of the warrants. The Company accounts for these warrants as non-cash liabilities and estimates their fair value as described in Note 4, Fair Value Measurements; Note 9, Convertible Promissory Notes and Other Notes Payable, and Note 10, Capital Stock. The Company computes the fair value of the warrant liability at each reporting period and the change in the fair value is recorded as non-cash expense or non-cash income. The key component in determining the fair value of the warrant and the related liability is the Company's stock price, which is subject to significant fluctuation and is not under the Company's control. The resulting change in the fair value of the warrant liability on the Company's net income or loss is therefore also subject to significant fluctuation and will continue to be so until all of the warrants are issued and exercised, amended or expire. Assuming all other fair value inputs remain generally constant, the Company will record an increase in the warrant liability and non-cash expense when its stock price increases and a decrease in the warrant liability and non-cash income when its stock price decreases.

Comprehensive Loss

The Company has no components of other comprehensive loss other than net loss, and accordingly the Company's comprehensive loss is equivalent to its net loss for the periods presented.

Loss per Common Share

Basic income (loss) per share of common stock excludes the effect of dilution and is computed by dividing net income (loss) by the weighted-average number of shares of common stock outstanding for the period. Diluted income (loss) per share of common stock reflects the potential dilution that could occur if securities or other contracts to issue shares of common stock were exercised or converted into shares of common stock. In calculating diluted net income (loss) per share, the Company adjusts the numerator for the change in the fair value of the warrant liability attributable to outstanding warrants, only if dilutive, and increases the denominator to include the number of potentially dilutive common shares assumed to be outstanding during the period using the treasury stock method. As a result of the Company's net loss for both periods presented, potentially dilutive securities were excluded from the computation, as their effect would be antidilutive. Additionally, no potentially dilutive securities were assumed to be converted into common shares and outstanding during either period for purposes of calculating diluted earnings per share.

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Basic and diluted net loss attributable to common stockholders per share was computed as follows:

	Years Ended March 31,	
	2014	2013
Numerator:		
Net loss attributable to common stockholders for basic earnings per share	$ (2,967,700)	$ (23,079,900)
less: change in fair value of warrant liability attributable to Exchange, Investment and July 2013 Warrants issued to Platinum	(1,219,500)	-
Net loss for diluted earnings per share attributable to common stockholders	$ (4,187,200)	$ (23,079,900)
Denominator:		
Weighted average basic common shares outstanding	21,973,149	18,108,444
Assumed conversion of dilutive securities:		
Warrants to purchase common stock	-	-
Potentially dilutive common shares assumed converted	-	-
Denominator for diluted earnings per share - adjusted weighted average shares	21,973,149	18,108,444
Basic net loss attributable to common stockholders per common share	$ (0.14)	$ (1.27)
Diluted net loss attributable to common stockholders per common share	$ (0.19)	$ (1.27)

Potentially dilutive securities excluded in determining diluted net loss per common share for the fiscal years ended March 31, 2014 and 2013 are as follows:

	Fiscal Years Ended March 31,	
	2014	2013
Series A preferred stock issued and outstanding (1)	15,000,000	15,000,000
Warrant shares issuable to Platinum upon exercise of common stock warrants by Platinum upon exchange of Series A preferred stock under the terms of the October 11, 2012 Note Purchase and Exchange Agreement	7,500,000	7,500,000
Outstanding options under the 2008 and 1999 Stock Incentive Plans	4,249,271	4,912,604
Outstanding warrants to purchase common stock	17,095,633	14,660,335
10% convertible Exchange Note and Investment Notes issued to Platinum in October 2012, February 2013 and March 2013, including accrued interest through March 31, 2014 (2)	7,495,957	6,775,682
10% convertible note issued to Platinum on July 26, 2013, including accrued interest through March 31, 2014	535,506	-

10% convertible notes issued as a component of Unit Private Placements, including accrued interest through March 31, 2014	2,186,811	-
Total	54,063,178	48,848,621

(1) Assumes exchange under the terms of the October 11, 2012 Note Exchange and Purchase Agreement with Platinum
(2) Assumes conversion under the terms of the October 11, 2012 Note Exchange and Purchase Agreement with Platinum and the terms of the individual notes

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Recent Accounting Pronouncements

In May 2014, the Financial Accounting Standards Board ("FASB") issued Accounting Standards Update ("ASU") No. 2014-09, Revenue from Contracts with Customers (Topic 606), which supersedes the revenue recognition requirements in ASC 605, Revenue Recognition. This ASU is based on the principle that revenue is recognized to depict the transfer of goods or services to customers in an amount that reflects the consideration to which the entity expects to be entitled in exchange for those goods or services. The ASU also requires additional disclosure about the nature, amount, timing and uncertainty of revenue and cash flows arising from customer contracts, including significant judgments and changes in judgments and assets recognized from costs incurred to obtain or fulfill a contract. The effective date will be the first annual period beginning after December 15, 2016, using one of two retrospective application methods. The Company is currently evaluating the impact on its Consolidated Financial Statements of adopting this ASU.

In June 2014, the FASB issued ASU No. 2014-10, Development Stage Entities (Topic 915): Elimination of Certain Financial Reporting Requirements, Including an Amendment to Variable Interest Entities Guidance in Topic 810, Consolidation. The amendments in this ASU remove all incremental financial reporting requirements for development stage entities. Among other changes, this ASU will no longer require development stage entities to present inception-to-date information about income statement line items, cash flows, and equity transactions. The presentation and disclosure requirements in Topic 915 will no longer be required for the first annual period beginning after December 15, 2014. The Company's adoption of this ASU will result in the elimination of the inception-to-date information currently included in its Consolidated Statements of Operations and Comprehensive Loss, Cash Flows and Stockholders' Deficit effective with the fiscal year beginning in April 2015.

4. Fair Value Measurements

The Company follows the principles of fair value accounting as they relate to its financial assets and financial liabilities. Fair value is defined as the estimated exit price received to sell an asset or paid to transfer a liability in an orderly transaction between market participants at the measurement date, rather than an entry price that represents the purchase price of an asset or liability. Where available, fair value is based on observable market prices or parameters, or derived from such prices or parameters. Where observable prices or inputs are not available, valuation models are applied. These valuation techniques involve some level of management estimation and judgment, the degree of which is dependent on several factors, including the instrument's complexity. The required fair value hierarchy that prioritizes observable and unobservable inputs used to measure fair value into three broad levels is described as follows:

Level 1 — Quoted prices (unadjusted) in active markets that are accessible at the measurement date for assets or liabilities. The fair value hierarchy gives the highest priority to Level 1 inputs.

Level 2 — Inputs other than Level 1 that are observable, either directly or indirectly, such as quoted prices for similar assets or liabilities; quoted prices in markets that are not active; or other inputs that are observable or can be corroborated by observable market data for substantially the full term of the assets or liabilities.

Level 3 — Unobservable inputs (i.e., inputs that reflect the reporting entity's own assumptions about the assumptions that market participants would use in estimating the fair value of an asset or liability) are used when little or no market data is available. The fair value hierarchy gives the lowest priority to Level 3 inputs.

A financial instrument's categorization within the valuation hierarchy is based upon the lowest level of input that is significant to the fair value measurement. Where quoted prices are available in an active market, securities are classified as Level 1 of the valuation hierarchy. If quoted market prices are not available for the specific financial

instrument, then the Company estimates fair value by using pricing models, quoted prices of financial instruments with similar characteristics or discounted cash flows. In certain cases where there is limited activity or less transparency around inputs to valuation, financial assets or liabilities are classified as Level 3 within the valuation hierarchy.

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The Company does not use derivative instruments for hedging of market risks or for trading or speculative purposes. In conjunction with the Senior Secured Convertible Promissory Notes and related Exchange Warrant and Investment Warrants issued to Platinum in October 2012, February 2013 and March 2013 (see Note 9, Convertible Promissory Notes and Other Notes Payable), and the potential issuance of the Series A Exchange Warrant (see Note 10, Capital Stock), all pursuant to the October 2012 Agreement, and the Senior Secured Convertible Promissory Note and related warrant issued to Platinum in July 2013, the Company determined that the warrants included certain exercise price adjustment features requiring the warrants to be treated as liabilities, which were recorded at their estimated fair value. The Company determined the fair value of the warrant liability using a Monte Carlo simulation model with Level 3 inputs. Inputs used to determine fair value include the remaining contractual term of the notes, risk-free interest rates, expected volatility of the price of the underlying common stock, and the probability of a financing transaction that would trigger a reset in the warrant exercise price, and, in the case of the Series A Exchange Warrant, the probability of Platinum's exchange of the shares of Series A Preferred it holds into shares of common stock. Changes in the fair value of these warrant liabilities have been recognized as non-cash income or expense in the Consolidated Statements of Operations and Comprehensive Loss for the fiscal years ended March 31, 2014 and 2013.

The fair value hierarchy for liabilities measured at fair value on a recurring basis is as follows:

		Fair Value Measurements at Reporting Date Using		
	Total Carrying Value	Quoted Prices in Active Markets for Identical Assets (Level 1)	Significant Other Observable Inputs (Level 2)	Significant Unobservable Inputs (Level 3)
March 31, 2014:				
Warrant liability	$ 2,973,900	$ -	$ -	$ 2,973,900
March 31, 2013:				
Warrant liability	$ 6,394,000	$ -	$ -	$ 6,394,000

During the fiscal years ended March 31, 2014 and 2013, there were no significant changes to the valuation models used for purposes of determining the fair value of the Level 3 warrant liability.

The changes in Level 3 liabilities measured at fair value on a recurring basis are as follows:

	(Level 3) Warrant Liability
Balance at March 31, 2012	$-
Recognition of warrant liability upon issuance of Exchange and Investment Warrants to Platinum under October 2012 Agreement	1,690,000
Recognition of warrant liability in connection with Series A Exchange Warrant potentially issuable to Platinum under October 2012 Agreement	3,068,200
Mark to market loss included in net loss	1,635,800
Balance at March 31, 2013	6,394,000
	146,800

Recognition of warrant liability upon issuance of Senior Secured Convertible Promissory Note and warrant to Platinum on July 26, 2013	
Mark to market gain included in net loss	(3,566,900)
Balance at March 31, 2014	$2,973,900

No assets or other liabilities were measured on a recurring basis at fair value at March 31, 2014 or 2013.

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5. Prepaid Expenses and Other Current Assets

Prepaid expenses and other current assets consist of the following:

	March 31, 2014	2013
Insurance	$21,800	$19,700
Legal fees	3,400	3,400
Interest receivable on note receivable from sale of common stock	2,800	1,600
Technology license fees and all other	12,500	9,000
	$40,500	$33,700

6. Property and Equipment

Property and equipment consists of the following:

	March 31, 2014	2013
Laboratory equipment	$653,600	$649,500
Tenant improvements	27,000	-
Computers and network equipment	32,100	12,900
Office furniture and equipment	69,600	69,600
	782,300	732,000
Accumulated depreciation and amortization	(606,000)	(551,300)
Property and equipment, net	$176,300	$180,700

In connection with the issuance of Senior Secured Convertible Promissory Notes to Platinum in July and August 2012, and under the October 2012 Agreement with Platinum, the Company entered into a Security Agreement with Platinum under which the repayment of all amounts due under the terms of the various Senior Secured Convertible Promissory Notes is secured by the Company's assets, including its tangible and intangible personal property, licenses, patent licenses, trademarks and trademark licenses (see Note 9, Convertible Promissory Notes and Other Notes Payable).

7. AV-101 Acquisition

In November 2003, pursuant to an Agreement and Plan of Merger (the "Artemis Agreement"), the Company acquired Artemis Neurosciences ("Artemis"), a privately-held company also in the development stage, for the purpose of acquiring exclusive licenses to patents and other intellectual property related to the use and function of AV-101, a prodrug candidate then in nonclinical development, with the potential to treat neuropathic pain, depression, and other neurological diseases and disorders, epilepsy, Huntington's disease and Parkinson's disease. Pursuant to the Artemis Agreement, all shares of Artemis common stock were converted into shares of VistaGen California's Series B-1 Preferred Stock, resulting in VistaGen California's pre-merger issuance of 1,356,750 shares of its Series B-1 Preferred Stock, valued, pre-merger, at $5.545 per share, resulting in the pre-merger purchase price of all outstanding shares of

Artemis of $7,523,200. The total purchase price was allocated to AV-101 acquired in-process research and development and was expensed concurrent with the Artemis acquisition, since AV-101 required further research and development before the Company could commence clinical trials and did not have any proven alternative future uses.

To date, the Company has received an aggregate of $8.8 million from the NIH for non-clinical and clinical development of AV-101. The Company successfully completed a Phase 1a clinical trial of AV-101 during the fiscal year ended March 31, 2012 and successfully completed a Phase 1b clinical trial of AV-101 in the fiscal year ended March 31, 2013.

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8. Accrued Expenses

Accrued expenses consist of:

	March 31,	
	2014	2013
Accrued professional services	$135,700	$67,800
Accrued compensation	489,900	219,300
Accrued royalties and license fees	-	25,000
All other	-	30,800