VistaGen Therapeutics, Inc.
Form 10-Q
August 12, 2016

UNITED STATES

SECURITIES AND EXCHANGE COMMISSION

Washington, DC 20549

Form 10-Q

(Mark One)

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

For the quarterly period ended June 30, 2016
or

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

For the transition period from to .

Commission File Number: 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, CA 94080
(Address of principal executive offices including zip code)

(650) 577-3600
(Registrant's telephone number, including area code)

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 x No 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	[ ]	Accelerated filer	[ ]
Non-Accelerated filer	[ ]	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 Exchange Act). Yes o No x

As of August 11, 2016, 7,970,705 shares of the registrant’s common stock, $0.001 par value, were issued and outstanding.

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VistaGen Therapeutics, Inc.
Quarterly Report on Form 10-Q
for the Quarter Ended June 30, 2016

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PART I. FINANCIAL INFORMATION

Item 1. Condensed Consolidated Financial Statements (Unaudited)

VISTAGEN THERAPEUTICS, INC.

CONDENSED CONSOLIDATED BALANCE SHEETS
(Amounts in Dollars, except share amounts)

	June 30, 2016 (Unaudited)	
ASSETS		
Current assets:		
Cash and cash equivalents	$8,499,900	$
Prepaid expenses and other current assets	134,700	
Total current assets	8,634,600	
Property and equipment, net	76,300	
Security deposits and other assets	46,900	
Total assets	$8,757,800	$
LIABILITIES AND STOCKHOLDERS' EQUITY (DEFICIT)		
Current liabilities:		
Accounts payable	$930,100	$
Accrued expenses	564,800	
Current portion of notes payable and accrued interest	105,900	
Capital lease obligations	900	
Total current liabilities	1,601,700	
Non-current liabilities:		
Notes payable	-	
Accrued dividends on Series B Preferred Stock	946,000	
Deferred rent liability	47,600	
Total non-current liabilities	993,600	
Total liabilities	2,595,300	
Commitments and contingencies		
Stockholders' equity (deficit):		
Preferred stock, $0.001 par value; 10,000,000 shares authorized at June 30, 2016 and March 31, 2016:		
Series A Preferred, 500,000 shares authorized and outstanding at June 30, 2016 and March 31, 2016	500	
Series B Preferred; 4,000,000 shares authorized at June 30, 2016 and March 31, 2016; 1,247,740 shares and 3,663,077 shares issued and outstanding at June 30, 2016 and March 31, 2016, respectively	1,200	
Series C Preferred; 3,000,000 shares authorized at June 30, 2016 and March 31, 2016; 2,318,012 shares issued and outstanding at June 30, 2016 and March 31, 2016	2,300	
Common stock, $0.001 par value; 30,000,000 shares authorized at June 30, 2016 and March 31, 2016; 8,106,370 and 2,623,145 shares issued at June 30, 2016 and March 31, 2016, respectively	8,100	
Additional paid-in capital	143,828,800	

Treasury stock, at cost, 135,665 shares of common stock held at June 30, 2016 and March 31, 2016	(3,968,100	)
Accumulated deficit	(133,710,300	)
Total stockholders' equity (deficit)	6,162,500	
Total liabilities and stockholders' equity (deficit)	$8,757,800	$

See accompanying notes to Condensed Consolidated Financial Statements.

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VISTAGEN THERAPEUTICS, INC.

CONDENSED CONSOLIDATED STATEMENTS OF OPERATIONS AND COMPREHENSIVE LOSS
(Unaudited)
(Amounts in dollars, except share amounts)

	Three Months Ended June 30,	
	2016	2015
Operating expenses:		
Research and development	825,700	372,600
General and administrative	1,137,600	1,448,500
Total operating expenses	1,963,300	1,821,100
Loss from operations	(1,963,300)	(1,821,100)
Other expenses, net:		
Interest expense, net	(1,400)	(755,100)
Change in warrant liability	-	(1,894,700)
Loss on extinguishment of debt	-	(25,050,900)
Loss before income taxes	(1,964,700)	(29,521,800)
Income taxes	(2,400)	(2,300)
Net loss	$(1,967,100)	$(29,524,100)
Accrued dividend on Series B Preferred stock	(539,800)	(213,300)
Deemed dividend on Series B Preferred Units	(111,100)	(256,200)
Net loss attributable to common stockholders	$(2,618,000)	$(29,993,600)
Basic and diluted net loss attributable to common stockholders per common share	$(0.51)	$(19.23)
Weighted average shares used in computing basic and diluted net loss attributable to common stockholders per common share	5,097,832	1,559,483
Comprehensive loss	$(1,967,100)	$(29,524,100)

See accompanying notes to Condensed Consolidated Financial Statements.

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VISTAGEN THERAPEUTICS, INC.

CONDENSED CONSOLIDATED STATEMENTS OF CASH FLOWS
(Unaudited)
(Amounts in Dollars)

	Three Months Ended June 30,	
	2016	2015
Cash flows from operating activities:		
Net loss	$(1,967,100)	$(29,524,100)
Adjustments to reconcile net loss to net cash used in operating activities:		
Depreciation and amortization	13,300	14,500
Amortization of discounts on convertible and promissory notes	-	550,800
Change in warrant liability	-	1,894,700
Stock-based compensation	107,900	29,200
Expense related to modification of warrants, including exchange of warrants for Series C Preferred and common stock	40,300	122,300
Amortization of deferred rent	(7,900)	(4,700)
Fair value of common stock granted for services	-	500,000
Fair value of Series B Preferred stock granted for services	375,000	250,000
Loss on currency fluctuation	-	10,300
Loss on extinguishment of debt	-	25,050,900
Changes in operating assets and liabilities:		
Prepaid expenses and other current assets	34,600	(8,200)
Accounts payable and accrued expenses, including accrued interest	(267,100)	291,000
Net cash used in operating activities	(1,671,000)	(823,300)
Cash flows from investing activities:		
Purchases of equipment	(2,000)	-
Net cash used in investing activities	(2,000)	-
Cash flows from financing activities:		
Net proceeds from issuance of common stock and warrants, including Units	9,537,100	280,000
Net proceeds from issuance of Series B Preferred Units	278,000	607,500
Repayment of capital lease obligations	(300)	(200)
Repayment of notes	(70,400)	(13,000)
Net cash provided by financing activities	9,744,400	874,300
Net increase in cash and cash equivalents	8,071,400	51,000
Cash and cash equivalents at beginning of period	428,500	70,000
Cash and cash equivalents at end of period	$8,499,900	$121,000
Supplemental disclosure of noncash activities:		
Conversion of Senior Secured Notes, Subordinate Convertible Notes, Promissory Notes, Accounts payable and other debt into Series B Preferred	$-	$17,335,400
Insurance premiums settled by issuing note payable	$117,500	$79,400
Accrued dividends on Series B Preferred	$539,800	$213,300
Accrued dividends on Series B Preferred settled upon conversion by issuance of common stock	$1,683,400	$-

See accompanying notes to Condensed Consolidated Financial Statements.

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VISTAGEN THERAPEUTICS, INC.
NOTES TO CONDENSED CONSOLIDATED FINANCIAL STATEMENTS
(Unaudited)

Note 1. Description of Business

Overview

VistaGen Therapeutics, Inc. (NASDAQ: VTGN), a Nevada corporation, is a clinical-stage biopharmaceutical company dedicated to developing and commercializing innovative product candidates for patients with diseases and disorders involving the central nervous system (CNS). Our principal executive offices are located at 343 Allerton Avenue, South San Francisco, California 94080, and our telephone number is (650) 577-3600. Our website address is www.vistagen.com. 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.

Our lead product candidate, AV-101, is a new generation, oral antidepressant drug candidate in Phase 2 development for the adjunctive treatment of Major Depressive Disorder (MDD) in patients with an inadequate response to standard antidepressants approved by the U.S. Food and Drug Administration (FDA). We believe AV-101 may also have therapeutic potential in chronic neuropathic pain, epilepsy, Huntington's disease and Parkinson's disease.

AV-101's mechanism of action, as an N-methyl D aspartate receptor (NMDAR) antagonist binding selectively at the glycine binding (GlyB) co-agonist site of the NMDAR, is fundamentally differentiated from all FDA-approved antidepressants, as well as all atypical antipsychotics used to augment standard antidepressant therapy.

Our ongoing Phase 2a clinical study of AV-101 in subjects with treatment-resistant MDD is being fully funded by the U.S. National Institute of Mental Health (NIMH) under our February 2015 Cooperative Research and Development Agreement (CRADA) with the NIMH, and is being conducted by Dr. Carlos Zarate, Jr., Chief of the NIMH's Experimental Therapeutics & Pathophysiology Branch and its Section on Neurobiology and Treatment of Mood and Anxiety Disorders. The first patient in this NIMH-sponsored Phase 2a study was dosed in November 2015. Previous NIMH studies, including studies conducted by Dr. Zarate, have focused on the antidepressant effects of low dose, intravenous (I.V.) ketamine in patients with treatment-resistant depression. These NIMH studies, as well as clinical research by others, have demonstrated robust antidepressant effects in patients with treatment-resistant MDD within twenty-four hours of a single low dose of I.V. ketamine. We believe AV-101 may have potential to deliver ketamine-like fast-acting antidepressant effects without ketamine's serious side effects.

We are preparing to launch our Phase 2b clinical study of AV-101 for the adjunctive treatment of MDD in patients with an inadequate response to standard, FDA-approved antidepressants. We anticipate commencement of this multi-center, multi-dose, double blind, placebo-controlled Phase 2b efficacy and safety study at the end of the fourth quarter of 2016. Dr. Maurizio Fava, Professor of Psychiatry at Harvard Medical School and Director, Division of Clinical Research, Massachusetts General Hospital (MGH) Research Institute, will be the Principal Investigator of our Phase 2b study of AV-101 in treatment resistant MDD. Dr. Fava was the co-Principal Investigator with Dr. A. John Rush of the largest clinical trial conducted in depression to date, the STAR*D study, whose findings were published in journals such the New England Journal of Medicine (NEJM) and the Journal of the American Medical Association (JAMA). We currently anticipate top line results in this Phase 2b study in the second quarter of 2018.

In addition to clinical development of AV-101, we are focused on advancing potential commercial applications of our human pluripotent stem cell (hPSC) technology platform with respect to drug rescue programs aimed at developing proprietary small molecule new chemical entities (NCEs) for our drug candidate pipeline. We are also focused on potential regenerative medicine applications using blood, cartilage, heart and/or liver cells derived from hPSCs, and

may pursue these applications in collaboration with third-parties.

AV-101 and Major Depressive Disorder

Background

The World Health Organization (WHO) estimates that 350 million people worldwide are affected by depression. According to the U.S. National Institutes of Health (NIH) major depression is one of the most common mental disorders in the U.S. The NIMH reports that, in 2014, an estimated 15.7 million adults aged 18 or older in the U.S. had at least one major depressive episode in the past year. This represented 6.7 percent of all U.S. adults. According to the U.S. Centers for Disease Control and Prevention (CDC) one in 10 Americans over the age of 12 takes an antidepressant medication.

Most standard, FDA-approved antidepressants target neurotransmitter reuptake inhibition – either serotonin (SSRIs) or serotonin/norepinephrine (SNRIs). Even when effective, these standard depression medications take many weeks to achieve adequate antidepressant effects. Nearly two out of every three drug-treated depression patients, including an estimated 6.9 million drug-treated MDD patients in the U.S., obtain inadequate therapeutic benefit from initial treatment with a standard antidepressant. Unfortunately, even after treatment with as many as four different standard antidepressants, nearly one out of every three drug-treated depression patients do not achieve adequate therapeutic benefits. Such treatment-resistant depression patients often seek to treat their depression with non-drug-related approaches, such as Electroconvulsive Therapy (ECT), or to augment their inadequate response to standard antidepressants by adding an atypical antipsychotic (such as, for example, aripiprazole) to their treatment regimen, despite the modest potential therapeutic benefit and significant risk of additional side effects with such augmentation options.

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All standard antidepressants have risks of significant side effects, including, among others, potentially anxiety, metabolic syndrome, sleep disturbance and sexual dysfunction. They also have a "Black Box" warning due to risks of worsening depression and suicide in certain groups. Use of atypical antipsychotics to augment inadequately performing standard antidepressants increases the risk of serious side effects, including, potentially, tardive dyskinesia, significant weight gain, diabetes and heart disease, while offering only a modest potential increase in therapeutic benefit. Use of ECT increases the risk of serious side effects, including, headaches, tiredness, disorientation, intense sleepiness, hallucinations and long-term memory loss.

AV-101

AV-101, our oral new generation antidepressant drug candidate, is in Phase 2 clinical development for the adjunctive treatment of MDD patients with an inadequate response to standard antidepressants. As published in the October 2015 issue of the peer-reviewed, Journal of Pharmacology and Experimental Therapeutics, in an article entitled, The prodrug 4-chlorokynurenine causes ketamine-like antidepressant effects, but not side effects, by NMDA/glycineB-site inhibition, using well-established preclinical models of depression, AV-101 was shown to induce fast-acting, dose-dependent, persistent and statistically significant antidepressant-like responses, following a single treatment. These responses were equivalent to those seen with a single, sub-anesthetic control dose of the NMDAR antagonist ketamine. In the same preclinical studies, a standard antidepressant, the SSRI fluoxetine, did not induce rapid onset antidepressant-like responses. In addition, these studies confirmed that the fast-acting antidepressive effects of AV-101 were mediated through the GlyB site and involved the activation of a key neurological pathway, the alpha-amino-3-hydroxy-5-methyl-4-isoxazolepropionic acid (AMPA) receptor pathway. Activation of the AMPA receptor pathway is a common feature of fast-acting antidepressants.

Following the completion of our NIH-funded, randomized, double blind, placebo-controlled Phase 1a and Phase 1b safety studies, we are now collaborating with the NIMH in a Phase 2a study. Under our February 2015 CRADA, the NIMH is funding, and Dr. Carlos Zarate Jr. of the NIMH as Principal Investigator is conducting, our ongoing Phase 2a efficacy and safety study of AV-101 in subjects with treatment-resistant MDD. The trial is expected to enroll 20 to 28 patients. The first patient was dosed in November 2015, and we currently anticipate receiving topline results in the second quarter of 2017.

We are preparing to launch our Phase 2b clinical study of AV-101 for the adjunctive treatment of MDD in patients with an inadequate response to standard, FDA-approved antidepressants. We anticipate the launch of this Phase 2b study, with Dr. Maurizio Fava of Harvard Medical School serving as Principal Investigator, at the end of the fourth quarter of 2016. We anticipate top line results from this Phase 2b study in the second quarter of 2018.

Several preclinical studies support the hypothesis that AV-101 also has the potential to treat multiple CNS disorders and neurodegenerative diseases in addition to depression, including chronic neuropathic pain, epilepsy, Parkinson's disease and Huntington's disease, where modulation of the NMDAR, AMPA pathway and/or active metabolites of AV-101 may achieve therapeutic benefit.

CardioSafe 3D™; NCE Drug Rescue and Regenerative Medicine

CardioSafe 3D™ is our customized in vitro cardiac bioassay system capable of predicting potential human heart toxicity of small molecule NCEs in vitro, long before they are ever tested in animal and human studies. Our current strategic interests involving our stem cell technology platform include (i) advancing current internal efforts focused on CardioSafe 3D drug rescue to expand our drug candidate pipeline with selected proprietary small molecule NCEs, leveraging substantial prior research and development investments by pharmaceutical companies and others related to public domain NCEs terminated before FDA approval due to heart toxicity risks and (ii) establishing collaborative arrangements with qualified third-parties focused on regenerative medicine (RM) applications, including (A)

cell-based therapy (injection of stem cell-derived mature organ-specific cells obtained through directed differentiation), (B) cell repair therapy (induction of regeneration by biologically active molecules administered alone or produced by infused genetically engineered cells), or (C) tissue engineering (transplantation of in vitro grown complex tissues), involving hPSC-derived blood, bone, cartilage, heart and/or liver cells.

Subsidiaries

VistaGen Therapeutics, Inc., a California corporation (VistaGen California), is our wholly-owned subsidiary. Our Condensed Consolidated Financial Statements in this Report also include the accounts of VistaGen California's two wholly-owned subsidiaries, Artemis Neuroscience, Inc., a Maryland corporation, and VistaStem Canada, Inc., a corporation organized under the laws of Ontario, Canada.

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Note 2. Basis of Presentation

The accompanying unaudited Condensed Consolidated Financial Statements have been prepared in accordance with accounting principles generally accepted in the United States (U.S. GAAP) for interim financial information and with the instructions to Form 10-Q and Rule 8-03 of Regulation S-X. Accordingly, they do not contain all of the information and footnotes required for complete consolidated financial statements. In the opinion of management, the accompanying unaudited Condensed Consolidated Financial Statements reflect all adjustments, which include only normal recurring adjustments, necessary to present fairly our interim financial information. The accompanying Condensed Consolidated Balance Sheet at March 31, 2016 has been derived from our audited consolidated financial statements at that date but does not include all disclosures required by U.S. GAAP. The operating results for the three months ended June 30, 2016 are not necessarily indicative of the operating results to be expected for our fiscal year ending March 31, 2017 or for any other interim period or any other future period.

The accompanying unaudited Condensed Consolidated Financial Statements and notes to Condensed Consolidated Financial Statements should be read in conjunction with our audited Consolidated Financial Statements for the fiscal year ended March 31, 2016 contained in our Annual Report on Form 10-K, as filed with the Securities and Exchange Commission (SEC) on June 24, 2016.

The accompanying Condensed Consolidated Financial Statements have been prepared assuming we will continue as a going concern. As a developing-technology company having not yet developed commercial products or achieved sustainable revenues, we have experienced recurring losses and negative cash flows from operations resulting in a deficit of $133.7 million accumulated from inception through June 30, 2016. We expect losses and negative cash flows from operations to continue for the foreseeable future as we engage in further potential development of AV-101 and launch and execute our drug rescue programs and pursue potential drug development and regenerative medicine opportunities.

Since our inception in May 1998 through June 30, 2016, we have financed our operations and technology acquisitions primarily through the issuance and sale of our equity and debt securities, including convertible promissory notes and short-term promissory notes, for cash proceeds of approximately $44.3 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 value at issuance of $29.1 million in non-cash settlements of certain liabilities, including liabilities for professional services rendered to us or as compensation for such services.

Between April 1, 2016 and May 4, 2016, we sold to accredited investors Series B Preferred Units consisting of 39,714 unregistered shares of our Series B Preferred Stock, par value $0.001 per share (Series B Preferred), and five year warrants to purchase 39,714 shares of our common stock, from which we received cash proceeds of $278,000. Further, on May 16, 2016 we consummated an underwritten public offering pursuant to which we issued an aggregate of 2,570,040 registered shares of our common stock at the public offering price of $4.24 per share and five-year warrants to purchase up to 2,705,883 registered shares of common stock, with an exercise price of $5.30 per share, at the public offering price of $0.01 per warrant, including shares and warrants issued pursuant to the exercise of the underwriters' over-allotment option (the May 2016 Public Offering). We received net cash proceeds of approximately $9.5 million from the May 2016 Public Offering after deducting fees and expenses. We expect the proceeds of these transactions to provide sufficient cash to sustain our operations through our fiscal year ending March 31, 2017, however they will not be adequate to enable the completion of our Phase 2b clinical trial of AV-101 in MDD. Accordingly, we intend to raise additional capital through sales of our securities, which may include both debt and equity securities. We may also seek research and development collaborations that could generate revenue, as well as government grant awards. Further, strategic collaborations, such as our February 2015 CRADA with the NIMH providing for the NIMH to fund our Phase 2a study of AV-101 in MDD, may provide resources to support a

portion of our future cash needs and working capital requirements. Although we may seek additional collaborations that could generate revenue, as well as new government grant awards, no assurance can be provided that any such collaborations or awards will occur in the future. Our future working capital requirements will depend on many factors, including, without limitation, the scope and nature of opportunities related to our success and the success of certain other companies in clinical trials, including our development of AV-101 as a treatment for MDD and other CNS conditions, and our stem cell technology platform, the availability of, and our ability to obtain, government grant awards and our ability to enter into collaborations on terms acceptable to us. To further advance the clinical development of AV-101 and our stem cell technology platform, as well as support our operating activities, we plan to continue to carefully manage our routine operating costs, including our employee headcount and related expenses, as well as costs relating to regulatory consulting, contract research and development, investor relations and corporate development, legal, accounting, public company compliance and other professional services and working capital costs.

Notwithstanding the foregoing, substantial additional financing may not be available to us on a timely basis, on acceptable terms, or at all. If we are unable to obtain substantial additional financing on a timely basis in the near term, our business, financial condition, and results of operations may be harmed, the price of our stock may decline, we may be required to reduce, defer, or discontinue certain of our research and development activities and we may not be able to continue as a going concern. These Condensed Consolidated Financial Statements do not include any adjustments that might result from the outcome of this uncertainty.

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Note 3. Summary of Significant Accounting Policies

Use of Estimates

The preparation of financial statements in conformity with 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 those relating to share-based compensation, and assumptions that have been used to value warrants, warrant modifications, warrant liabilities. We do not currently have, nor have we had during the periods covered by this report, any arrangements requiring the recognition of revenue.

Research and Development Expenses

Research and development expenses are composed of both internal and external costs. Internal costs include salaries and employment-related expenses of scientific personnel and direct project costs. External research and development expenses consist primarily of costs associated with nonclinical and clinical development of AV-101, now in Phase 2 clinical development, initially for Major Depressive Disorder, stem cell technology-related research and development costs, and costs related to the filing, maintenance and prosecution of patents and patent applications. All such costs are charged to expense as incurred.

Stock-Based Compensation

We recognize compensation cost for all stock-based awards to employees or consultants based on the grant date fair value of the award. Non-cash, stock-based compensation expense is recognized over the period during which the employee or consultant is required to perform services 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.

The table below summarizes stock-based compensation expense included in the accompanying Condensed Consolidated Statements of Operations and Comprehensive Loss for the three months ended June 30, 2016 and 2015.

	Three Months Ended June 30,	
	2016	2015
Research and development expense:		
Stock option grants	$44,000	$15,500
Warrants granted to officer in March 2014	-	2,800
	44,000	18,300
General and administrative expense:		
Stock option grants	63,900	7,000
Warrants granted to officers and directors in March 2014	-	3,900
	63,900	10,900
Total stock-based compensation expense	$107,900	$29,200

On June 19, 2016, our Board of Directors (Board) approved the grant of options to purchase an aggregate of 655,000 shares of our common stock at an exercise price of $3.49 per share to the independent members of our Board and to our officers, including our newly-hired Chief Medical Officer. We did not grant any stock options during the three months ended June 30, 2015. At June 30, 2016, there were stock options outstanding to purchase 986,987 shares of our common stock at a weighted average exercise price of $5.53 per share. We valued the options granted in June 2016 using the Black-Scholes Option Pricing Model and the following weighted average assumptions:

Assumption:		
Market price per share at grant date	$	3.49
Exercise price per share	$	3.49
Risk-free interest rate		1.34%
Contractual or estimated term in years		6.68
Volatility		81.69%
Dividend rate		0.0%
Shares		655,000
Fair Value per share	$	2.50

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Comprehensive Loss

We have no components of other comprehensive loss other than net loss, and accordingly our comprehensive loss is equivalent to our net loss for the periods presented.

Income (Loss) per Common Share

Basic net 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 net 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, we have historically adjusted the numerator for the change in the fair value of the warrant liability attributable to outstanding warrants, only if dilutive, and increased the denominator to include the number of potentially dilutive common shares assumed to be outstanding during the period using the treasury stock method. The change in the fair value of the warrant liability, which was eliminated in May 2015, had no impact on the diluted net earnings per share calculation in any period included in these Condensed Consolidated Financial Statements.

As a result of our net loss for the periods presented, potentially dilutive securities were excluded from the computation of net loss per share, as their effect would be antidilutive. For the three month periods ended June 30, 2016 and 2015, the accrual for dividends on our Series B Preferred and the deemed dividend attributable to the issuance of our Series B Preferred Units represent deductions from our net loss to arrive at net loss attributable to common stockholders for those periods.

Potentially dilutive securities excluded in determining diluted net loss attributable to common stockholders per common share are as follows:

	As of June 30,	
	2016	2015
Series A Preferred stock issued and outstanding (1)	750,000	750,000
Series B Preferred stock issued and outstanding (2)	1,247,740	2,995,579
Series C Preferred stock issued and outstanding (3)	2,318,012	-
Outstanding options under the 2008 and 1999 Stock Incentive Plans	986,987	206,788
Outstanding warrants to purchase common stock	4,606,480	3,735,023
Warrant shares issuable to PLTG upon exchange of Series A Preferred under the terms of the October 11, 2012 Note Exchange and Purchase Agreement, as subsequently amended	-	535,715
Total	9,909,219	8,223,105

(1) Assumes exchange under the terms of the October 11, 2012 Note Exchange and Purchase Agreement with PLTG, as amended

(2) Assumes exchange under the terms of the Certificate of Designation of the Relative Rights and Preferences of the Series B 10% Convertible Preferred Stock, effective May 5, 2015

(3) Assumes exchange under the terms of the Certificate of Designation of the Relative Rights and Preferences of the Series C Convertible Preferred Stock, effective January 25, 2016

Recent Accounting Pronouncements

There have been no recent accounting pronouncements or changes in accounting pronouncements during the three months ended June 30, 2016, as compared to the recent accounting pronouncements described in the Company's Form 10-K for the fiscal year ended March 31, 2016, that are of significance or potential significance to the Company.

Note 4. Fair Value Measurements

We do not use derivative instruments for hedging of market risks or for trading or speculative purposes.

In conjunction with certain Senior Secured Convertible Promissory Notes that we issued to Platinum Long Term Growth VII, LLC (PLTG) between October 2012 and July 2013 and the related PLTG Warrants, and the contingently issuable Series A Exchange Warrant, we determined that the warrants included certain exercise price adjustment features requiring the warrants to be treated as liabilities, which were recorded at their issuance-date estimated fair values and marked to market at each subsequent reporting period. We determined the fair value of the warrant liabilities using Level 3 (unobservable) inputs, since there was minimal comparable external market data available. Inputs used to determine fair value included the remaining contractual term of the warrants, 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 PLTG's exchange of the shares of Series A Preferred it holds into shares of common stock. The change in the fair value of these warrant liabilities between March 31, 2015 and their subsequent elimination (described below) was recognized as a non-cash expense in the Condensed Consolidated Statement of Operations and Comprehensive Loss for the three months ended June 30, 2015.

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On May 12, 2015, we entered into an agreement with PLTG pursuant to which PLTG agreed to amend the PLTG Warrants to (i) fix the exercise price thereof at $7.00 per share, (ii) eliminate the exercise price reset features and (iii) fix the number of shares of our common stock issuable thereunder. This agreement and the related modification of the PLTG Warrants resulted in the elimination of the warrant liability with respect to the PLTG Warrants during the quarter ended June 30, 2015.

In January 2016, we entered into an Exchange Agreement with PLTG pursuant to which PLTG exchanged all outstanding PLTG Warrants plus the shares issuable pursuant to the Series A Preferred Exchange Warrant for unregistered shares of our Series C Convertible Preferred Stock (Series C Preferred) in the ratio of 0.75 share of Series C Preferred for each warrant share cancelled.

We carried no assets or liabilities at fair value at June 30, 2016 or March 31, 2016.

Note 5. Prepaid Expenses and Other Current Assets

Prepaid expenses and other current assets are composed of the following at June 30, 2016 and March 31, 2016:

	June 30, 2016	March 31, 2016
Insurance	$131,500	$27,000
Prepaid compensation under financial advisory and other consulting agreements	-	337,500
Public offering expenses	-	57,400
Technology license fees and all other	3,200	4,900
	$134,700	$426,800

Note 6. Accrued Expenses

Accrued expenses are composed of the following at June 30, 2016 and March 31, 2016:

	June 30, 2016	March 31, 2016
Accrued professional services	$326,900	$318,000
Accrued AV-101 development expenses	$232,900	$186,000
Accrued compensation	-	310,000
All other	5,000	-
	$564,800	$814,000

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Note 7. Notes Payable

The following table summarizes our unsecured promissory notes at June 30, 2016 and March 31, 2016.

	June 30, 2016			March 31, 2016		
	Principal Balance	Accrued Interest	Total	Principal Balance	Accrued Interest	Total
5.75% Note payable to insurance premium financing company (current)	$105,900	$-	$105,900	$-	$-	$-
7.0% Note payable to Progressive Medical Research	$-	$-	$-	$58,800	$12,000	$70,800
less: current portion	-	-	-	(31,600)	(12,000)	(43,600)
7.0% Notes payable - non-current portion	$-	$-	$-	$27,200	$-	$27,200
Total notes payable to unrelated parties	$105,900	$-	$105,900	$58,800	$12,000	$70,800
less: current portion	(105,900)	-	(105,900)	(31,600)	(12,000)	(43,600)
Net non-current portion	$-	$-	$-	$27,200	$-	$27,200

Between May 2015 and August 2015, we extinguished the outstanding balances of approximately $17.2 million of indebtedness, including all senior secured promissory notes, all except $85,100 principal of unsecured promissory notes, and a substantial portion of other indebtedness, and certain adjustments thereto, that were either due and payable or would have become due and payable prior to March 31, 2016, by converting all such indebtedness into shares of our Series B Preferred. Evaluating each note or debt class separately, we determined that the conversion of each of the notes or other debt instruments into Series B Preferred should be accounted for as an extinguishment of debt. Because the fair value of the Series B Preferred into which the debt instruments were converted in all cases exceeded the carrying value of the debt, we recorded an aggregate loss on extinguishment of debt of $26,700,200, in our fiscal year ended March 31, 2016, of which $25,050,900 was recorded in the quarter ended June 30, 2015, as reflected in the accompanying Consolidated Statement of Operations and Comprehensive Loss for that period.

On January 5, 2016, we paid in full the $33,300 outstanding balance (principal and accrued but unpaid interest) of the promissory note previously issued to University of California at Davis.

On June 13, 2016, we paid in full the $71,600 outstanding balance (principal and accrued but unpaid interest) of the promissory note we issued to Progressive Medical Research in August 2012.

In May 2016, we executed a promissory note in the face amount of $117,500 to finance certain insurance policy premiums. The note is payable in monthly installments of $12,100, including principal and interest, through March 2017.

Note 8. Capital Stock

Series B Preferred Unit Offering

In April and May 2016, in self-placed private placement transactions, we sold to accredited investors an aggregate of $278,000 of units in our Series B Preferred Unit offering, which units consist of Series B Preferred and Series B Warrants (together Series B Preferred Units). We issued 39,714 shares of Series B Preferred and Series B Warrants to purchase 39,714 shares of our common stock. Through the termination of the Series B Preferred Unit offering in May 2016, we received an aggregate of $5,303,800 in cash proceeds from our self-placed private placement and sale of the Series B Preferred Units.

We allocated the proceeds from the sale of the Series B Preferred Units during the quarter ended June 30, 2016 to the Series B Preferred and the Series B Warrants based on their relative fair values on the dates of the sales. We determined that the fair value of a share of Series B Preferred was equal to the quoted market value of a share of our common stock on the date of a Series B Preferred Unit sale. We calculated the fair value of the Series B Warrants using the Black Scholes Option Pricing Model and the weighted average assumptions indicated in the table below. The table below also presents the aggregate allocation of the Series B Preferred Unit sales proceeds based on the relative fair values of the Series B Preferred and the Series B Warrants as of their respective Series B Preferred Unit sales dates. The difference between the relative fair value per share of the Series B Preferred, approximately $4.20 per share, and its Conversion Price (or stated value) of $7.00 per share represents a deemed dividend to the purchasers of the Series B Preferred Units. Accordingly, we have recognized a deemed dividend in the aggregate amount of $111,100 in arriving at net loss attributable to common stockholders in the accompanying Condensed Consolidated Statement of Operations and Comprehensive Loss for the three months ended June 30, 2016.

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Unit Warrants											
	Weighted Average Issuance Date Valuation Assumptions									Aggregate Allocation of Proceeds Based on Relative Fair Value of:	
Warrant Shares Issued	Market Price	Exercise Price	Term(Years)	Risk free Interest Rate	Volatility	Dividend Rate	Per Share Fair Value of Warrant	Aggregate Fair Value of Unit Warrants	Aggregate Proceeds of Unit Sales	Unit Stock	Unit Warrant
39,714	$8.45	$7.00	5.00	1.27 %	78.43 %	0.0 %	$5.63	$223,500	$278,000	$166,900	$61,100

May 2016 Public Offering

Effective on May 16, 2016, we consummated a fully underwritten public offering, pursuant to which we issued an aggregate of 2,570,040 registered shares of our common stock at a public sales price of $4.24 per share and five-year warrants exercisable at $5.30 per share to purchase an aggregate of 2,705,883 shares of our common stock at a public sales price of $0.01 per warrant share, including shares and warrants issued in June 2016 pursuant to the exercise of the underwriters' over-allotment option (the May 2016 Public Offering). We received gross proceeds of approximately $10.9 million and net proceeds of approximately $9.5 million from the May 2016 Public Offering after deducting underwriters' commissions and other expenses. The warrants issued in the May 2016 Public Offering have no anti-dilution or other exercise price or share reset features, except as is customary with respect to a change in the Company's capital structure in the event of a stock split or dividend, and, accordingly, we have accounted for them as equity warrants.

The securities included in the May 2016 Public Offering Warrants were offered, issued and sold under a prospectus filed with the Securities and Exchange Commission (SEC) pursuant to an effective registration statement (Registration Statement) filed with the SEC on Form S-1 (File No. 333-210152) pursuant to the Securities Act of 1933, as amended (Securities Act). The Registration Statement was first filed with the SEC on March 14, 2016, and was declared effective on May 10, 2016.

NASDAQ Uplisting

In connection with the completion of our May 2016 Public Offering, our common stock was approved for listing on The NASDAQ Capital Market, and began trading under the symbol "VTGN" on May 11, 2016.

Conversion of Series B Preferred into Common Stock

During April 2016, prior to the May 2016 Public Offering, holders of an aggregate of 7,500 shares of Series B Preferred voluntarily converted such shares into an equivalent number of registered shares of our common stock. In connection with such conversions, we issued an aggregate of 510 shares of our unregistered common stock in payment of $4,000 in accrued dividends on the Series B Preferred converted.

On May 19, 2016, upon the consummation of the May 2016 Public Offering, an aggregate of 2,403,051 shares of Series B Preferred were automatically converted into an aggregate of 2,192,847 registered shares of our common stock and an aggregate of 210,204 shares of our unregistered common stock. Additionally, we issued an aggregate of 416,806 shares of our unregistered common stock in payment of $1,642,100 in accrued dividends, at the rate of one share of common stock for each $3.94 of accrued dividends. On June 15, 2016, pursuant to the underwriters' exercise of their over-allotment option, an additional 44,500 shares of Series B Preferred were converted into 44,500 shares of our registered common stock. We issued an additional 9,580 shares of our unregistered common stock in payment of

$37,400 in accrued dividends, at the rate of one share of common stock for each $3.90 in accrued dividends.

Modification of Warrants

Between April 1, 2016 and May 4, 2016, we entered into Warrant Exchange Agreements with certain holders of outstanding warrants to purchase an aggregate of 41,469 shares of our common stock pursuant to which the holders agreed to the cancellation of such warrants in exchange for our issuance to them of an aggregate of 31,238 shares of our unregistered common stock.

We accounted for the exchange of these warrants as warrant modifications, comparing their fair value prior to the exchange with the fair value of the common stock issued. We calculated the weighted average fair value of the warrants prior to the exchange to be $5.37 per share, or $223,700, using the Black Scholes Option Pricing Model and the following weighted average assumptions: market price per share: $8.44; exercise price per share: $7.37; risk-free interest rate: 1.23%; remaining contractual term: 4.77 years; volatility: 79.0%; expected dividend rate: 0%. The weighted average fair value of the aggregate of 31,238 shares of common stock issued in the exchange was $8.45 per share or $264,000. Accordingly, we recognized the additional fair value, $40,300, as warrant modification expense, included as a component of general and administrative expenses in the accompanying Condensed Consolidated Statement of Operations and Comprehensive Loss for the three months ended June 30, 2016.

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Warrants Outstanding

Following the warrant issuances in the May 2016 Public Offering, the Series B Warrant issuances and the warrant exchanges described above, at June 30, 2016, we had outstanding warrants to purchase shares of our unregistered common stock at a weighted average exercise price of $6.48 per share as follows:

Exercise Price per Share	Expiration Date	Shares Subject to Purchase at June 30, 2016
$ 5.30	5/16/2021	2,705,883
$ 7.00	12/11/2018 to 3/3/2023	1,417,125
$ 8.00	3/25/2021	230,000
$ 10.00	8/31/2016 to 1/11/2020	135,384
$ 15.00	8/31/2016	4,040
$ 20.00	9/15/2019	110,448
$ 30.00	11/20/2017	3,600
		4,606,480

Note 9. Related Party Transactions

Cato Holding Company (CHC), doing business as Cato BioVentures (CBV), and the parent of Cato Research Ltd (CRL) a contract research organization (CRO) engaged with us for the development of AV-101, is one of our largest institutional stockholders at June 30, 2016, holding approximately 7% of our outstanding common stock. Shawn Singh, our Chief Executive Officer and member of our Board of Directors, served as Managing Principal of CBV and as an officer of CRL until August 2009. On October 10, 2012, we issued to CHC an unsecured promissory note in the principal amount of $310,400 (the 2012 CHC Note) and a five-year warrant to purchase 12,500 restricted shares of our common stock at a price of $30.00 per share (the CHC Warrant). Additionally, on October 10, 2012, we issued to CRL: (i) an unsecured promissory note in the initial principal amount of $1,009,000, payable solely in restricted shares of our common stock and which accrued interest at the rate of 7.5% per annum, compounded monthly (the CRL Note), as payment in full for all contract research and development services and regulatory advice rendered to us by CRL through December 31, 2012 with respect to the preclinical and clinical development of AV-101, and (ii) a five-year warrant to purchase, at a price of $20.00 per share, 50,450 restricted shares of our common stock, such number of shares to be adjusted in relation to accrued interest on the CRL Note (CRL Warrant). The Cato Notes and additional amounts payable to CRL for CRO services were extinguished in June 2015 in exchange for our issuance of an aggregate of 328,571 shares of Series B Preferred to CHC, which shares of Series B Preferred were automatically converted into an equal number of registered shares of our common stock in connection with the May 2016 Public Offering. CHC also participated in our February 2016 warrant exchange, exchanging the CHC Warrant and the CRL Warrant, as adjusted to reflect accrued interest, for an aggregate of 54,894 shares of our unregistered common stock.

Under the terms of VistaGen California's contract research organization arrangement with CRL related to the development of AV-101, we incurred expenses of $50,400 and $11,200 for the three month periods ended June 30, 2016 and 2015, respectively. Total interest expense, including amortization of note discount, on the notes payable to CHC and CRL was $28,200 for the three month period ended June 30, 2015.

Note 10. Subsequent Events

We have evaluated subsequent events through the date of this report and have found no material matters requiring disclosure.

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Item 2. MANAGEMENT'S DISCUSSION AND ANALYSIS OF FINANCIAL CONDITION AND RESULTS OF OPERATIONS

Cautionary Note Regarding Forward-Looking Statements

This Quarterly Report on Form 10-Q (Quarterly Report) includes forward-looking statements. All statements contained in this Quarterly Report 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. Our business is subject to significant risks including, but not limited to, our ability to obtain additional financing, the results of our research and development efforts, the results of non-clinical and clinical testing, the effect of regulation by the United States Food and Drug Administration (FDA) and other agencies, the impact of competitive products, product development, commercialization and technological difficulties, the effect of our accounting policies, and other risks as detailed in the section entitled "Risk Factors" in this Quarterly Report. Further, even if our product candidates appear promising at various stages of development, our share price may decrease such that we are unable to raise additional capital without significant dilution or other terms that may be unacceptable to our management, Board of Directors and stockholders.

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 Quarterly Report 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 Quarterly Report 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 clinical-stage biopharmaceutical company dedicated to developing and commercializing innovative product candidates for patients with diseases and disorders involving the central nervous system (CNS). Unless the context otherwise requires, the words "VistaGen Therapeutics, Inc." "VistaGen," "we," "the Company," "us" and "our" refer to Vista Therapeutics, Inc., a Nevada corporation.

Our lead product candidate, AV-101, is a new generation, oral antidepressant drug candidate in Phase 2 development for the adjunctive treatment of Major Depressive Disorder (MDD) in patients with an inadequate response to standard antidepressants approved by the U.S. Food and Drug Administration (FDA). We believe AV-101 may also have therapeutic potential in chronic neuropathic pain, epilepsy, Huntington's disease and Parkinson's disease.

AV-101's mechanism of action, as an N-methyl D aspartate receptor (NMDAR) antagonist binding selectively at the glycine binding (GlyB) co-agonist site of the NMDAR, is fundamentally differentiated from all FDA-approved antidepressants, as well as all atypical antipsychotics used to augment standard antidepressant therapy.

Our ongoing Phase 2a clinical study of AV-101 in subjects with treatment-resistant MDD is being conducted and fully funded by the U.S. National Institute of Mental Health (NIMH) under our February 2015 Cooperative Research and Development Agreement (CRADA) with the NIMH. The first patient in this NIMH-sponsored Phase 2a study was dosed in November 2015. The Principal Investigator of the study is Dr. Carlos Zarate, Jr., Chief of the NIMH's Experimental Therapeutics & Pathophysiology Branch and its Section on Neurobiology and Treatment of Mood and Anxiety Disorders. Previous NIMH studies, including studies conducted by Dr. Zarate, have focused on the antidepressant effects of low dose, intravenous (I.V.) ketamine in patients with treatment-resistant depression. These NIMH studies, as well as clinical research by others, have demonstrated robust antidepressant effects in patients with treatment-resistant MDD within twenty-four hours of a single low dose of I.V. ketamine. We believe AV-101 may have potential to deliver ketamine-like fast-acting antidepressant effects without ketamine's serious side effects.

We are preparing to launch our Phase 2b clinical study of AV-101 for the adjunctive treatment of MDD in patients with an inadequate response to standard, FDA-approved antidepressants. We anticipate commencement of this multi-center, multi-dose, double blind, placebo-controlled Phase 2b efficacy and safety study at the end of the fourth quarter of 2016. Dr. Maurizio Fava, Professor of Psychiatry at Harvard Medical School and Director, Division of Clinical Research, Massachusetts General Hospital (MGH) Research Institute, will be the Principal Investigator of our Phase 2b study of AV-101 in treatment-resistant MDD. Dr. Fava was the co-Principal Investigator with Dr. A. John Rush of the largest clinical trial conducted in depression to date, the STAR*D study, whose findings were published in journals such the New England Journal of Medicine (NEJM) and the Journal of the American Medical Association (JAMA). We currently anticipate top line results in this Phase 2b study in the second quarter of 2018.

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In addition to clinical development of AV-101, we are focused on advancing potential commercial applications of our human pluripotent stem cell (hPSC) technology platform with respect to drug rescue programs aimed at developing proprietary small molecule new chemical entities (NCEs) for our drug candidate pipeline. We are also focused on potential regenerative medicine (RM) applications using blood, cartilage, heart and/or liver cells derived from hPSCs, and may pursue these applications in collaboration with third-parties.

AV-101 and Major Depressive Disorder

Background

The World Health Organization (WHO) estimates that 350 million people worldwide are affected by depression. According to the U.S. National Institutes of Health (NIH) major depression is one of the most common mental disorders in the U.S. The NIMH reports that, in 2014, an estimated 15.7 million adults aged 18 or older in the U.S. had at least one major depressive episode in the past year. This represented 6.7 percent of all U.S. adults. According to the U.S. Centers for Disease Control and Prevention (CDC) one in 10 Americans over the age of 12 takes an antidepressant medication.

Most standard, FDA-approved antidepressants target neurotransmitter reuptake inhibition – either serotonin (SSRIs) or serotonin/norepinephrine (SNRIs). Even when effective, these standard depression medications take many weeks to achieve adequate antidepressant effects. Nearly two out of every three drug-treated depression patients, including an estimated 6.9 million drug-treated MDD patients in the U.S., obtain inadequate therapeutic benefit from initial treatment with a standard antidepressant. Unfortunately, even after treatment with as many as four different standard antidepressants, nearly one out of every three drug-treated depression patients do not achieve adequate therapeutic benefits. Such treatment-resistant depression patients often seek to treat their depression with non-drug-related approaches, such as Electroconvulsive Therapy (ECT), or to augment their inadequate response to standard antidepressants by adding an atypical antipsychotic (such as, for example, aripiprazole) to their treatment regimen, despite the modest potential therapeutic benefit and significant risk of additional side effects from such augmentation options.

All standard antidepressants have risks of significant side effects, including, among others, potentially anxiety, metabolic syndrome, sleep disturbance and sexual dysfunction. They also have a “Black Box” warning due to risks of worsening depression and suicide in certain groups. Use of atypical antipsychotics to augment inadequately performing standard antidepressants increases the risk of serious side effects, including, potentially, tardive dyskinesia, significant weight gain, diabetes and heart disease, while offering only a modest potential increase in therapeutic benefit. Use of ECT increases the risk of serious side effects, including, headaches, tiredness, disorientation, intense sleepiness, hallucinations and long-term memory loss.

AV-101

AV-101, our oral new generation antidepressant drug candidate, is in Phase 2 clinical development for the adjunctive treatment of MDD patients with an inadequate response to standard antidepressants. As published in the October 2015 issue of the peer-reviewed, Journal of Pharmacology and Experimental Therapeutics, in an article entitled, The prodrug 4-chlorokynurenine causes ketamine-like antidepressant effects, but not side effects, by NMDA/glycineB-site inhibition, using well-established preclinical models of depression, AV-101 was shown to induce fast-acting, dose-dependent, persistent and statistically significant antidepressant-like responses, following a single treatment. These responses were equivalent to those seen with a single, sub-anesthetic control dose of the NMDAR antagonist ketamine. In the same preclinical studies, a standard antidepressant, the SSRI fluoxetine, did not induce rapid onset antidepressant-like responses. In addition, these studies confirmed that the fast-acting antidepressive effects of AV-101 were mediated through the GlyB site and involved the activation of a key neurological pathway, the

alpha-amino-3-hydroxy-5-methyl-4-isoxazolepropionic acid (AMPA) receptor pathway. Activation of the AMPA receptor pathway is a common feature of fast-acting antidepressants.

Following the completion of our NIH-funded, randomized, double blind, placebo-controlled Phase 1a and Phase 1b safety studies, we are now collaborating with the NIMH in a Phase 2a study. Under our February 2015 CRADA, the NIMH is funding, and Dr. Carlos Zarate Jr. of the NIMH as Principal Investigator is conducting, our ongoing Phase 2a efficacy and safety study of AV-101 in subjects with treatment-resistant MDD. The trial is expected to enroll 20 to 28 patients. The first patient was dosed in November 2015, and we currently anticipate receiving topline results in the second quarter of 2017.

We are preparing to launch our Phase 2b clinical study of AV-101 for the adjunctive treatment of MDD in patients with an inadequate response to standard, FDA-approved antidepressants. We anticipate the launch of this Phase 2b study, with Dr. Maurizio Fava of Harvard Medical School serving as Principal Investigator, at the end of the fourth quarter of 2016. We anticipate top line results from this Phase 2b study in the second quarter of 2018.

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Several preclinical studies support the hypothesis that AV-101 also has the potential to treat multiple CNS disorders and neurodegenerative diseases in addition to depression, including chronic neuropathic pain, epilepsy, Parkinson's disease and Huntington's disease, where modulation of the NMDAR, AMPA pathway and/or active metabolites of AV-101 may achieve therapeutic benefit.

CardioSafe 3D™; NCE Drug Rescue and Regenerative Medicine

CardioSafe 3D™ is our customized in vitro cardiac bioassay system capable of predicting potential human heart toxicity of small molecule NCEs in vitro, long before they are ever tested in animal and human studies. Our current strategic interests involving our stem cell technology platform include (i) advancing current internal efforts focused on CardioSafe 3D drug rescue to expand our drug candidate pipeline with selected proprietary small molecule NCEs, leveraging substantial prior research and development investments by pharmaceutical companies and others related to public domain NCEs terminated before FDA approval due to heart toxicity risks and (ii) establishing collaborative arrangements with qualified third-parties focused on regenerative medicine (RM) applications, including (A) cell-based therapy (injection of stem cell-derived mature organ-specific cells obtained through directed differentiation), (B) cell repair therapy (induction of regeneration by biologically active molecules administered alone or produced by infused genetically engineered cells), or (C) tissue engineering (transplantation of in vitro grown complex tissues), involving hPSC-derived blood, bone, cartilage, heart and/or liver cells.

Financial Operations Overview and Results of Operations

Our critical accounting policies and estimates and recent accounting pronouncements are disclosed in our Annual Report on Form 10-K for the fiscal year ended March 31, 2016, as filed with the SEC on June 24, 2016, and in Note 3 to the accompanying unaudited Condensed Consolidated Financial Statements included in Part 1, Item 1 of this Quarterly Report on Form 10-Q.

Summary

Net Loss

We have not yet achieved revenue-generating status from any of our product candidates. Since inception, we have devoted substantially all of our time and efforts to developing AV-101 from early preclinical studies to our ongoing Phase 2a clinical study in MDD, as well as stem cell research and bioassay development, small molecule drug development, and creating, protecting and patenting intellectual property, with the corollary initiatives of recruiting personnel and raising working capital. As of June 30, 2016, we had an accumulated deficit of approximately $133.7 million. Our net loss for the quarter ended June 30, 2016 was approximately $2.0 million. Our net loss for the quarter ended June 30, 2015 was $29.5 million, including a non-recurring, non-cash loss of approximately $25.1 million attributable to converting over $15.4 million of our indebtedness into equity securities during May and June 2015. We expect losses to continue for the foreseeable future, primarily related to our further development of AV-101 for MDD and additional CNS indications.

Summary of Three Months Ended June 30, 2016

During the quarter ended June 30, 2016, we have continued to advance clinical development of AV-101 for depression and other CNS indications and, on a more limited basis, explore drug rescue and regenerative medicine opportunities related to our stem cell technology platform. Pursuant to our February 2015 Cooperative Research and Development Agreement (CRADA) with the NIH, the NIH continues to fund and conduct a Phase 2a clinical study of AV-101 in treatment-resistant MDD. We are preparing to launch a Phase 2b clinical study of AV-101 for the adjunctive treatment of MDD in patients with an inadequate response to standard, FDA-approved antidepressants

(Phase 2b Study). Following the enabling consummation of the May 2016 Public Offering (described below), we anticipate the launch of the Phase 2b Study, with Dr. Maurizio Fava of Harvard Medical School serving as Principal Investigator, at the end of the fourth calendar quarter of 2016. In May 2016, we consummated a fully underwritten public offering, pursuant to which we issued an aggregate of 2,570,040 registered shares of our common stock and five-year warrants exercisable at $5.30 per share to purchase an aggregate of 2,705,883 shares of our common stock and received net proceeds, after deducting underwriters' commissions and other expenses, of approximately $9.5 million (May 2016 Public Offering). While we expect the proceeds from the May 2016 Public Offering to provide sufficient cash to sustain our operations through our fiscal year ending March 31, 2017 and to enable advance preparations for and commencement of our Phase 2b Study, they will not be adequate to enable the completion of our Phase 2b Study. Accordingly, our executive management continues to focus significant efforts on raising additional capital to complete the Phase 2b Study and for other operational requirements through sales of our securities, which may include both debt and equity securities, or from other sources.

A further significant accomplishment during the quarter ended June 30, 2016, is the June 2016 addition to our management team of Mark A. Smith, MD, Ph.D., as our Chief Medical Officer. Dr. Smith has over 20 years of pharmaceutical industry experience CNS drug development. He has been a successful project leader in both drug discovery and development on projects resulting in approximately 20 investigational new drugs (INDs). Dr. Smith has directed clinical trials examining depression, bipolar disorder, anxiety, schizophrenia, Alzheimer's disease, ADHD and agitation in Phase 1 through Phase 2b. In addition, Dr. Smith has vast knowledge and expertise in translational neuroscience, clinical trial design and regulatory interactions.

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

Results of Operations

Comparison of Three Months Ended June 30, 2016 and 2015

The following table summarizes the results of our operations for the three months ended June 30, 2016 and 2015 (amounts in thousands).

	Three Months Ended June 30,	
	2016	2015
Operating expenses:		
Research and development	$826	$373
General and administrative	1,138	1,448
Total operating expenses	1,964	1,821
Loss from operations	(1,964)	(1,821)
Interest expense, net	(1)	(755)
Change in warrant liabilities	-	(1,895)
Loss on extinguishment of debt	-	(25,051)
Loss before income taxes	(1,965)	(29,522)
Income taxes	(2)	(2)
Net loss	$(1,967)	$(29,524)
Accrued dividend on Series B Preferred Stock	(540)	(213)
Deemed dividend on Series B Preferred Stock	(111)	(256)
Net loss attributable to common stockholders	$(2,618)	$(29,993)

Revenue

We reported no revenue for the quarters ended June 30, 2016 or 2015 and we presently have no revenue generating arrangements. However, as indicated previously, we have entered into a CRADA with the NIH providing for a Phase 2a clinical study of AV-101 in treatment resistant Major Depressive Disorder. This Phase 2a study, which began in late-2015, is being funded by the NIH and conducted at the NIMH.

Research and Development Expense

Research and development expense totaled $825,700 for the quarter ended June 30, 2016, more than at two-fold increase when compared with the $372,600 reported for the quarter ended June 30, 2015. This increase is primarily

the result of increased spending on the development of AV-101 and legal and application costs related to a number of U.S. and foreign pending patent applications with respect to AV-101 and our stem cell technology platform. The following table indicates the primary components of research and development expense for each of the periods (amounts in thousands):

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	Three Months Ended June 30,	
	2016	2015
Salaries and benefits	$250	$202
Stock-based compensation	44	18
Consulting and other professional services	27	22
Technology licenses and royalties	160	53
Project-related research and supplies:		
AV-101	252	11
Stem cell and all other	28	2
	280	13
Rent	56	53
Depreciation	9	11
All other	-	1
Total Research and Development Expense	$826	$373

The increase in salaries and benefits is primarily attributable to bonus payments made to the four non-officer members of our scientific staff in June 2016 and the hiring of Dr. Mark Smith as our Chief Medical Officer (CMO) in June 2016.

Stock based compensation expense reflects the ratable amortization of option grants made to our Chief Scientific Officer (CSO) and CMO, scientific staff and consultants, most recently in June 2016 (CSO and CMO only) and September 2015 (in 2016 expense only). Our stock options are generally amortized over a two-year or four-year vesting period. Essentially all of the option grants made in or prior to October 2013 and in March 2014 became fully-vested and were fully-expensed prior to the quarter ended June 30, 2016.

Consulting services reflects fees paid or accrued for scientific services rendered to us by third-parties, primarily by members of our scientific and clinical advisory board.

Technology license expense reflects both recurring annual fees as well as legal counsel and other costs related to patent prosecution and protection that we are required to fund under the terms of certain of our stem cell technology license agreements or have elected to pursue for commercial purposes. We recognize these costs as they are invoiced to us by the licensors and they do not occur ratably throughout the year or between years. Additionally, in the quarter ended June 30, 2016, this expense includes legal counsel and other costs we have incurred to advance in the U.S. and numerous foreign countries several pending patent applications with respect to AV-101 and our stem cell technology platform.

AV-101 project expenses in both periods reflect the costs associated with monitoring for and responding to potential feedback related to the Phase 1 clinical trial and preparing other reports required under the terms of our prior NIH grant, primarily through our contract research collaborator, Cato Research Ltd. Expense for the quarter ended June 30, 2016 also includes costs incurred to explore and develop more efficient and cost-effective production methods for AV-101 and certain pre-production studies and procedures as well as costs related to updating documentation to facilitate the Phase 2 clinical trial of AV-101 in treatment resistant MDD sponsored and conducted by the NIH. Stem cell and other project related expenses in both periods were minimal.

General and Administrative Expense

General and administrative expense decreased to $1,137,600 from $1,448,500, for the quarters ended June 30, 2016 and 2015, respectively, primarily as a result of the decrease in noncash expense related to both grants of equity securities in payment of certain professional services during 2015 and the modification of certain outstanding warrants in June 2015. The following table indicates the primary components of general and administrative expenses for each of the periods (amounts in thousands):

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	Three Months Ended June 30,	
	2016	2015
Salaries and benefits	$190	$176
Stock-based compensation	64	11
Consulting Services	33	28
Legal, accounting and other professional fees	542	959
Investor relations	108	34
Insurance	40	38
Travel expenses	49	17
Rent and utilities	40	37
Warrant modification expense	40	122
All other expenses	32	26
Total General and Administrative Expense	$1,138	$1,448

The increase in salaries and benefits is primarily attributable to a bonus payment made to a non-officer member of our administrative staff in June 2016 and the change in that employee's status from part-time to full-time effective in November 2015.

Stock based compensation expense reflects the ratable amortization of option grants made to our Chief Executive Officer (CEO) and Chief Financial Officer (CFO), independent members of our Board of Directors and administrative staff and consultants, most recently in June 2016 (CEO, CFO and independent Board members only) and September 2015 (in 2016 expense only). Our stock options are generally amortized over a two-year or four-year vesting period. Essentially all of the option grants made in or prior to October 2013 and in March 2014 became fully-vested and were fully-expensed prior to the quarter ended June 30, 2016.

Consulting services primarily includes fees recognized for the services of independent members of our Board of Directors. We added an additional independent director to our Board in March 2016.

Legal, accounting and other professional fees in the quarter ended June 30, 2015 included non-cash expense related to the grant of an aggregate of 50,000 shares of our common stock having a fair value of $500,000 pursuant to two corporate development contracts initiated during that quarter and the grant of 25,000 shares of our Series B Preferred having a fair value of $250,000 to legal counsel as compensation for services in connection with our debt restructuring and other corporate finance matters. Expense for the quarter ended June 30, 2016 included $337,500 of noncash expense recognized during the quarter pursuant to the June 30, 2015 grant of an aggregate of 90,000 shares of our Series B Preferred having an aggregate fair value of $1,350,000 as compensation for financial advisory and corporate development service contracts with two independent contractors for services to be performed through June 30, 2016. In both years, professional services fees also include the expense related to the annual audit of the prior year financial statements.

Investor relations expense includes the fees of our external service providers and, in the quarter ended June 30, 2016, initiatives that include meetings and other communication activities focused on expanding market awareness of the Company, including among investment professionals and advisors, and individual and institutional investors, both before and following the May 2016 Public Offering.

In both periods, travel expense reflects costs associated with presentations to and meetings with existing and potential investors in connection with self-placed private placement offerings of our securities and/or the May 2016 Public

Offering, as well as various investor relations and market awareness initiatives.

In April and May 2016, we entered into warrant exchange agreements with certain warrant holders pursuant to which the warrant holders exchanged outstanding warrants to purchase an aggregate of 41,649 shares of our common stock for an aggregate of 31,238 shares of our unregistered common stock. As we had with similar transactions during the quarter ended March 31, 2016, we accounted for these transactions as warrant modifications, resulting in our recognition of $40,300 in noncash expense in the quarter ended June 30, 2016. Warrant modification expense in the quarter ended June 30, 2015 reflects the impact of June 2015 strategic reductions in the exercise price of certain outstanding warrants, generally from $30.00 per share to $10.00 per share.

Interest and Other Expenses, Net

Interest expense, net totaled $1,400 for the quarter ended June 30, 2016, a significant decrease compared to the $755,100 reported for the quarter ended June 30, 2015, resulting from the extinguishment of a substantial portion of our promissory notes, as well as other indebtedness, during May and June 2015 by conversion into our Series B Preferred and the related elimination of note interest and discount amortization. The following table summarizes the primary components of interest expense for each of the periods (amounts in thousands):

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	Three Months Ended June 30,	
	2016	2015
Interest expense on promissory notes	$1	$193
Amortization of discount on promissory notes	-	551
Other interest expense, including on capital leases and premium financing	-	1
	1	745
Effect of foreign currency fluctuations on notes payable	-	10
Interest income	-	-
Interest expense, net	$1	$755

Interest expense on promissory notes in the quarter ended June 30, 2016 represents only the quarterly interest accrued on our August 2012 note to Progressive Medical Research prior to its repayment in June 2016. The substantial overall decrease in interest expense on promissory notes and the related amortization of discounts on such notes between the periods reflects the cessation of interest accrual and discount amortization upon the extinguishment and conversion of all outstanding Senior Secured Convertible Notes, certain 10% convertible notes (2014 Unit Notes) and other outstanding promissory notes into shares of our Series B Preferred between May 2015 and June 2015.

Under the terms of our October 2012 Note Exchange and Purchase Agreement with Platinum Long Term Growth VII, LLC (PLTG), we issued certain Senior Secured Convertible Promissory Notes and a related Exchange Warrant and Investment Warrants between October 2012 and July 2013. Upon PLTG's exchange of the shares of our Series A Preferred Stock held by PLTG into shares of our common stock, we will also be required to issue a Series A Exchange Warrant to PLTG. We determined that the various warrants included certain exercise price adjustment features requiring us to treat the warrants as liabilities. Accordingly, we recorded a noncash warrant liability at its estimated fair value as of the date of warrant issuance or contract execution. As described in Note 4, Fair Value Measurements, to the Condensed Consolidated Financial Statements included in Part 1, Item 1 of this Quarterly Report on Form 10-Q, on May 12, 2015, we entered into an agreement with PLTG pursuant to which we amended the various warrants to fix the exercise price thereof and eliminate the anti-dilution reset features that had previously required the warrants to be treated as liabilities and carried at fair value. Accordingly, during the quarter ended June 30, 2015, we adjusted these warrants to their fair value, reflecting an increase of $1,894,700 since March 31, 2015, resulting primarily from the increase in the market price of our common stock in relation to the exercise price of the warrants, and then subsequently eliminated the entire warrant liability with respect to these warrants. In January 2016, the PLTG warrants were exchanged for shares of our Series C Preferred stock.

Between May 2015 and June 2015, we extinguished the then-outstanding balances of approximately $15.4 million of promissory notes, including our Senior Secured Notes, our 2014 Unit Notes and other debt and certain adjustments thereto that were either already due and payable or would have otherwise matured prior to March 31, 2016 by converting such balances into shares of our Series B Preferred. We treated the conversion of the indebtedness into Series B Preferred as extinguishments of debt for accounting purposes. Since the fair value of the Series B Preferred we negotiated in settlement of the promissory notes and other indebtedness exceeded the carrying value of the debts, we incurred noncash losses on each of the extinguishments. Additionally, under the terms of our May 2015 agreement with PLTG in which they agreed to, among other things, convert the Senior Secured Notes and certain other of our convertible promissory notes into Series B Preferred, we issued to PLTG 400,000 shares of Series B Preferred having an aggregate fair value of $4.0 million and Series B Warrants to purchase 1.2 million shares of our common stock having an aggregate of fair value of $8,270,900. We recognized this aggregate fair value as an additional noncash component of loss on extinguishment of debt. Many of the 2014 Unit Notes that were converted into Series B Preferred contained a beneficial conversion feature at the time they were originally issued. We accounted for the

repurchase of the beneficial conversion feature at the time the 2014 Unit Notes were extinguished and converted, an aggregate of $2,237,100, as a reduction to the loss on extinguishment of debt. We recorded an aggregate net noncash loss of $25.1 million attributable to the extinguishment of the indebtedness converted into Series B Preferred during the quarter ended June 30, 2015.

We allocated the proceeds from the self-placed private placement sales of Series B Preferred Units to the Series B Preferred and the Series B Warrants based on their relative fair values on the dates of the sales. The difference between the relative fair value per share of the Series B Preferred, approximately $4.20 per share and $4.05 per share for the quarters ended June 30, 2016 and 2015, respectively, and its Conversion Price (or stated value) of $7.00 per share represents a deemed dividend to the purchasers of the Series B Preferred Units. Accordingly, we recognized a deemed dividend in the aggregate amount of $111,100 and $256,200 in arriving at net loss attributable to common stockholders for the quarters ended June 30, 2016 and 2015, respectively, in the accompanying Condensed Consolidated Statement of Operations and Comprehensive Loss included in Part I of this Report. Further, we have recognized $539,800 and $213,300 for the quarters ended June 30, 2016 and 2015, respectively, representing the 10% cumulative dividend payable on our Series B Preferred as an additional deduction in arriving at net loss attributable to common stockholders in the accompanying Condensed Consolidated Statement of Operations and Comprehensive Loss, included elsewhere in this Report.

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Liquidity and Capital Resources

Since our inception in May 1998 through June 30, 2016, we have financed our operations and technology acquisitions primarily through the issuance and sale of our equity and debt securities, including convertible promissory notes and short-term promissory notes, for cash proceeds of approximately $44.3 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 value at issuance of $29.1 million in non-cash settlements of certain liabilities, including liabilities for professional services rendered to us or as compensation for such services.

Between April 1, 2016 and May 4, 2016, we sold to accredited investors Series B Preferred Units consisting of 39,714 unregistered shares of our Series B Preferred Stock, par value $0.001 per share (Series B Preferred), and five year warrants to purchase 39,714 shares of our common stock, and we received cash proceeds of $278,000. Further, on May 16, 2016 we consummated the May 2016 Public Offering, an underwritten public offering pursuant to which we issued an aggregate of 2,570,040 registered shares of our common stock at the public offering price of $4.24 per share and five-year warrants to purchase up to 2,705,883 registered shares of common stock, with an exercise price of $5.30 per share, at the public offering price of $0.01 per warrant, including shares and warrants issued pursuant to the exercise of the underwriters' over-allotment option. We received net cash proceeds of approximately $9.5 million from the May 2016 Public offering after deducting fees and expenses. We expect the proceeds of these transactions to provide sufficient cash to sustain our operations through our fiscal year ending March 31, 2017, however they will not be adequate to enable the completion of our Phase 2b clinical trial of AV-101 in MDD. Accordingly, we intend to raise additional capital through sales of our securities, which may include both debt and equity securities. We may also seek research and development collaborations that could generate revenue, as well as government grant awards. Further, strategic collaborations, such as our February 2015 CRADA with the NIMH providing NIMH funding of our Phase 2a study of AV-101 in MDD, may provide resources to support a portion of our future cash needs and working capital requirements. Although we may seek additional collaborations that could generate revenue, as well as new government grant awards, no assurance can be provided that any such collaborations or awards will occur in the future. Our future working capital requirements will depend on many factors, including, without limitation, the scope and nature of opportunities related to our success and the success of certain other companies in clinical trials, including our development of AV-101 as a treatment for MDD and other CNS conditions, and our stem cell technology platform, the availability of, and our ability to obtain, government grant awards and our ability to enter into collaborations on terms acceptable to us. To further advance the clinical development of AV-101 and our stem cell technology platform, as well as support our operating activities, we plan to continue to carefully manage our routine operating costs, including our employee headcount and related expenses, as well as costs relating to regulatory consulting, contract research and development, investor relations and corporate development, legal, accounting, public company compliance and other professional services and working capital costs.

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

Cash and Cash Equivalents

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

	Three Months Ended June 30,

	2016	2015
Net cash used in operating activities	$(1,671)	$(823)
Net cash used in investing activities	(2)	-
Net cash provided by financing activities	9,744	874
Net increase in cash and cash equivalents	8,071	51
Cash and cash equivalents at beginning of period	429	70
Cash and cash equivalents at end of period	$8,500	$121

Off-Balance Sheet Arrangements

We have no off-balance sheet arrangements.

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Item 4. CONTROLS AND PROCEDURES

Disclosure Controls and Procedures

Our management, with the participation of our Chief Executive Officer and Chief Financial Officer, evaluated the effectiveness of our disclosure controls and procedures (as defined in Rule 13a-15(e) of the Exchange Act) as of the end of the period covered by this Report. Based on that evaluation, our Chief Executive Officer and our Chief Financial Officer concluded that our disclosure controls and procedures as of the end of the period covered by this Report were effective.

Internal Control over Financial Reporting

There was no change in our internal control over financial reporting (as defined in Rule 13a-15(f) of the Exchange Act) that occurred during the fiscal quarter to which this Report relates that has materially affected, or is reasonably likely to materially affect, our internal control over financial reporting.

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PART II: OTHER INFORMATION

Item 1. Legal Proceedings

None.

Item 1A. Risk Factors

Investing in our securities involves a high degree of risk. You should consider carefully the risks and uncertainties described below, together with all of the other information in this Quarterly Report on Form 10-Q and in our Annual Report on Form 10-K filed with the Securities and Exchange Commission for the fiscal year ended March 31, 2016 before investing in our securities. The risks described below are not the only risks facing our Company. Additional risks and uncertainties not currently known to us or that we currently deem to be immaterial may also materially adversely affect our business, financial condition and/or operating results. If any of the following risks are realized, our business, financial condition and results of operations could be materially and adversely affected.

Risks Related to Product Development, Regulatory Approval and Commercialization

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

We currently have no drug products for sale and may never be able to develop and commercialize marketable drug products. Our business depends heavily on the successful development, regulatory approval and commercialization of AV-101 for depression, including for MDD, and various other diseases and disorders involving the CNS, as well as, but to a more limited extent, our ability to produce, develop and commercialize NCEs from our drug rescue programs. AV-101 will require substantial additional Phase 2 and Phase 3 clinical development, testing and regulatory approval before we are permitted to commence its commercialization and is unlikely to achieve regulatory approval until at least 2021, if at all. Each drug rescue NCE will require substantial non-clinical development, all phases of clinical development, and regulatory approval before we are permitted to commence its commercialization. The non-clinical studies and clinical trials of our product candidates are, and the manufacturing and marketing of our product candidates will be, subject to extensive and rigorous review and regulation by numerous government authorities in the United States and in other countries where we intend to test and, if approved, market any product candidate. Before obtaining regulatory approvals for the commercial sale of any product candidate, we must demonstrate through non-clinical studies and clinical trials that the product candidate is safe and effective for use in each target indication. Drug development is a long, expensive and uncertain process, and delay or failure can occur at any stage of any of our non-clinical studies or clinical trials. This process can take many years and may also include post-marketing studies and surveillance, which will require the expenditure of substantial resources beyond the proceeds we have raised to date. Of the large number of drugs in development in the United States, only a small percentage will successfully complete the FDA regulatory approval process and will be commercialized. Accordingly, even if we are able to obtain the requisite financing to continue to fund our non-clinical studies and clinical trials, we cannot assure you that AV-101, any drug rescue NCE, or any other product candidate will be successfully developed or commercialized.

We are not permitted to market our product candidates in the United States until we receive approval of a New Drug Application (NDA) from the FDA, or in any foreign countries until we receive the requisite approval from such countries. In late 2015, in collaboration with the NIMH under our CRADA, we began a Phase 2a clinical trial involving AV-101, to study its safety, tolerability and efficacy in patients with MDD. If our Phase 2a clinical trial of AV-101 is successful, we expect the FDA to require us to complete at least one pivotal Phase 2B clinical trial and at least one pivotal Phase 3 clinical trial in order to submit an NDA for AV-101 as an adjunctive treatment for MDD. However, the FDA may require that we conduct more than one Phase 2B clinical study and more than one Phase 3

pivotal trial of AV-101 before we can submit an NDA. Also, we anticipate that the FDA will require that we conduct additional toxicity studies and additional non-clinical studies before submitting an NDA for AV-101.

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

- if our NDA, if and when submitted, is reviewed by an advisory committee, the FDA may have difficulties scheduling an advisory committee meeting in a timely manner or the advisory committee may recommend against approval of our application or may recommend that the FDA require, as a condition of approval, additional non-clinical studies or clinical trials, limitations on approved labeling or distribution and use restrictions;

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

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

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

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

We intend to seek a Fast Track designation from the FDA for AV-101 for treatment of MDD. Even if the FDA approves Fast Track designation for AV-101 for treatment of MDD, it may not actually lead to a faster development or regulatory review or approval process.

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

We intend to seek FDA Fast Track designation for AV-101 for adjunctive treatment of MDD, and we may do so for other product candidates as well. The FDA has broad discretion whether or not to grant this designation, and even if we believe AV-101 and other product candidates are eligible for this designation, we cannot be sure that the review or approval will compare to conventional FDA procedures. Even if granted, the FDA may withdraw Fast Track designation if it believes that the designation is no longer supported by data from our clinical development programs.

The number of patients suffering from MDD has not been established with precision. If the actual number of patients with MDD is smaller than we anticipate, we or our collaborators may encounter difficulties in enrolling patients in AV-101 clinical trials, including our NIH-funded Phase 2a clinical study of AV-101 in treatment-resistant MDD, thereby delaying or preventing clinical development. Further, if AV-101 is approved for adjunctive treatment of MDD, and the market for this indication is smaller than we anticipate, our ability to achieve profitability could be limited.

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

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

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

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

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Positive results from early preclinical studies and clinical trials of AV-101 or other product candidates are not necessarily predictive of the results of later preclinical studies and clinical trials of such product candidates. If we cannot replicate the positive results from our earlier preclinical studies and clinical trials of AV-101 or other product candidates in our later preclinical studies and clinical trials, we may be unable to successfully develop, obtain regulatory approval for and commercialize our product candidates.

Positive results from preclinical studies of our product candidates, and any positive results we may obtain from early clinical trials of our product candidates, may not necessarily be predictive of the results from required later preclinical studies and clinical trials. Similarly, even if we are able to complete our planned preclinical studies or clinical trials of our product candidates according to our current development timeline, the positive results from our preclinical studies and clinical trials of our product candidates may not be replicated in subsequent preclinical studies or clinical trial results. Many companies in the pharmaceutical and biotechnology industries have suffered significant setbacks in late-stage clinical trials after achieving positive results in early-stage development, and we cannot be certain that we will not face similar setbacks. These setbacks have been caused by, among other things, preclinical findings made while clinical trials were underway or safety or efficacy observations made in preclinical studies and clinical trials, including previously unreported adverse events. Moreover, preclinical and clinical data are often susceptible to varying interpretations and analyses, and many companies that believed their product candidates performed satisfactorily in preclinical studies and clinical trials nonetheless failed to obtain FDA approval. We have not yet completed a Phase 2a clinical trial for AV-101, and if we fail to produce positive results in our NIH-sponsored Phase 2a clinical trial of AV-101 in MDD, the development timeline and regulatory approval and commercialization prospects for AV-101 and, correspondingly, our business and financial prospects, could be materially adversely affected.

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

Under our CRADA, we and the NIH have commenced an NIH-funded Phase 2a clinical trial of AV-101 as a treatment for MDD. We will need to complete at least two additional large clinical trials prior to the submission of an NDA for AV-101 as a treatment for MDD. Successful completion of our clinical trials is a prerequisite to submitting an NDA to the FDA and, consequently, the ultimate approval and commercial marketing of AV-101 for MDD and any other product candidates we may develop. We do not know whether the NIH-funded Phase 2a study of AV-101 or any of our future-planned clinical trials will be completed on schedule, if at all, as the commencement and completion of clinical trials can be delayed or prevented for a number of reasons, including, among others:

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

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

negative results from our ongoing pre-clinical studies;

delays in reaching or failing to reach agreement on acceptable terms with prospective 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;

inadequate quantity or quality of a product candidate or other materials necessary to conduct clinical trials, for example delays in the manufacturing of sufficient supply of finished drug product;

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

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

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

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

delays in validating any endpoints utilized in a clinical trial;

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

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

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

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

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

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

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

changes in government regulations or administrative actions;

problems with clinical supply materials; and

lack of adequate funding to continue clinical trials.

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

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

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

We do not have the ability to independently conduct clinical trials. We rely on medical institutions, clinical investigators, contract laboratories and other third parties, such as CROs, to conduct clinical trials on our product candidates. We enter into agreements with third-party CROs to provide monitors for and to manage data for our clinical trials. We rely heavily on these parties for execution of clinical trials for our product candidates and control only certain aspects of their activities. As a result, we have less direct control over the conduct, timing and completion of these clinical trials and the management of data developed through clinical trials than would be the case if we were relying entirely upon our own staff. Communicating with outside parties can also be challenging, potentially leading

to mistakes as well as difficulties in coordinating activities. Outside parties may:

have staffing difficulties;

fail to comply with contractual obligations;

experience regulatory compliance issues;

undergo changes in priorities or become financially distressed; or

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

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

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

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

We rely completely on third-party suppliers to manufacture our clinical drug supplies for our product candidates, and we intend to rely on third parties to produce non-clinical, clinical and commercial supplies of any future product candidate.

We do not currently have, nor do we plan to acquire, the infrastructure or capability to internally manufacture our clinical drug supply of AV-101 or any other product candidates for use in the conduct of our nonclinical studies and clinical trials, and we lack the internal resources and the capability to manufacture any product candidates on a clinical or commercial scale. The facilities used by our contract manufacturers to manufacture the active pharmaceutical ingredient and final drug product must complete a pre-approval inspection by the FDA and other comparable foreign regulatory agencies to assess compliance with applicable requirements, including cGMPs, after we submit our NDA or relevant foreign regulatory submission to the applicable regulatory agency.

We do not control the manufacturing process of, and are completely dependent on, our contract manufacturers to comply with cGMPs for manufacture of both active drug substances and finished drug products. If our contract manufacturers cannot successfully manufacture material that conforms to our specifications and the strict regulatory requirements of the FDA or applicable foreign regulatory agencies, they will not be able to secure and/or maintain regulatory approval for their manufacturing facilities. In addition, we have no direct control over our contract manufacturers' ability to maintain adequate quality control, quality assurance and qualified personnel. Furthermore, all of our contract manufacturers are engaged with other companies to supply and/or manufacture materials or products for such companies, which exposes our third-party contract manufacturers to regulatory risks for the production of such materials and products. As a result, failure to satisfy the regulatory requirements for the production of those materials and products may affect the regulatory clearance of our contract manufacturers' facilities generally. If the FDA or an applicable foreign regulatory agency determines now or in the future that these facilities for the manufacture of our product candidates are noncompliant, we may need to find alternative manufacturing facilities, which would adversely impact our ability to develop, obtain regulatory approval for or market our product candidates.

Our reliance on contract manufacturers also exposes us to the possibility that they, or third parties with access to their facilities, will have access to and may appropriate our trade secrets or other proprietary information.

We do not yet have long-term supply agreements in place with our contract manufacturers and each batch of our product candidates are individually contracted under a quality and supply agreement. If we engage new contract manufacturers, such contractors must complete an inspection by the FDA and other applicable foreign regulatory agencies. We plan to continue to rely upon contract manufacturers and, potentially, collaboration partners, to manufacture commercial quantities of AV-101 and other product candidates, if approved. Our current scale of manufacturing for AV-101 is adequate to support our currently planned needs for additional pre-clinical studies and clinical trial supplies.

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Even if we receive marketing approval for our product candidates in the United States, we may never receive regulatory approval to market our product candidates outside of the United States.

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

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

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

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

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

- the efficacy and safety of our product candidates as demonstrated in clinical trials, and, if required by any applicable regulatory authority in connection with the approval for the applicable indications, to provide patients with incremental health benefits, as compared with other available therapies;
- limitations or warnings contained in the labeling approved for our product candidates by the FDA or other applicable regulatory authorities;
- the clinical indications for which our product candidates are approved;
- availability of alternative treatments already approved or expected to be commercially launched in the near future;

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

the willingness of the target patient population to try new therapies and of physicians to prescribe these therapies;

the strength of marketing and distribution support and timing of market introduction of competitive products;

publicity concerning our products or competing products and treatments;

pricing and cost effectiveness;

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the effectiveness of our sales and marketing strategies;

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

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

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

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

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

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

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

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

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

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

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

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

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

our reputation may suffer.

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

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

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

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

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issue warning letters or untitled letters;

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

suspend or withdraw marketing approval;

suspend any ongoing clinical trials;

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

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

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

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

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

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

In the field of new generation antidepressants focused on modulation of the NMDA receptor at the glycine binding co-agonist site, we believe our principal competitor is Allergan, which recently acquired from and is now developing both the intravenously-administered peptide, rapastinel (formerly GLYX-13), and NRX-1074, which may be or may become orally-available, for treatment-resistant MDD.

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

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

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

We face significant competition in seeking appropriate collaborators. Whether we reach a definitive agreement for collaboration will depend, among other things, upon our assessment of the collaborator's resources and expertise, the terms and conditions of the proposed collaboration and the proposed collaborator's evaluation of a number of factors. Those factors may include the design or results of clinical trials, the likelihood of approval by the FDA or similar regulatory authorities outside the United States, the potential market for the subject product candidate, the costs and complexities of manufacturing and delivering such product candidate to patients, the potential of competing products, the existence of uncertainty with respect to our ownership of technology, which can exist if there is a challenge to such ownership without regard to the merits of the challenge and industry and market conditions generally. The collaborator may also consider alternative product candidates or technologies for similar indications that may be available to collaborate on and whether such collaboration could be more attractive than the one with us for our product candidate. The terms of any collaboration or other arrangements that we may establish may not be favorable to us.

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

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

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

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

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

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

If any of these events occur, we may be forced to abandon our development efforts for a program or programs, which would have a material adverse effect on our business and could potentially cause us to cease operations. Research

programs to identify new product candidates require substantial technical, financial and human resources. We may focus our efforts and resources on potential programs or product candidates that ultimately prove to be unsuccessful.

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

Although we do not currently have any products on the market, once we begin commercializing our products, we may be subject to additional healthcare statutory and regulatory requirements and enforcement by the federal government and the states and foreign governments in which we conduct our business. Healthcare providers, physicians and others will play a primary role in the recommendation and prescription of our product candidates, if approved. Our future arrangements with third-party payors will expose us to broadly applicable fraud and abuse and other healthcare laws and regulations that may constrain the business or financial arrangements and relationships through which we market, sell and distribute our product candidates, if we obtain marketing approval. Restrictions under applicable federal and state healthcare laws and regulations include the following:

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

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

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

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

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

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

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

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

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

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

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

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

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

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

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

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

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

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

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

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

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

import or export licensing requirements;

longer accounts receivable collection times;

longer lead times for shipping;

language barriers for technical training;

reduced protection of intellectual property rights in some foreign countries;

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

foreign currency exchange rate fluctuations; and

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

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

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

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

produce product candidates;

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

maintain, leverage and expand our intellectual property portfolio;

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

gain market acceptance for our products; and

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

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

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

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

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

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

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

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

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

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

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

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

- our ability to identify potential drug rescue candidates in the public domain, obtain sufficient quantities of them, and assess them using our bioassay systems;
- if we seek to rescue drug rescue candidates that are not available to us in the public domain, the extent to which third parties may be willing to out-license or sell certain drug rescue candidates to us on commercially reasonable terms;
- our medicinal chemistry collaborator's ability to design and produce proprietary drug rescue NCEs based on the novel biology and structure-function insight we provide using CardioSafe 3D; and
- financial resources available to us to develop and commercialize lead drug rescue NCEs internally, or, if we out-license them to strategic partners, the resources such partners choose to dedicate to development and commercialization of any drug rescue NCEs they license from us.

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

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

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

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

The success of our drug rescue programs is highly dependent upon CardioSafe 3D being more accurate, efficient and clinically predictive than long-established surrogate safety models, including animal cells and live animals, and immortalized, primary and transformed cells, currently used by pharmaceutical companies and others. We cannot give

assurance that CardioSafe 3D will be more efficient or accurate at predicting the heart safety of new drug candidates than the testing models currently used. If CardioSafe 3D fails to provide a meaningful difference compared to existing or new models in predicting the behavior of human heart, respectively, their utility for drug rescue will be limited and our drug rescue business will be adversely affected.

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

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

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

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

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

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

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

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

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

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

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

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

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

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

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

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

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

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

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

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

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

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Risks Related to Our Financial Position

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

We have incurred significant net losses in each fiscal year since our inception in 1998, including net losses of $47.2 million, which includes $26.7 million of non-cash expense related to the extinguishment of essentially all of our outstanding promissory notes and certain other indebtedness, and $13.9 million during the fiscal years ended March 31, 2016 and 2015, respectively, and a net loss of $2.0 million in the quarter ended June 30, 2016. As of June 30, 2016, we had an accumulated deficit of approximately $133.7 million. We do not know whether or when we will become profitable. Substantially all of our operating losses have resulted from costs incurred in connection with our research and development programs and from general and administrative costs associated with our operations. We expect to incur increasing levels of operating losses over the next several years and for the foreseeable future. Our prior losses, combined with expected future losses, have had and will continue to have an adverse effect on our stockholders' deficit and working capital. We expect our research and development expenses to significantly increase in connection with non-clinical studies and clinical trials of our product candidates. In addition, if we obtain marketing approval for our product candidates, we may incur significant sales, marketing and outsourced-manufacturing expenses should we elect not to collaborate with one or more third parties for such services and capabilities. As a public company, we incur additional costs associated with operating as a public company. As a result, we expect to continue to incur significant and increasing operating losses for the foreseeable future. Because of the numerous risks and uncertainties associated with developing pharmaceutical products, we are unable to predict the extent of any future losses or when we will become profitable, if at all. Even if we do become profitable, we may not be able to sustain or increase our profitability on a quarterly or annual basis.

Our ability to become profitable depends upon our ability to generate revenues. To date, we have generated approximately $16.4 million in revenues, primarily from the receipt of research and development grants from the NIH. We have not yet commercialized any product or generated any revenues from product sales, and we do not know when, or if, we will generate any revenue from product sales. We do not expect to generate significant revenue unless and until we obtain marketing approval of, and begin to experience sales of, AV-101, or we enter into one or more development and commercialization agreements with respect to AV-101 or one or more other product candidates. Our ability to generate revenue depends on a number of factors, including, but not limited to, our ability to:

- initiate and successfully complete preclinical and clinical trials that meet their prescribed endpoints;
- initiate and successfully complete all safety studies required to obtain U.S. and foreign marketing approval for our product candidates;
- commercialize our product candidates, if approved, by developing a sales force or entering into collaborations with third parties; and
- achieve market acceptance of our product candidates in the medical community and with third-party payors.

Unless we enter into a development and commercialization collaboration or partnership agreement, we expect to incur significant sales and marketing costs as we prepare to commercialize AV-101 or other product candidates. Even if we initiate and successfully complete pivotal clinical trials of AV-101 or other product candidates, and AV-101 or other product candidates are approved for commercial sale, and despite expending these costs, AV-101 or other product candidates may not be commercially successful. We may not achieve profitability soon after generating product sales, if ever. If we are unable to generate product revenue, we will not become profitable and may be unable to continue

operations without continued funding.

Despite consummation of the May 2016 Public Offering (defined below), we require additional financing to execute our business plan and continue to operate as a going concern.

Our audited consolidated financial statements for the year ended March 31, 2016 and our condensed consolidated financial statements for the quarter ended June 30, 2016 have been prepared assuming we will continue to operate 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 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 our securities or from alternative sources, we may be required to reduce, defer, or discontinue certain or all of our research and development activities or we may not be able to continue as a going concern.

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Since our inception, most of our resources have been dedicated to research and development of AV-101 and the drug rescue capabilities of our stem cell technology platform. In particular, we have expended substantial resources advancing AV-101 through preclinical development and Phase 1 clinical safety studies, and developing CardioSafe 3D for drug rescue applications, and we will continue to expend substantial resources for the foreseeable future developing and commercializing AV-101, and, potentially, developing drug rescue NCEs and RM therapies. 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.

At March 31, 2016, our existing cash and cash equivalents were not sufficient to fund our current operations for the next 12 months. However, as described in Note 8, Capital Stock, to the accompanying Condensed Consolidated Financial Statements for the quarter ended June 30, 2016 included elsewhere in this Quarterly Report, on May 16, 2016, we consummated an underwritten public offering, pursuant to which we issued an aggregate of 2,570,040 registered shares of our common stock at a public sales price of $4.24 per share and five-year warrants, exercisable at $5.30 per share, to purchase an aggregate of 2,705,883 shares of our common stock at a public sales price of $0.01 per warrant share, including shares and warrants issued pursuant to the exercise of the underwriters' over-allotment option, resulting in gross proceeds of approximately $10.9 million (May 2016 Public Offering). Our net proceeds from the May 2016 Public Offering were approximately $9.5 million after deducting underwriters' commissions and other expenses of the offering. Additionally, in February 2015, we entered into the CRADA with the NIH, under which the NIH is fully funding and conducting the initial Phase 2a clinical efficacy and safety of AV-101 in MDD. However, we have no current source of revenue to sustain our present activities, and we do not expect to generate revenue until, and unless, we (i) out-license or sell AV-101, a drug rescue NCE, and/or another drug candidate unrelated to AV-101 to third-parties, (ii) enter into license arrangements involving our stem cell technology, or (iii) obtain approval from the FDA or other regulatory authorities and successfully commercialize, on our own or through a future collaboration, one or more of our compounds.

As the outcome of our AV-101 and NCE drug rescue activities and future anticipated clinical trials is highly uncertain, we cannot reasonably estimate the actual amounts necessary to successfully complete the development and commercialization of our product candidates, on our own or in collaboration with others. In addition, other unanticipated costs may arise. As a result of these and other factors, we will need to seek additional capital in the near term to meet our future operating requirements, including capital necessary to obtain regulatory approval for, and to commercialize, our product candidates, and may seek additional capital in the event there exists favorable market conditions or strategic considerations even if we believe we have sufficient funds for our current or future operating plans. We are considering a range of potential sources of funding, including public or private equity or debt financings, government or other third-party funding, marketing and distribution arrangements and other collaborations, strategic alliances and licensing arrangements or a combination of these approaches, and we may complete additional financing arrangements in 2016. Raising funds in the current economic environment may present additional challenges. Even if we believe we have sufficient funds for our current or future operating plans, we may seek additional capital if market conditions are favorable or if we have specific strategic considerations.

Our future capital requirements depend on many factors, including:

- the number and characteristics of the product candidates we pursue, including AV-101 and drug rescue NCEs;
- the scope, progress, results and costs of researching and developing our product candidates, and conducting preclinical and clinical studies;
- the timing of, and the costs involved in, obtaining regulatory approvals for our product candidates;

- the cost of commercialization activities if any of our product candidates are approved for sale, including marketing, sales and distribution costs;
- the cost of manufacturing our product candidates and any products we successfully commercialize;
- our ability to establish and maintain strategic partnerships, licensing or other arrangements and the financial terms of such agreements;
- market acceptance of our products;
- the effect of competing technological and market developments;
- our ability to obtain government funding for our programs;
- the costs involved in obtaining and enforcing patents to preserve our intellectual property;

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

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

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

Any additional fundraising efforts will divert our management from their day-to-day activities, which may adversely affect our ability to develop and commercialize our product candidates. In addition, we cannot guarantee that future financing wil