

CATALYST PHARMACEUTICALS, INC.

Form 10-Q

August 10, 2015

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UNITED STATES

SECURITIES AND EXCHANGE COMMISSION

Washington, D.C. 20549

FORM 10-Q

[Mark One]

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

For the Quarterly Period Ended June 30, 2015

OR

TRANSITION REPORT UNDER SECTION 13 OR 15(d) OF THE SECURITIES EXCHANGE ACT OF 1934

Commission File No. 001-33057

CATALYST PHARMACEUTICALS, INC.

(Exact name of registrant as specified in its charter)

Delaware (State or other jurisdiction of	76-0837053 (IRS Employer
incorporation or organization)	Identification No.)
355 Alhambra Circle	
Suite 1500	
Coral Gables, Florida	33134
(Address of principal executive offices)	(Zip Code)
Registrant's telephone number, including area code: <u>(305) 529-2522</u>	

Indicate by checkmark 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 report(s), and (2) has been subject to such filing requirements for the past 90 days. Yes No

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

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

Large Accelerated Filer Accelerated Filer

Non-Accelerated Filer (Do not check if a smaller reporting company) Smaller reporting company

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

Indicate the number of shares outstanding of each of the issuer's classes of common stock, as of the latest practicable date 82,475,936 shares of common stock, \$0.001 par value per share, were outstanding as of August 7, 2015.

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CATALYST PHARMACEUTICALS, INC.

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	June 30, 2015	December 31, 2014
	(unaudited)	
ASSETS		
Current Assets:		
Cash and cash equivalents	\$ 37,221,453	\$ 9,096,778
Certificates of deposit	3,716,399	3,715,383
Short-term investments	26,465,464	26,462,962
Prepaid expenses and other current assets	861,268	4,552,698
Total current assets	68,264,584	43,827,821
Property and equipment, net	63,294	71,377
Deposits	8,888	8,888
Total assets	\$ 68,336,766	\$ 43,908,086
LIABILITIES AND STOCKHOLDERS EQUITY		
Current Liabilities:		
Accounts payable	\$ 1,316,292	\$ 1,814,210
Accrued expenses and other liabilities	1,236,955	4,040,816
Total current liabilities	2,553,247	5,855,026
Accrued expenses and other liabilities, non-current	12,452	15,839
Warrants liability, at fair value	2,979,038	2,794,891
Total liabilities	5,544,737	8,665,756
Commitments and contingencies		
Stockholders' equity:		
Preferred stock, \$0.001 par value, 5,000,000 shares authorized: none issued and outstanding		
Common stock, \$0.001 par value, 150,000,000 and 100,000,000 shares authorized; 82,096,806 shares and 69,119,092 shares issued and outstanding at June 30, 2015 and December 31, 2014, respectively	82,097	69,119
Additional paid-in capital	142,521,354	105,015,871
Accumulated deficit	(79,811,422)	(69,842,660)
Total stockholders' equity	62,792,029	35,242,330
Total liabilities and stockholders' equity	\$ 68,336,766	\$ 43,908,086

The accompanying notes are an integral part of these financial statements.

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CATALYST PHARMACEUTICALS, INC.
STATEMENTS OF OPERATIONS (unaudited)

	For the Three Months Ended June 30,		For the Six Months Ended June 30,	
	2015	2014	2015	2014
Operating costs and expenses:				
Research and development	\$ 2,577,508	\$ 2,098,958	\$ 4,927,060	\$ 4,847,641
General and administrative	2,319,822	891,215	4,262,185	1,650,897
Total operating costs and expenses	4,897,330	2,990,173	9,189,245	6,498,538
Loss from operations	(4,897,330)	(2,990,173)	(9,189,245)	(6,498,538)
Other income, net	4,871	15,744	66,805	48,504
Change in fair value of warrants liability	333,956	(223,591)	(846,322)	(559,105)
Loss before income taxes	(4,558,503)	(3,198,020)	(9,968,762)	(7,009,139)
Provision for income taxes				
Net loss	\$ (4,558,503)	\$ (3,198,020)	\$ (9,968,762)	\$ (7,009,139)
Net loss per share basic and diluted	\$ (0.06)	\$ (0.05)	\$ (0.13)	\$ (0.12)
Weighted average shares outstanding basic and diluted	82,037,560	66,167,556	79,054,960	60,186,297

The accompanying notes are an integral part of these financial statements.

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CATALYST PHARMACEUTICALS, INC.

STATEMENT OF STOCKHOLDERS EQUITY (unaudited)

For the six months ended June 30, 2015

	Preferred Stock	Common Stock	Additional Paid-in Capital	Accumulated Deficit	Total
Balance at December 31, 2014	\$	\$ 69,119	\$ 105,015,871	\$ (69,842,660)	\$ 35,242,330
Issuance of common stock, net		11,500	34,862,369		34,873,869
Issuance of stock options for services			640,922		640,922
Amortization of restricted stock for services			37,424		37,424
Exercise of warrants for common stock		804	1,965,442		1,966,246
Exercise of stock options for common stock		674	(674)		
Net loss				(9,968,762)	(9,968,762)
Balance at June 30, 2015	\$	\$ 82,097	\$ 142,521,354	\$ (79,811,422)	\$ 62,792,029

The accompanying notes are an integral part of these financial statements.

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CATALYST PHARMACEUTICALS, INC.
STATEMENTS OF CASH FLOWS (unaudited)

	For the Six Months Ended, June 30,	
	2015	2014
Operating Activities:		
Net loss	\$ (9,968,762)	\$ (7,009,139)
Adjustments to reconcile net loss to net cash used in operating activities:		
Depreciation	17,123	11,368
Stock-based compensation	678,346	45,700
Change in fair value of warrants liability	846,322	559,105
(Increase) decrease in:		
Prepaid expenses and other current assets and deposits	191,430	767,348
Increase (decrease) in:		
Accounts payable	(497,918)	387,387
Accrued expenses and other liabilities	692,753	(170,673)
Net cash used in operating activities	(8,040,706)	(5,408,904)
Investing Activities:		
Capital expenditures	(9,040)	(30,308)
Redemption (purchase) of short-term investments	(2,502)	(9,014,527)
Redemption (purchase) of certificates of deposit	(1,016)	297,691
Net cash used in investing activities	(12,558)	(8,747,144)
Financing Activities:		
Proceeds from issuance of common stock, net	34,873,869	26,725,130
Proceeds from exercise of warrants	1,304,070	16,504
Net cash provided by financing activities	36,177,939	26,741,634
Net increase (decrease) in cash	28,124,675	12,585,586
Cash and cash equivalents at beginning of period	9,096,778	2,215,958
Cash and cash equivalents at end of period	\$ 37,221,453	\$ 14,801,544
Supplemental disclosures of non-cash investing and financing activity		
Exercise of liability classified warrants for common stock	\$ 662,175	\$ 18,537

The accompanying notes are an integral part of these financial statements.

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CATALYST PHARMACEUTICALS, INC.

NOTES TO UNAUDITED FINANCIAL STATEMENTS

1. Organization and Description of Business.

Catalyst Pharmaceuticals, Inc. (the Company) is a development-stage biopharmaceutical company focused on the development and commercialization of prescription drugs targeting rare (orphan) neuromuscular and neurological diseases and disorders, including Lambert-Eaton Myasthenic Syndrome (LEMS) and infantile spasms.

Since inception, the Company has devoted substantially all of its efforts to business planning, research and development, recruiting management and technical staff, acquiring operating assets and raising capital. The Company's primary focus is on the development and commercialization of its drug candidates. The Company has incurred operating losses in each period from inception through June 30, 2015. The Company has been able to fund its cash needs to date through several public and private offerings of its common stock and warrants, through government grants, and through an investment by a strategic purchaser. See Note 9.

Capital Resources

On January 31, 2014, the Company filed a Shelf Registration Statement on Form S-3 (the 2014 Shelf Registration Statement) with the U.S. Securities and Exchange Commission (SEC) to sell up to \$100 million of common stock. This registration statement (file No. 333-193699) was declared effective by the SEC on March 19, 2014. The Company has conducted two registered direct offerings under the 2014 Shelf Registration Statement. See Note 9.

While there can be no assurance, based on currently available information, the Company estimates that it currently has sufficient resources to support its operations through the end of 2016. The Company will require additional funding to support the Company's operations in periods after 2016.

The Company may raise required funds through public or private equity offerings, debt financings, corporate collaborations, governmental research grants or other means. The Company may also seek to raise new capital to fund additional product development efforts, even if it has sufficient funds for its planned operations. Any sale by the Company of additional equity or convertible debt securities could result in dilution to the Company's current stockholders. There can be no assurance that any such required additional funding will be available to the Company at all or available on terms acceptable to the Company. Further, to the extent that the Company raises additional funds through collaborative arrangements, it may be necessary to relinquish some rights to the Company's drug candidates or grant sublicenses on terms that are not favorable to the Company. If the Company is not able to secure additional funding when needed, the Company may have to delay, reduce the scope of, or eliminate one or more research and development programs, which could have an adverse effect on the Company's business.

2. Basis of Presentation and Significant Accounting Policies.

- a. INTERIM FINANCIAL STATEMENTS.** The accompanying unaudited interim financial statements have been prepared in accordance with U.S. generally accepted accounting principles (U.S. GAAP), and pursuant to the rules and regulations of the SEC for reporting of interim financial information. Pursuant to such rules

and regulations, certain information and footnote disclosures normally included in financial statements prepared in accordance with U.S. GAAP have been or omitted. The balance sheet as of December 31, 2014 included in this Form 10-Q was derived from the audited financial statements and does not include all disclosures required by U.S. GAAP.

In the opinion of management, the accompanying unaudited interim financial statements of the Company contain all adjustments (consisting of only normal recurring adjustments) necessary to present fairly the financial position of the Company as of the dates and for the periods presented. Accordingly, these statements should be read in conjunction with the financial statements and notes thereto for the year ended December 31, 2014 included in the 2014 Annual Report on Form 10-K filed by the Company with the SEC. The results of operations for the three and six months ended June 30, 2015 are not necessarily indicative of the results to be expected for any future period or for the full 2015 fiscal year.

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2. Basis of Presentation and Significant Accounting Policies (continued).

- b. USE OF ESTIMATES.** The preparation of financial statements in conformity with U.S. GAAP requires management to make estimates and assumptions that affect the amounts reported in the financial statements and accompanying notes. Actual results could differ from those estimates.
- c. CASH AND CASH EQUIVALENTS.** The Company considers all highly liquid instruments purchased with an original maturity of three months or less to be cash equivalents. Cash equivalents consist mainly of money market funds. The Company has substantially all of its cash and cash equivalents deposited with one financial institution.
- d. CERTIFICATES OF DEPOSIT.** The certificates of deposit are issued by a banking institution and are recorded at cost plus accrued interest. The original maturity is greater than three months but does not exceed one year. Interest income is recorded in the statement of operations as it is earned. Carrying value at June 30, 2015 and December 31, 2014 approximates fair value.
- e. SHORT-TERM INVESTMENTS.** The Company invests in short-term investments in high credit-quality funds in order to obtain higher yields on its cash available for investments. As of June 30, 2015 and December 31, 2014 short-term investments consisted of short-term bond funds. Such investments are not insured by the Federal Deposit Insurance Corporation. Short-term investments at June 30, 2015 and December 31, 2014 were considered trading securities. Trading securities are recorded at fair value based on the closing market price of the security. For trading securities, the Company recognizes realized gains and losses and unrealized gains and losses to earnings. Unrealized and realized gains (losses) for the three and six months ended June 30, 2015 and 2014 were nominal, and are included in other income, net in the accompanying statements of operations.
- f. PREPAID EXPENSES AND OTHER CURRENT ASSETS.** Prepaid expenses and other current assets consist primarily of insurance recoverable, prepaid research fees, prepaid insurance and prepaid subscription fees. Insurance recoverable at December 31, 2014 related to the securities class action lawsuit proposed settlement that was paid by the Company's insurance carrier. Prepaid research fees consists of advances for the Company's product development activities, including drug manufacturing, contracts for preclinical studies, clinical trials and studies, regulatory affairs and consulting. Such advances are recorded as expense as the related goods are received or the related services are performed.
- g. FAIR VALUE OF FINANCIAL INSTRUMENTS.** The Company's financial instruments consist of cash and cash equivalents, certificates of deposit, short-term investments, accounts payables, accrued expenses and other liabilities, and warrants liability. At June 30, 2015 and December 31, 2014, the fair value of these instruments approximated their carrying value.

- h. FAIR VALUE MEASUREMENTS.** Current Financial Accounting Standards Board (FASB) fair value guidance emphasizes that fair value is a market-based measurement, not an entity-specific measurement. Therefore, a fair value measurement should be determined based on the assumptions that market participants would use in pricing the asset or liability. As a basis for considering market participant assumptions in fair value measurements, current FASB guidance establishes a fair value hierarchy that distinguishes between market participant assumptions based on market data obtained from sources independent of the reporting entity (observable inputs that are classified within Levels 1 and 2 of the hierarchy) and the reporting entity's own assumptions that market participants would use in pricing assets or liabilities (unobservable inputs classified within Level 3 of the hierarchy).

Table of Contents**2. Basis of Presentation and Significant Accounting Policies (continued).**

Level 1 inputs utilize quoted prices (unadjusted) in active markets for identical assets or liabilities that the Company has the ability to access at the measurement date. Level 2 inputs are inputs other than quoted prices included in Level 1 that are observable for the asset or liability, either directly or indirectly. Level 2 inputs may include quoted prices for similar assets and liabilities in active markets, as well as inputs that are observable for the asset or liability (other than quoted prices), such as interest rates, foreign exchange rates, and yield curves that are observable at commonly quoted intervals. Level 3 inputs are unobservable inputs for the asset or liability, which is typically based on an entity's own assumptions, as there is little, if any, related market activity. In instances where the determination of the fair value measurement is based on inputs from different levels of the fair value hierarchy, the level in the fair value hierarchy within which the entire fair value measurement falls is based on the lowest level input that is significant to the fair value measurement in its entirety. The Company's assessment of the significance of a particular input to the fair value measurement in its entirety requires judgment, and considers factors specific to the asset or liability.

Fair Value Measurements at Reporting Date Using

	Balances as of June 30, 2015	Quoted Prices in Active Markets for Identical Assets/Liabilities (Level 1)	Significant Other Observable Inputs (Level 2)	Significant Unobservable Inputs (Level 3)
Money market funds	\$ 36,109,973	\$ 36,109,973	\$	\$
Certificates of deposit	\$ 3,716,399	\$	\$ 3,716,399	\$
Short-term investments	\$ 26,465,464	\$ 26,465,464	\$	\$
Warrants liability	\$ 2,979,038	\$	\$	\$ 2,979,038

Fair Value Measurements at Reporting Date Using

	Balances as of December 31, 2014	Quoted Prices in Active Markets for Identical Assets/Liabilities (Level 1)	Significant Other Observable Inputs (Level 2)	Significant Unobservable Inputs (Level 3)
Money market funds	\$ 7,053,310	\$ 7,053,310	\$	\$
Certificates of deposit	\$ 3,715,383	\$	\$ 3,715,383	\$
Short-term investments	\$ 26,462,962	\$ 26,462,962	\$	\$
Warrants liability	\$ 2,794,891	\$	\$	\$ 2,794,891

- i. WARRANTS LIABILITY.** In October 2011, the Company issued 1,523,370 warrants (the 2011 warrants) to purchase shares of the Company's common stock in connection with a registered direct offering under the 2010 Shelf Registration Statement. The Company accounted for these warrants as a liability measured at fair value due to a provision included in the warrants agreement that provides the warrants holders with an option to require the Company (or its successor) to purchase their warrants for cash in an amount equal to their Black-Scholes Option Pricing Model (the Black-Scholes Model) value, in the event that certain fundamental transactions, as defined, occur. The fair value of the warrants liability is estimated using the Black-Scholes Model which requires inputs such as the expected term of the warrants, share price volatility and risk-free interest rate. These assumptions are reviewed on a quarterly basis and changes in the estimated fair value of the outstanding warrants are recognized each reporting period in the Change in fair value of warrants liability line in the statement of operations. As of June 30, 2015 and December 31, 2014, 1,003,043 and 1,242,174, respectively, of the 2011 warrants remained outstanding.

Table of Contents**2. Basis of Presentation and Significant Accounting Policies (continued).**

- j. STOCK-BASED COMPENSATION.** The Company recognizes expense in the statement of operations for the fair value of all stock-based payments to employees, directors, consultants and scientific advisors, including grants of stock options and other share-based awards. For stock options, the Company uses the Black-Scholes option valuation model, the single-option award approach, and the straight-line attribution method. Using this approach, compensation cost is amortized on a straight-line basis over the vesting period of each respective stock option, generally three years. The Company estimates forfeitures and adjusts this estimate periodically based on actual forfeitures.

As of June 30, 2015, there were outstanding stock options to purchase 3,370,000 shares of common stock, of which stock options to purchase 1,953,333 shares of common stock were exercisable as of June 30, 2015.

For the three and six month periods ended June 30, 2015 and 2014, the Company recorded stock-based compensation expense as follows:

	Three months ended June 30,		Six months ended June 30,	
	2015	2014	2015	2014
Research and development	\$ 73,203	\$ 12,006	\$ 140,144	\$ 23,879
General and administrative	290,692	10,564	538,202	21,821
Total stock-based compensation	\$ 363,895	\$ 22,570	\$ 678,346	\$ 45,700

- k. COMPREHENSIVE INCOME (LOSS).** U.S. GAAP require that all components of comprehensive income (loss) be reported in the financial statements in the period in which they are recognized. Comprehensive income (loss) is net income (loss), plus certain other items that are recorded directly into stockholders' equity. For all periods presented, the Company's net loss equals comprehensive loss, since the Company has no items which are considered other comprehensive income (loss).
- l. NET LOSS PER SHARE.** Basic loss per share is computed by dividing net loss for the period by the weighted average number of common shares outstanding during the period. The calculation of basic and diluted net loss per share is the same for all periods presented, as the effect of potential common stock equivalents is anti-dilutive due to the Company's net loss position for all periods presented. The potential shares, which are excluded from the determination of basic and diluted net loss per share as their effect is anti-dilutive, are as follows:

	June 30,	
	2015	2014
Options to purchase common stock	3,370,000	3,421,906
Warrants to purchase common stock	2,781,793	4,835,924
Unvested restricted stock	80,000	

Potential equivalent common stock excluded	6,231,793	8,257,830
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Potentially dilutive options to purchase common stock as of June 30, 2015 and 2014 have exercise prices per share ranging from \$0.47 to \$4.64 and \$0.47 to \$6.00, respectively. Potentially dilutive warrants to purchase common stock as of June 30, 2015 and 2014 have exercise prices ranging from \$1.04 to \$2.08 per share.

Table of Contents**2. Basis of Presentation and Significant Accounting Policies (continued).**

- m. RECENTLY ISSUED ACCOUNTING STANDARDS.** In August 2014, the FASB issued ASU No. 2014-15, Presentation of Financial Statements – Going Concern (Subtopic 205-40): Disclosure of Uncertainties about an Entity’s Ability to Continue as a Going Concern. The amendments in this ASU, require management to assess a company’s ability to continue as a going concern and to provide related disclosures in certain circumstances. The guidance will be effective for the annual period ending after December 15, 2016 and subsequent interim and annual periods thereafter. The Company is currently evaluating the impact of this accounting standard update on its financial statements.

3. Warrants Liability, at Fair Value.*2011 Warrants*

The Company allocated approximately \$1.3 million of proceeds from its October 2011 registered direct offering to the fair value of common stock purchase warrants issued in connection with the offering that are classified as a liability (the 2011 warrants). The 2011 warrants are classified as a liability because of provisions in such warrants that allow for the net cash settlement of such warrants in the event of certain fundamental transactions (as defined in the warrant agreement). The valuation of the 2011 warrants is determined using the Black-Scholes Model. This model uses inputs such as the underlying price of the shares issued when the warrant is exercised, volatility, risk free interest rate and expected life of the instrument. The Company has determined that the 2011 warrants liability should be classified within Level 3 of the fair value hierarchy by evaluating each input for the Black-Scholes Model against the fair value hierarchy criteria and using the lowest level of input as the basis for the fair value classification. There are six inputs: closing price of the Company’s common stock on the day of evaluation; the exercise price of the warrants; the remaining term of the warrants; the volatility of the Company’s common stock; annual rate of dividends; forfeiture rate; and the risk free rate of return. Of those inputs, the exercise price of the warrants and the remaining term are readily observable in the warrants agreement. The annual rate of dividends is based on the Company’s historical practice of not granting dividends. The closing price of the Company’s common stock would fall under Level 1 of the fair value hierarchy as it is a quoted price in an active market. The risk free rate of return is a Level 2 input, while the historical volatility is a Level 3 input in accordance with the fair value accounting guidance. Since the lowest level input is a Level 3, the Company determined the warrants liability is most appropriately classified within Level 3 of the fair value hierarchy. This liability is subject to fair value mark-to-market adjustment each reporting period.

The calculated value of the 2011 warrants liability was determined using the Black-Scholes Model with the following assumptions:

	June 30, 2015	December 31, 2014
Risk free interest rate	0.58%	0.81%
Expected term	1.84 years	2.34 years
Expected volatility	73%	112%
Expected dividend yield	0%	0%
Expected forfeiture rate	0%	0%

Table of Contents**3. Warrants Liability, at Fair Value (continued).**

The following table rolls forward the fair value of the Company's warrants liability activity for the three and six month periods ended June 30, 2015 and 2014:

	Three months ended June 30,		Six months ended June 30,	
	2015	2014	2015	2014
Fair value, beginning of period	\$ 3,564,299	\$ 2,136,539	\$ 2,794,891	\$ 1,819,562
Issuance of warrants				
Exercise of warrants	(251,305)		(662,175)	(18,537)
Change in fair value	(333,956)	223,591	846,322	559,105
Fair value, end of period	\$ 2,979,038	\$ 2,360,130	\$ 2,979,038	\$ 2,360,130

During the three and six month periods ended June 30, 2015, 86,957 and 239,131 of the 2011 warrants were exercised, with proceeds to the Company of \$113,044 and \$310,870, respectively. During the three month period ending June 30, 2014 none of the 2011 warrants were exercised. During the six month period ended June 30, 2014 12,696 of the 2011 warrants were exercised, with proceeds to the Company of \$16,504. The Company recognizes the change in the fair value of the warrants liability as a non-operating income or loss in the accompanying statements of operations.

4. Prepaid Expenses and Other Current Assets.

Prepaid expenses consist of the following:

	June 30, 2015	December 31, 2014
Insurance recoverable (see Notes 6 and 7)	\$	\$ 3,500,000
Prepaid research fees	411,842	571,428
Prepaid insurance	193,295	385,496
Prepaid subscription fees	65,269	30,495
Prepaid offering costs		20,029
Prepaid rent		10,870
Prepaid pre-commercialization expenses	153,607	
Other	37,255	34,380
Total prepaid expenses and other current assets	\$ 861,268	\$ 4,552,698

5. Property and Equipment.

Property and equipment, net consists of the following:

	June 30, 2015	December 31, 2014
Computer equipment	\$ 104,794	\$ 95,754
Furniture and equipment	88,816	88,816
	193,610	184,570
Less: Accumulated depreciation	(130,316)	(113,193)
Total property and equipment, net	\$ 63,294	\$ 71,377

Depreciation expense was \$8,975 and \$17,123 for the three and six month periods ended June 30, 2015, and \$6,083 and \$11,368, for the three and six month periods ended June 30, 2014, respectively. The Company has executed a noncancellable operating lease agreement for its corporate offices. During March 2015, the Company entered into the third amendment of the corporate office lease for an additional space within the same building under substantially the same terms in order to accommodate the Company's expanding operations.

Table of Contents**6. Accrued Expenses and Other Liabilities.**

Accrued expenses and other liabilities consist of the following:

	June 30, 2015	December 31, 2014
Accrued settlement liability (see Note 7)	\$	\$ 3,500,000
Accrued preclinical and clinical trial expenses	349,760	333,928
Accrued professional fees	92,146	43,973
Accrued compensation and benefits	568,886	31,956
Accrued license fees	142,500	115,000
Deferred rent	5,702	4,158
Other	77,961	11,801
Current accrued expenses and other liabilities	1,236,955	4,040,816
Deferred rent- non-current	12,452	15,839
Non-current accrued expenses and other liabilities	12,452	15,839
Total accrued expenses and other liabilities	\$ 1,249,407	\$ 4,056,655

The accrued settlement liability of \$3,500,000 as of December 31, 2014 is related to the securities class action lawsuit settlement, as disclosed with more particularity in Note 7. The settlement amount was covered by the Company's insurance carrier; therefore, there was a corresponding insurance recoverable recorded in Prepaid Expenses and Other Current Assets in the accompanying balance sheet as of December 31, 2014. See Notes 4 and 7. The settlement became final on April 16, 2015.

7. Commitments and Contingencies.

- a. **LICENSE AGREEMENT WITH NORTHWESTERN UNIVERSITY.** On August 27, 2009, the Company entered into a license agreement with Northwestern University (Northwestern), under which it acquired worldwide rights to commercialize new GABA aminotransferase inhibitors and derivatives of vigabatrin that have been discovered by Northwestern. Under the terms of the license agreement, Northwestern granted the Company an exclusive worldwide license to certain composition of matter patents related to the new class of inhibitors and a patent application relating to derivatives of vigabatrin. The Company has identified and designated the lead compound under this license as CPP-115.

Under the license agreement with Northwestern, the Company is responsible for continued research and development of any resulting product candidates. As of June 30, 2015, the Company has paid \$251,590 in connection with the license and has accrued license fees of \$142,500 in the accompanying June 30, 2015 balance sheet for expenses, maintenance fees and milestones. In addition, the Company is obligated to pay certain milestone payments in future years relating to clinical development activities with respect to CPP-115, and royalties on any products resulting from the license agreement. The next milestone payment of \$150,000 is due on August 27, 2015.

- b. LICENSE AGREEMENT WITH NEW YORK UNIVERSITY AND THE FEINSTEIN INSTITUTE FOR MEDICAL RESEARCH.** On December 13, 2011, the Company entered into a license agreement with New York University (NYU) and the Feinstein Institute for Medical Research (FIMR) under which it acquired worldwide rights to commercialize GABA aminotransferase inhibitors in the treatment for Tourette's Disorder. The Company is obligated to pay certain milestone payments in future years relating to clinical development activities and royalties on any products resulting from the license agreement.

Table of Contents**7. Commitments and Contingencies (continued).**

- c. **LICENSE AGREEMENT WITH BIOMARIN.** On October 26, 2012, the Company entered into a strategic collaboration with BioMarin Pharmaceutical, Inc. (BioMarin) for Firdapse[®]. The key components of the collaboration include: (i) the Company licensed the exclusive North American rights to Firdapse[®] pursuant to a License Agreement, dated as of October 26, 2012 (the License Agreement) between the Company and BioMarin, and (ii) BioMarin made a \$5,000,000 investment in the Company pursuant to the terms of a Convertible Promissory Note and Note Purchase Agreement, dated as of October 26, 2012 (the Investment Agreement).

As part of the License Agreement, the Company agreed: (i) to pay BioMarin royalties for seven years from the first commercial sale of Firdapse[®] equal to 7% of net sales (as defined in the license agreement) in North America for any calendar year for sales up to \$100 million, and 10% of net sales in North America in any calendar year in excess of \$100 million; (ii) to pay to the third-party licensor of the rights sublicensed to us royalty payments for seven years from the first commercial sale of Firdapse[®] equal to 7% of net sales (as defined in the license agreement between BioMarin and the third-party licensor) in any calendar year; and (iii) to pay certain milestone payments that BioMarin is obligated to pay (approximately \$2.6 million of which will be due upon acceptance by the FDA of a filing of an NDA for Firdapse[®] for the treatment of LEMS, and approximately \$7.2 million of which will be due on the unconditional approval by the FDA of an NDA for Firdapse[®] for the treatment of LEMS). The Company also agreed to share in the cost of certain post-marketing studies being conducted by BioMarin, and, as of June 30, 2015, the Company had paid BioMarin \$3.7 million related to expenses in connection with Firdapse[®] studies and trials.

- d. **AGREEMENTS FOR DRUG DEVELOPMENT, PRE-CLINICAL AND CLINICAL STUDIES.** The Company has entered into agreements with contract manufacturers for the manufacture of drug and study placebo for the Company's trials and studies, with contract research organizations (CRO) to conduct and monitor the Company's trials and studies and with various entities for laboratories and other testing related to the Company's trials and studies. The contractual terms of the agreements vary, but most require certain advances as well as payments based on the achievement of milestones. Further, these agreements are cancellable at any time, but obligate the Company to reimburse the providers for any time or costs incurred through the date of termination.

Securities Class Action Lawsuit

The Company has settled its previously disclosed class action lawsuit. For historic information about the class action lawsuit that was filed against the Company in late 2013, see Note 7 to the Notes to Financial Statements in the Company's financial statements for the year ended December 31, 2014. In connection with the settlement, which became final on April 16, 2015, the Company paid \$3.5 million to settle this matter, all of which was paid by the Company's insurance carrier. Under the settlement, the defendants, and various of their related persons and entities, received a full release of all claims that were or could have been brought in the action, as well as all claims that arise out of, are based upon, or relate to the allegations, transactions, facts, representations, omissions or other matters involved in the action related in any way to the purchase or acquisition of the Company's securities by class members during the class period.

The settlement contains no admission of any liability or wrongdoing on the part of the defendants, each of whom continue to deny all of the allegations against each of them and believe that the claims were without merit. Because the full amount of the settlement payment was paid by the Company's insurance carrier, the settlement did not have a material adverse effect on the Company's financial position or results of operations. There were no opt outs from the

settlement.

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8. Income Taxes.

The Company is subject to income taxes in the U.S. federal jurisdiction and various states jurisdictions. Tax regulations within each jurisdiction are subject to the interpretation of the related tax laws and regulations and require significant judgment to apply. The Company is not subject to U.S. federal, state and local tax examinations by tax authorities for any years before 2010. If the Company were to subsequently record an unrecognized tax benefit, associated penalties and tax related interest expense would be reported as a component of income tax expense.

9. Stockholders Equity.

2014 Shelf Registration Statement

On January 31, 2014, the Company filed a Shelf Registration Statement on Form S-3 (the 2014 Shelf Registration Statement) with the SEC to sell up to \$100 million of shares of common stock. This registration statement (file No. 333-193699) was declared effective by the SEC on March 19, 2014. The Company has to date conducted the following sales under the 2014 Shelf Registration Statement:

- (a) On April 3, 2014, the Company filed a prospectus supplement and offered for sale 13,023,750 shares of its common stock at a price of \$2.21 per share in an underwritten public offering. The Company received gross proceeds in the public offering of approximately \$28.8 million before underwriting commission and incurred expenses of approximately \$2.1 million.
- (b) On February 4, 2015, the Company filed a prospectus supplement and offered for sale 11,500,000 shares of its common stock at a price of \$3.25 per share in an underwritten public offering. The Company received gross proceeds in the public offering of approximately \$37.4 million before underwriting commission and incurred expenses of approximately \$2.5 million.

Warrant Exercises

During the three and six month periods ended June 30, 2015, the Company issued an aggregate of 86,957 and 804,131 shares of its authorized but unissued common stock upon the exercise of previously issued common stock purchase warrants, raising gross proceeds of \$113,044 and \$1,304,070, respectively. No warrants were exercised during the three month period ended June 30, 2014. During the six month period ended June 30, 2014, the Company issued an aggregate of 12,696 shares of its authorized but unissued common stock upon the exercise of previously issued common stock purchase warrants, raising gross proceeds of \$16,504.

10. Stock Compensation.

Stock Options

During the three and six month periods ended June 30, 2015, the Company granted seven-year options to purchase an aggregate of 230,000 and 415,000 shares of the Company's common stock to employees and directors, respectively. The options vest over a period of 2 to 3 years. The Company recorded stock-based compensation related to stock options totaling \$345,080 and \$640,922 respectively, during the three and six month periods ended June 30, 2015. During the three and six month periods ended June 30, 2015, respectively, 70,000 and 95,000 options vested.

During the three and six month periods ended June 30, 2014, the Company granted five-year options to purchase an aggregate of 20,000 and 45,000 shares of the Company's common stock to employees and consultants. The Company recorded stock-based compensation related to stock options totaling \$22,570 and \$45,700 respectively, during the three and six month periods ended June 30, 2014. During the three and six month periods ended June 30, 2014, respectively, 13,333 and 38,333 options vested.

No options were exercised on a cashless basis during the three months ended June 30, 2015, or the three and six month periods ended June 30, 2014. During the six month period ended June 30, 2015, options to purchase 829,608 shares of the Company's common stock were exercised on a cashless basis, resulting in the issuance of an aggregate of 673,583 shares of the Company's common stock.

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10. Stock Compensation (continued).

As of June 30, 2015, there was approximately \$2.7 million of unrecognized compensation expense related to non-vested stock compensation awards granted under the 2006 and 2014 Stock Incentive Plans. The cost is expected to be recognized over a weighted average period of approximately 2.35 years.

Restricted Stock Units

No restricted stock units were granted during the three and six month periods ended June 30, 2015 and 2014. The Company recorded stock-based compensation related to restricted stock units totaling \$18,815 and \$37,424, respectively, during the three and six month periods ended June 30, 2015. As of June 30, 2015, there was \$178,845 of total restricted stock unit compensation expense related to non-vested awards not yet recognized, which is expected to be recognized over a weighted average period of 2.37 years.

11. Subsequent Events.

Subsequent to quarter end, the Company issued an aggregate of 329,130 shares of its authorized but unissued common stock upon the exercise of previously issued common stock warrants, raising gross proceeds of \$498,069.

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

Introduction

Management's Discussion and Analysis of Financial Condition and Results of Operations (MD&A) is intended to provide an understanding of our financial condition, changes in financial condition and results of operations. The discussion and analysis is organized as follows:

Overview. This section provides a general description of our business and information about our business that we believe is important in understanding our financial condition and results of operations.

Basis of Presentation. This section provides information about key accounting estimates and policies that we followed in preparing our financial statements for the second quarter of fiscal 2015.

Critical Accounting Policies and Estimates. This section discusses those accounting policies that are both considered important to our financial condition and results of operations, and require significant judgment and estimates on the part of management in their application. All of our significant accounting policies, including our critical accounting policies, are also summarized in the notes to our interim financial statements that are included in this report.

Results of Operations. This section provides an analysis of our results of operations for the three and six month periods ended June 30, 2015 as compared to the same periods ended June 30, 2014.

Liquidity and Capital Resources. This section provides an analysis of our cash flows, capital resources, off-balance sheet arrangements and our outstanding commitments, if any.

Caution Concerning Forward-Looking Statements. This section discusses how certain forward-looking statements made throughout this MD&A and in other sections of this report are based on management's present expectations about future events and are inherently susceptible to uncertainty and changes in circumstance.

Overview

We are a biopharmaceutical company focused on developing and commercializing innovative therapies for people with rare debilitating diseases. We currently have three drug candidates in development:

Firdapse[®] In October 2012, we licensed the North American rights to Firdapse[®], a proprietary form of amifampridine phosphate, or chemically known as 3,4-diaminopyridine phosphate, from BioMarin Pharmaceutical Inc. (BioMarin). In August 2013, we were granted breakthrough therapy designation by the U.S.

Food & Drug Administration (FDA) for Firdapse® for the treatment of patients with Lambert-Eaton Myasthenic Syndrome, or LEMS, a rare and sometimes fatal autoimmune disease characterized by muscle weakness, and, in March 2015, we were granted Orphan Drug Designation for Firdapse® for the treatment of patients with Congenital Myasthenic Syndromes, or CMS.

The chemical entity, 3,4-diaminopyridine (3,4-DAP), has never been approved by the FDA for any indication. If we are the first pharmaceutical company to obtain approval for an amifampridine-based product, we will be eligible to receive five years of marketing exclusivity with respect to the use of this product for any indication. Further, because Firdapse® for the treatment of LEMS has previously been granted Orphan Drug Designation by the FDA, the product is also eligible to receive seven years of marketing exclusivity for this indication, running concurrently with the five years of marketing exclusivity described above if both exclusivities are granted.

As part of our agreements with BioMarin, we took over the sponsorship of an ongoing Phase 3 clinical trial evaluating Firdapse® for the treatment of LEMS. The Phase 3 trial was designed as a double blind, randomized withdrawal trial in which all patients were initially treated with Firdapse® during a 91-day run-in period followed by treatment with either Firdapse® or placebo (randomly assigned, about 1:1) during a two-week randomization period. A total of 38 patients completed the three month run-in period and subsequent two week randomization period. In a trial of this design, the clinically significant findings, when present, are worsening of symptoms in the placebo group.

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On September 29, 2014, we reported top-line results from this trial. A summary of the results is as follows:

Primary endpoints:

The primary endpoint of change in quantitative myasthenia gravis score, or QMG, at day 14 reached statistical significance ($p=0.0452$), with a worsening of 2.2 points observed in the placebo group and a worsening of 0.4 points observed in the treatment group.

The primary endpoint of change in subject global impression, or SGI, at day 14 was highly statistically significant ($p=0.0028$), with a worsening of 2.6 points observed in the placebo group versus a worsening of 0.8 points observed in the treatment group.

Secondary endpoints:

The secondary endpoint for the physician's clinical global impression of improvement, or CGI-I, reached statistical significance ($p=0.0267$), with a worsening at day 14 of 1.1 points between the placebo group and the treatment group.

The secondary endpoint of change in walking speed at day 14 showed a worsening of 9.7 feet per minute in the placebo group. The magnitude of the change relative to the variance in this test prevented the change from achieving statistical significance.

Patient tolerance of Firdapse®:

Firdapse® was generally safe and well tolerated. During the 91-day open label run-in period, treatment emergent adverse events occurred more frequently in treatment-naïve patients than in previously treated patients (approximately 10% of patients withdrew during this part of the study due to adverse events). During the placebo-controlled portion of the study, side effects occurring more frequently in the Firdapse® group were benign and consisted primarily of perioral and digital paresthesias and infections. No patients withdrew during this period.

All subjects who were randomized into the trial elected to continue with Firdapse® in the two year safety follow-up phase of the trial.

During 2014, we established an expanded access program (EAP) to make Firdapse® available to any patients diagnosed with LEMS, Congenital Myasthenic Syndromes (CMS) or Downbeat Nystagmus in the United States, who meet the inclusion and exclusion criteria, with Firdapse® being provided to patients for free until sometime after NDA approval. We are working with various rare disease advocacy organizations to inform physicians and patients as to the availability of the Firdapse® EAP.

During January 2015, we met with the FDA to discuss our anticipated submission of an NDA for Firdapse® for the treatment of LEMS. Based on our discussions with the FDA, we believe that our Phase 3 clinical program will provide acceptable support for submission of an NDA for Firdapse® for LEMS.

On July 22, 2015, we announced that we have initiated a rolling submission of an NDA for Firdapse® for the treatment of LEMS. Based on currently available information, we expect to complete the submission of our NDA during the fourth quarter of 2015, at which time we intend to request a priority review for our application. If our NDA is accepted for filing by the FDA and we are granted a priority review, of which there can be no assurance, we are hopeful that we will be able to obtain an approval from the FDA of our NDA for Firdapse® in third quarter of 2016 and commercially launch Firdapse® for the treatment of LEMS sometime shortly thereafter. There can be no assurance that we will obtain an approval of our NDA for Firdapse® for LEMS.

In addition to LEMS, we plan to evaluate Firdapse® for the treatment of other neuromuscular orphan indications, including certain forms of CMS and Myasthenia Gravis (MuSK antibody positive myasthenia gravis). We are currently completing the steps that we believe will be necessary to seek to include certain forms of CMS on our initial label for Firdapse®, assuming we are successful in obtaining approval of an NDA for Firdapse®. However, there can be no assurance that we will be successful in this effort. We are also currently in the process of developing a clinical program to evaluate Firdapse® for the treatment of a certain form of Myasthenia Gravis (MuSK myasthenia gravis). There can be no assurance that Firdapse® will be determined to be effective in the treatment of MuSK myasthenia gravis.

In anticipation of the commercialization of Firdapse®, we have been taking steps to prepare for the marketing of Firdapse® in the United States. We have recruited three rare disease clinical liaisons that are working with several rare disease advocacy organizations to help increase awareness of LEMS and CMS and to provide education for the physicians who treat these rare diseases and the patients they treat. We also intend to develop a sales force of 15-20 representatives experienced

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in selling drugs that treat rare disease. This sales force will market Firdapse® to the approximately 900 neuromuscular and oncology specialists who we believe most often diagnose and treat neuromuscular diseases such as LEMS and CMS.

CPP-115. We are developing CPP-115, a GABA aminotransferase inhibitor that, based on our preclinical studies to date, we believe is a more potent form of vigabatrin, and may have fewer side effects (e.g., visual field defects, or VFDs and reduction of somnolence) than those associated with vigabatrin. We are hoping to develop CPP-115 for the treatment of epilepsy (initially infantile spasms) and for the treatment of other selected neurological indications such as complex partial seizures and Tourette s Disorder. CPP-115 has been granted Orphan Drug Designation by the FDA for the treatment of infantile spasms and Orphan Medicinal Product Designation in the European Union, or E.U., for West syndrome (a form of infantile spasms).

We are currently evaluating CPP-115 in a Phase 1(b) multi-dose safety and tolerance study. We recently revised the protocol for this study to add additional testing to assess whether surrogate markers of potential efficacy in treating Tourette s Disorder might be observed in study participants. These changes have delayed slightly our reporting of the top-line results from this study, and, based on currently available information, we now expect to report top-line results from this study during the fourth quarter of 2015. There can be no assurance that this trial will be successful.

CPP-109. During June 2015, we announced encouraging results from an academic investigator proof-of-concept study evaluating the use of CPP-109 (our formulation of vigabatrin, another GABA aminotransferase inhibitor) for the treatment of Tourette s Disorder. The 8-week clinical trial was designed as an open label trial to evaluate the potential effect of GABA-aminotransferase inhibition as a mechanism for reducing tics in patients with treatment-refractory Tourette s Disorder. Vigabatrin was used as a research surrogate in this study to demonstrate the utility of GABA-aminotransferase (GABA-AT) blockade, with the expectation that upon successfully demonstrating the utility of this mechanism, further development activities would focus on the potentially safer, more potent GABA-AT inhibitor, CPP-115.

We believe that the top-line results from this study demonstrate an encouraging signal of activity in adult treatment-refractory patients with Tourette s Disorder. Further, we believe that CPP-109 s mechanism of action validates the potential for CPP-115 to be a candidate for the treatment of Tourette s Disorder. However, there can be no assurance that CPP-115 will be effective for the treatment of refractory patients with Tourette s Disorder.

Securities Class Action Lawsuit

We have settled our previously disclosed securities class action lawsuit. For historic information about the securities class action lawsuit that was filed against us in late 2013, see Note 7 to the Notes to Financial Statements in our financial statements for the year ended December 31, 2014. In connection with the settlement, which became final on April 16, 2015, we paid \$3.5 million to settle this matter, all of which was paid by our insurance carrier. Under the settlement, the defendants, and various of their related persons and entities, received a full release of all claims that were or could have been brought in the action, as well as all claims that arise out of, are based upon, or relate to the allegations, transactions, facts, representations, omissions or other matters involved in the action related in any way to the purchase or acquisition of our securities by class members during the class period.

The settlement contains no admission of any liability or wrongdoing on the part of the defendants, each of whom continue to deny all of the allegations against each of them and believe that the claims were without merit. Because the full amount of the settlement payment was paid by our insurance carrier, the settlement did not have a material

adverse effect on our financial position or results of operations. There were no opt outs from the settlement.

Risks Associated with Product Development

The successful development of our current drug candidates or any other drug candidate we may acquire, develop or license in the future is highly uncertain. We cannot reasonably estimate or know the nature, timing, or estimated expenses of the efforts necessary to complete the development of, or the period in which material net cash inflows are expected to commence due to the numerous risks and uncertainties associated with developing such products, including the uncertainty of:

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the scope, rate of progress and expense of our clinical studies and trials, preclinical studies, proof-of-concept studies and other product development activities;

the results of our preclinical studies and clinical studies and trials, and the number of such studies and trials (and the scope of such studies and trials) that will be required for us to seek and obtain approval of our product candidates;

the risk that another pharmaceutical company will receive an approval for its formulation of amifampridine for the treatment of Lambert-Eaton Myasthenic Syndrome (LEMS) before we do;

whether the FDA will accept for filing any NDA for Firdapse[®] that we may file, and even if such NDA filing is accepted, whether the FDA will grant a priority review of that NDA; and

the expense of filing, and potentially prosecuting, defending and enforcing any patent claims and other intellectual property rights we may have for our drug candidates.

Available Capital Resources

Based on our current financial condition and forecasts of available cash, we believe that we have sufficient resources to support our operations through the end of 2016. However, we will require additional funding to support our operations beyond the end of 2016. There can be no assurance that we will obtain additional funding or that we will ever be in a position to commercialize any of our drug candidates. See [Liquidity and Capital Resources](#) below for further information on our liquidity and cash flow.

Basis of presentation

Revenues.

We are a development stage company and have had no revenues from product sales to date. We will not have revenues from product sales until such time as we receive approval of our drug candidates, successfully commercialize our products or enter into a licensing agreement which may include up-front licensing fees, of which there can be no assurance.

Research and development expenses.

Our research and development expenses consist of costs incurred for company-sponsored research and development activities, as well as occasional support for selected investigator-sponsored research. The major components of research and development costs include preclinical study costs, clinical manufacturing costs, clinical study and trial expenses, insurance coverage for clinical trials, consulting, scientific advisors and other third-party costs, salaries and employee benefits, stock-based compensation expense, supplies and materials and allocations of various overhead costs related to our product development efforts. To date, all of our research and development resources have been devoted to the development of CPP-109, CPP-115, and Firdapse[®], and we expect this to continue for the foreseeable future. Costs incurred in connection with research and development activities are expensed as incurred.

Our cost accruals for clinical studies and trials are based on estimates of the services received and efforts expended pursuant to contracts with numerous clinical study and trial sites and clinical research organizations (CROs). In the normal course of business we contract with third parties to perform various clinical study and trial activities in the on-going development of potential products. The financial terms of these agreements are subject to negotiation and vary from contract to contract and may result in uneven payment flows. Payments under the contracts depend on factors such as the achievement of certain events or milestones, the successful enrollment of patients, the allocation of responsibilities among the parties to the agreements, and the completion of portions of the clinical study or trial or similar conditions. The objective of our accrual policy is to match the recording of expenses in our financial statements to the actual services received and efforts expended. As such, expense accruals related to preclinical and clinical studies or trials are recognized based on our estimate of the degree of completion of the event or events specified in the specific study or trial contract. We monitor service provider activities to the extent possible; however, if we underestimate activity levels associated with various studies or trials at a given point in time, we could be required to record significant additional research and development expenses in future periods.

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Preclinical and clinical study and trial activities require significant up front expenditures. We anticipate paying significant portions of a study or trial's cost before such study or trial begins, and incurring additional expenditures as the study or trial progresses and reaches certain milestones.

Selling and marketing expenses.

We do not currently have any selling or marketing expenses. We are incurring costs tied to our future sales and marketing efforts, as we move closer to the potential commercialization of Firdapse®. We have started to put in place the personnel that will help us develop both a sales force and a patient advocacy and assistance program so that we are in a position to commence our selling efforts immediately if we are successful in obtaining approval of any NDA that we may file for Firdapse®, of which there can be no assurance. Pre-commercialization costs are included in general and administrative expenses.

General and administrative expenses.

General and administrative expenses consist primarily of salaries and personnel expenses for accounting, corporate and administrative functions. Other costs include administrative facility costs, regulatory fees, pre-commercialization costs, and professional fees for legal, information technology, accounting and consulting services.

Stock-based compensation.

We recognize expense for the fair value of all stock-based awards to employees, directors, scientific advisors and consultants in accordance with U.S. GAAP. For stock options we use the Black-Scholes option valuation model in calculating the fair value of the awards.

Warrants Liability.

We issued warrants to purchase shares of our common stock as part of the equity financing that we completed in October 2011. In accordance with U.S. GAAP, we have recorded the fair value of the warrants as a liability in the accompanying balance sheets at June 30, 2015 and December 31, 2014 using a Black-Scholes option-pricing model. We will re-measure the fair value of the warrants liability at each reporting date until the warrants are exercised or have expired. Changes in the fair value of the warrants liability are reported in the statements of operations as income or expense. The fair value of the warrants liability is subject to significant fluctuation based on changes in the inputs to the Black-Scholes option-pricing model, including our common stock price, expected volatility, expected life, the risk-free interest rate and dividend yield. The market price for our common stock has been and may continue to be volatile. Consequently, future fluctuations in the price of our common stock may cause significant increases or decreases in the fair value of the warrants.

Income taxes.

We have incurred operating losses since inception. Our net deferred tax asset has a 100% valuation allowance as of June 30, 2015 and December 31, 2014, as we believe it is more likely than not that the deferred tax asset will not be realized. If an ownership change, as defined under Internal Revenue Code Section 382, occurs, the use of any of our carry-forward tax losses may be subject to limitation.

As required by ASC 740, *Income Taxes*, we recognize the financial statement benefit of a tax position only after determining that the relevant tax authority would more likely than not sustain the position following the audit. For tax positions meeting the more-likely-than-not threshold, the amount recognized in the financial statements is the largest

benefit that has a greater than 50 percent likelihood of being realized upon ultimate settlement with the relevant tax authority.

Recently Issued Accounting Standards.

For discussion of recently issued accounting standards, please see Note 2, **Basis of Presentation and Significant Accounting Policies**, in the interim financial statements included in this report.

Non-GAAP Financial Measures.

We prepare our financial statements and footnotes thereto which accompany this report in accordance with U.S. Generally Accepted Accounting Principles (GAAP). To supplement our financial results presented on a GAAP basis, we may use non-GAAP financial measures in our reports filed with the Commission and/or our communications with investor. Non-GAAP measures are provided as additional information and not as an alternative to our financial statements presented in accordance with GAAP. Our non-GAAP financial measures are

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intended to enhance an overall understanding of our current financial performance. We believe that the non-GAAP financial measures we present provide investors and prospective investors with an alternative method for assessing our operating results in a manner that we believe is focused on the performance of ongoing operations and provide a more consistent basis for comparison between periods.

The non-GAAP financial measure that we typically present excludes from the calculation of net loss the expense (or the income) associated with the change in fair value of the liability-classified warrants.

Any non-GAAP financial measures that we report should not be considered in isolation or as a substitute for comparable GAAP accounting, and investors should read them in conjunction with our financial statements and notes thereto prepared in accordance with GAAP. Finally, the non-GAAP measures of net loss we may use may be different from, and not directly comparable to, similarly titled measures used by other companies.

Critical Accounting Policies and Estimates

Our discussion and analysis of our financial condition and results of operations is based on our financial statements, which have been prepared in accordance with GAAP. The preparation of these financial statements requires us to make judgments, estimates, and assumptions that affect the reported amounts of assets, liabilities, revenues, expenses and disclosures of contingent assets and liabilities. For a full discussion of our accounting policies, please refer to Note 2 on the Financial Statements included in our 2014 Annual Report on Form 10-K filed with the SEC. Our most critical accounting policies and estimates include: accounting for research and development expenses and stock-based compensation, measurement of fair value, fair value of warrants liability, income taxes and reserves. We continually evaluate our judgments, estimates and assumptions. We base our estimates on the terms of underlying agreements, our expected course of development, historical experience and other factors that we believe are reasonable based on the circumstances, the results of which form our management's basis for making judgments about the carrying value of assets and liabilities that are not readily apparent from other sources. Actual results may differ from these estimates. There have been no material changes to our critical accounting policies and estimates from the information provided in Part II, Item 7, *Management's Discussion and Analysis of Financial Condition and Results of Operations* included in our 2014 Annual Report on Form 10-K.

Results of Operations

Revenues.

We had no revenues for the three and six month periods ended June 30, 2015 and 2014.

Research and Development Expenses.

Research and development expenses for the three and six month periods ended June 30, 2015 were \$2,577,508 and \$4,927,060, respectively, including stock-based compensation expense in each of the three and six months periods of \$73,203 and \$140,144, respectively. Research and development expenses for the three and six month periods ended June 30, 2014 were \$2,098,958 and \$4,847,641 respectively, including stock-based compensation expense in each of the three and six months periods of \$12,006 and \$23,879 respectively. Research and development expenses, in the aggregate, represented approximately 53% and 54% of total operating costs and expenses for the three and six month periods ended June 30, 2015 and 70% and 75% for the three and six month periods ended June 30, 2014, respectively. The stock-based compensation is non-cash and relates to the expense of stock options awards to certain employees.

Expenses for research and development for the six month period June 30, 2015, excluding stock based compensation, increased compared to amounts expended in the same period in 2014, as we increased activities related to our NDA filing for Firdapse® and ongoing studies and trials and decreased activities related to our completed Phase 3 trial of Firdapse®.

As a result of our ongoing and projected studies and trials, as well as our efforts to complete an NDA filing for Firdapse®, we expect that costs related to research and development activities will continue to be substantial throughout the balance of 2015.

Selling and Marketing Expenses.

We had no selling expenses during the three and six months periods ended June 30, 2015 and 2014. We are incurring pre-commercialization costs, tied to our preparation for future sales and marketing efforts, as we move closer to the potential commercialization of Firdapse®. These costs are principally for personnel, and their related activities, to develop both a sales force and a patient advocacy and assistance program so that we are in a position to

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commence our selling efforts at such time as we are successful in obtaining an approval of any NDA that we may file for Firdapse[®], of which there can be no assurance. Pre-commercialization costs are included in general and administrative expenses.

General and Administrative Expenses.

General and administrative expenses for the three and six months ended June 30, 2015 were \$2,319,822 and \$4,262,185, respectively, including stock-based compensation expense in each of the three and six month periods ending June 30, 2015 of \$290,692 and \$538,202, respectively. General and administrative expenses for the three and six months ended June 30, 2014 were \$891,215 and \$1,650,897, respectively, including stock-based compensation expense in each of the three and six month periods ending June 30, 2014 of \$10,564 and \$21,821, respectively. General and administrative expenses represented 47% and 46% of total operating costs and expenses for the three and six months ended June 30, 2015 and 30% and 25% for the three and six months ended June 30, 2014, respectively. The stock-based compensation is non-cash and relates to the expense of stock option awards and restricted stock units to employees, directors and consultants. The increase in general and administrative expenses for the six month period ended June 30, 2015 when compared to the same period in 2014 is primarily due to increases in pre-commercialization expenses, payroll and benefits expenses, investor relations expenses and travel expenses. We expect general and administrative expenses to increase in future periods as we expand our operations and headcount in preparation for the future commercialization of Firdapse[®].

Stock-Based Compensation.

Total stock-based compensation for the three and six month periods ended June 30, 2015 were \$363,895 and \$678,346 and for the three and six month periods ended June 30, 2014 were \$22,570 and \$45,700, respectively. The increase in stock-based compensation for the six month periods ended June 30, 2015 when compared to the same period in 2014, is primarily due to the 2015 amortization of stock options and restricted stock grants made in 2014.

Change in fair value of warrants liability.

In connection with our October 2011 equity offering, we issued warrants to purchase an aggregate of 1,523,370 shares of common stock. The fair value of the portion of these warrants which remain outstanding is recorded in the liability section of the balance sheet and was estimated at \$2,979,038 and \$2,794,891 at June 30, 2015 and December 31, 2014, respectively. The fair value of the warrants liability is determined at the end of each reporting period with the resulting gains or losses recorded as the change in fair value of warrants liability in the statements of operations. For the three and six months ended June 30, 2015, we recognized a gain of \$333,956 and a loss of \$846,322, respectively, due to the change in the fair value of the warrants liability. The gain during the three months ended June 30, 2015 was principally a result of the decrease of our stock price between March 31, 2015 and June 30, 2015. The loss during the six months ended June 30, 2015 was principally a result of the increase of our stock price between December 31, 2014 and June 30, 2015. For the three and six months ended June 30, 2014, we recognized losses of \$223,591 and \$559,105 due to the change in the fair value of the warrants liability. The losses during the three and six months ended June 30, 2014 were principally a result of the increases of our stock price between March 31, 2014 and June 30, 2014, and December 31, 2013 and June 30, 2014, respectively. We believe, future changes in the fair value of the warrants liability will be due primarily to fluctuations in the value of our common stock and the timing of warrant exercises.

Other Income, Net.

We reported other income, net in all periods relating to our investment of funds received from offerings of our securities. The increase in other income, net for the six month period ended June 30, 2015 when compared to the same

period in 2014 is due to higher average investment balances from the proceeds of our offerings. Other income net, consists of interest income, dividend income and unrealized and realized gain (loss) on trading securities. These proceeds were used to fund our product-development activities and our operations. Substantially all such funds were invested in short-term interest bearing obligations and short-term bond funds.

Income taxes.

We have incurred net operating losses since inception. For the three and six month periods ended June 30, 2015 and 2014, we have applied a 100% valuation allowance against our deferred tax asset as we believe that it is more likely than not that the deferred tax asset will not be realized.

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Net Loss.

Our net loss was \$4,558,503 and \$9,968,762, respectively, for the three and six months ended June 30, 2015 (\$0.06 and \$0.13, respectively, per basic and diluted share) as compared to a net loss of \$3,198,020 and \$7,009,139, respectively, for the three and six months ended June 30, 2014 (\$0.05 and \$0.12, respectively, per basic and diluted share).

Non-GAAP Net Loss.

Our non-GAAP net loss, which excludes for the three and six months ended June 30, 2015 a gain of \$333,956 and a loss of \$846,322, respectively, associated with the change in the fair value of liability classified warrants, was \$4,892,459 and \$9,122,440 for the three and six months ended June 30, 2015 (\$0.06 and \$0.12, respectively, per basic and diluted share). Our non-GAAP net loss, which excludes for the three and six months ended June 30, 2014 losses of \$223,591 and \$559,105 associated with the change in the fair value of liability classified warrants, was \$2,974,429 and \$6,450,034 for the three and six months ended June 30, 2014 (\$0.05 and \$0.11, respectively, per basic and diluted share).

Liquidity and Capital Resources

Since our inception, we have financed our operations primarily through equity issuances, government grants, and an investment by a strategic purchaser. At June 30, 2015, we had cash and cash equivalents, certificates of deposit and short-term investments aggregating \$67.4 million and working capital of \$65.7 million. At December 31, 2014, we had cash and cash equivalents, certificates of deposit and short term investments aggregating \$39.3 million and working capital of \$38.0 million. At June 30, 2015, substantially all of our cash and cash equivalents were deposited with one financial institution, and such balances were in excess of federally insured limits.

We have to date incurred operating losses, and we expect these losses to be substantial in the future as we expand our product development programs and prepare for the commercialization of our product candidates. We anticipate using current cash on hand to finance these activities.

We cu