

CATALYST PHARMACEUTICALS, INC.

Form 10-Q

November 07, 2018

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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 September 30, 2018**

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)**

<b>Delaware</b> <b>(State or other jurisdiction of</b>	<b>76-0837053</b> <b>(IRS Employer</b>
<b>incorporation or organization)</b>	<b>Identification No.)</b>
<b>355 Alhambra Circle</b>	
<b>Suite 1250</b>	
<b>Coral Gables, Florida</b>	<b>33134</b>
<b>(Address of principal executive offices)</b>	<b>(Zip Code)</b>
<b>Registrant's telephone number, including area code: <u>(305) 420-3200</u></b>	

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 every Interactive Data File required to be submitted 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 such files). Yes No

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

Large accelerated filer

Accelerated Filer

Non-accelerated filer

Smaller reporting company

Emerging growth company

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

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 102,739,257 shares of common stock, \$0.001 par value per share, were outstanding as of November 2, 2018.



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	<b>September 30, 2018</b>	<b>December 31, 2017</b>
	(unaudited)	
<b>ASSETS</b>		
Current Assets:		
Cash and cash equivalents	\$ 10,616,313	\$ 57,496,702
Short-term investments	51,047,842	26,516,711
Prepaid expenses and other current assets	816,820	1,173,744
Total current assets	62,480,975	85,187,157
Investments	5,018,857	
Property and equipment, net	201,093	191,385
Deposits	8,888	8,888
Total assets	\$ 67,709,813	\$ 85,387,430
<b>LIABILITIES AND STOCKHOLDERS EQUITY</b>		
Current Liabilities:		
Accounts payable	\$ 1,340,984	\$ 1,945,575
Accrued expenses and other liabilities	2,127,450	2,320,587
Total current liabilities	3,468,434	4,266,162
Accrued expenses and other liabilities, non-current	164,781	157,456
Total liabilities	3,633,215	4,423,618
Commitments and contingencies		
Stockholders' equity:		
Preferred stock, \$0.001 par value, 5,000,000 shares authorized: none issued and outstanding at September 30, 2018 and December 31, 2017		
Common stock, \$0.001 par value, 150,000,000 shares authorized; 102,689,257 shares and 102,549,498 shares issued and outstanding at September 30, 2018 and December 31, 2017, respectively	102,689	102,549
Additional paid-in capital	210,084,209	207,421,710
Accumulated deficit	(146,064,352)	(126,560,447)
Accumulated other comprehensive loss	(45,948)	
Total stockholders' equity	64,076,598	80,963,812
Total liabilities and stockholders' equity	\$ 67,709,813	\$ 85,387,430

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

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## CATALYST PHARMACEUTICALS, INC.

## CONSOLIDATED STATEMENTS OF OPERATIONS AND COMPREHENSIVE LOSS (unaudited)

	For the Three Months Ended September 30,		For the Nine Months Ended September 30,	
	2018	2017	2018	2017
Operating costs and expenses:				
Research and development	\$ 4,538,369	\$ 2,704,923	\$ 11,502,235	\$ 7,970,603
General and administrative	3,644,234	1,601,785	8,949,663	5,197,247
Total operating costs and expenses	8,182,603	4,306,708	20,451,898	13,167,850
Loss from operations	(8,182,603)	(4,306,708)	(20,451,898)	(13,167,850)
Other income, net	343,730	129,059	947,993	330,075
Change in fair value of warrants liability				(186,904)
Loss before income taxes	(7,838,873)	(4,177,649)	(19,503,905)	(13,024,679)
Provision for income taxes				
Net loss	\$ (7,838,873)	\$ (4,177,649)	\$ (19,503,905)	\$ (13,024,679)
Net loss per share basic and diluted	\$ (0.08)	\$ (0.05)	\$ (0.19)	\$ (0.16)
Weighted average shares outstanding basic and diluted	102,641,504	84,797,969	102,598,740	83,898,724
Net loss	\$ (7,838,873)	\$ (4,177,649)	\$ (19,503,905)	\$ (13,024,679)
Other comprehensive loss:				
Unrealized gain (loss) on available-for-sale securities	(9,450)		(45,948)	
Comprehensive loss	\$ (7,848,323)	\$ (4,177,649)	\$ (19,549,853)	\$ (13,024,679)

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

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## CATALYST PHARMACEUTICALS, INC.

## CONSOLIDATED STATEMENT OF STOCKHOLDERS EQUITY (unaudited)

For the nine months ended September 30, 2018

	Preferred Stock	Common Stock	Additional Paid-in Capital	Accumulated Deficit	Accumulated Other Comprehensive Loss	Total
<b>Balance at December 31, 2017</b>	\$	\$ 102,549	\$ 207,421,710	\$ (126,560,447)	\$	\$ 80,963,812
Issuance of common stock, net		3	10,546			10,549
Issuance of stock options for services			2,506,026			2,506,026
Exercise of stock options for common stock		137	145,927			146,064
Other comprehensive loss					(45,948)	(45,948)
Net loss				(19,503,905)		(19,503,905)
<b>Balance at September 30, 2018</b>	\$	\$ 102,689	\$ 210,084,209	\$ (146,064,352)	\$ (45,948)	\$ 64,076,598

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



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## CATALYST PHARMACEUTICALS, INC.

## CONSOLIDATED STATEMENTS OF CASH FLOWS (unaudited)

	For the Nine Months Ended September 30,	
	2018	2017
<b>Operating Activities:</b>		
Net loss	\$ (19,503,905)	\$ (13,024,679)
Adjustments to reconcile net loss to net cash used in operating activities:		
Depreciation	25,485	38,754
Stock-based compensation	2,521,023	1,922,451
Change in fair value of warrants liability		186,904
(Increase) decrease in:		
Prepaid expenses and other current assets and deposits	356,924	537,452
Increase (decrease) in:		
Accounts payable	(604,591)	148,432
Accrued expenses and other liabilities	(185,812)	477,981
Net cash used in operating activities	(17,390,876)	(9,712,705)
<b>Investing Activities:</b>		
Purchases of property and equipment	(35,193)	
Purchases of investments	(37,141,968)	(64,748)
Proceeds from maturities of investments	7,546,032	
Net cash provided by (used in) investing activities	(29,631,129)	(64,748)
<b>Financing Activities:</b>		
Payment of employee withholding tax related to stock-based compensation	(4,448)	
Proceeds from exercise of warrants		3,209,423
Proceeds from exercise of stock options	146,064	3,950
Net cash provided by (used in) financing activities	141,616	3,213,373
Net increase (decrease) in cash and cash equivalents	(46,880,389)	(6,564,080)
Cash and cash equivalents beginning of period	57,496,702	13,893,064
Cash and cash equivalents end of period	\$ 10,616,313	\$ 7,328,984
<b>Non-cash investing and financing activities:</b>		
Unrealized gain (loss) on available-for-sale securities	\$ (45,948)	\$
Exercise of liability classified warrants for common stock	\$	\$ 309,130

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



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**CATALYST PHARMACEUTICALS, INC.**

**NOTES TO UNAUDITED CONSOLIDATED FINANCIAL STATEMENTS**

**1. Organization and Description of Business.**

Catalyst Pharmaceuticals, Inc. (the Company) is a development-stage biopharmaceutical company focused on developing and commercializing innovating therapies for people with rare debilitating, chronic neuromuscular and neurological diseases, including Lambert-Eaton Myasthenic Syndrome (LEMS), Congenital Myasthenic Syndromes (CMS), and MuSK antibody positive myasthenia gravis (MuSK-MG).

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 September 30, 2018. 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 10.

*Capital Resources*

While there can be no assurance, based on currently available information, the Company estimates that it has sufficient resources to support its operations for at least the next 12 months.

The Company may raise additional funds in the future, if required for its business, 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 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 consolidated 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 Securities and Exchange Commission (SEC) for reporting of interim financial information. Pursuant to such rules and regulations, certain information and note disclosures normally included in financial statements prepared in accordance with U.S. GAAP have been omitted. The consolidated balance sheet as of December 31, 2017 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 consolidated 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 consolidated statements should be read in conjunction with the financial statements and notes thereto for the year ended December 31, 2017 included in the 2017 Annual Report on Form 10-K filed by the Company with the SEC. The results of operations for the nine months ended September 30, 2018 are not necessarily indicative of the results to be expected for any future period or for the full 2018 fiscal year.

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

- b. PRINCIPLES OF CONSOLIDATION.** The consolidated financial statements include the Company's accounts and those of its wholly-owned subsidiary Catalyst Pharmaceuticals Ireland, Ltd. (Catalyst Ireland). All intercompany accounts and transactions have been eliminated in consolidation. Catalyst Ireland was organized in 2017.
- c. 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.
- d. 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. These amounts at times may exceed federally insured limits.
- e. INVESTMENTS.** The Company invests in high credit-quality funds in order to obtain higher yields on its cash available for investments. At September 30, 2018, investments consisted of a short-term bond fund and U.S. Treasuries. At December 31, 2017, investments consisted of a short-term bond fund. Such investments are not insured by the Federal Deposit Insurance Corporation.

*Short-term bond fund*

The short-term bond fund is classified in 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. At September 30, 2018 and December 31, 2017, the only investment classified as trading securities was the short-term bond fund. Unrealized gain (loss) on trading securities was \$0 and (\$29,430), respectively, for the three and nine months ended September 30, 2018, and \$29,431 and \$58,861 for the three and nine months ended September 30, 2017 and is included in other income, net in the accompanying consolidated statements of operations.

*U.S. Treasuries*

U.S. Treasuries are classified as available-for-sale securities. The Company classifies available-for-sale securities with stated maturities of greater than three months and less than one year from the date of purchase as short-term investments. Available-for-sale securities with stated maturities greater than one year are classified as non-current investments in the accompanying consolidated balance sheets. The Company records available-for-sale securities at fair value with unrealized gains and losses reported in accumulated other comprehensive income (loss) in stockholders equity. Realized gains and losses are included in other income, net and are derived using the specific identification method for determining the cost of securities sold. Interest income is recognized when earned and is included in other income, net in the consolidated statements of operations. The Company recognizes a charge when the declines in the fair value below the amortized cost basis of its available-for-sale securities are judged to be other-than-temporary. The Company considers various factors in determining whether to recognize an other-than-temporary charge, including

whether the Company intends to sell the security or whether it is more likely than not that the Company would be required to sell the security before recovery of the amortized cost basis. The Company has not recorded any other than temporary impairment charges on its available-for-sale securities. See Note 4.

- f. PREPAID EXPENSES AND OTHER CURRENT ASSETS.** Prepaid expenses and other current assets consist primarily of prepaid research fees, prepaid pre-commercialization expenses, prepaid insurance and prepaid subscription fees. Prepaid research fees consist of advances for the Company's product development activities, including drug manufacturing, contracts for pre-clinical 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.

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

**g. FAIR VALUE OF FINANCIAL INSTRUMENTS.** The Company's financial instruments consist of cash and cash equivalents, short-term investments, accounts payables, accrued expenses and other liabilities, and warrants liability. At September 30, 2018 and December 31, 2017, 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 it believes market participants would use in pricing assets or liabilities (unobservable inputs classified within Level 3 of the hierarchy).

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 are 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 September 30, 2018	Quoted Prices in Active Markets for Identical Assets/Liabilities (Level 1)	Significant Other Observable Inputs (Level 2)	Significant Unobservable Inputs (Level 3)
<i>Cash and cash equivalents:</i>				
Money market funds	\$ 10,372,148	\$ 10,372,148	\$	\$
<i>Short-term investments:</i>				

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Short-term bond fund	\$ 26,534,352	\$ 26,534,352	\$	\$
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U.S. Treasuries	\$ 24,513,490	\$	\$ 24,513,490	\$
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*Investments:*

U.S. Treasuries	\$ 5,018,857	\$	\$ 5,018,857	\$
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	Balances as of December 31, 2017	Quoted Prices in Active Markets for Identical Assets/ Liabilities (Level 1)	Significant Other Observable Inputs (Level 2)	Significant Unobservable Inputs (Level 3)
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*Cash and cash equivalents:*

Money market funds	\$ 56,820,688	\$ 56,820,688	\$	\$
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*Short-term investments:*

Short-term bond fund	\$ 26,516,711	\$ 26,516,711	\$	\$
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**Table of Contents****2. Basis of Presentation and Significant Accounting Policies (continued).**

- 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. During the period that the 2011 warrants were outstanding, the Company accounted for these warrants as a liability measured at fair value due to a provision included in the warrants agreement that provided 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, occurred. The fair value of the warrants liability was estimated using the Black-Scholes Model which required inputs such as the expected term of the warrants, share price volatility and risk-free interest rate. These assumptions were reviewed on a quarterly basis and changes in the estimated fair value of the outstanding warrants were recognized each reporting period in the Change in fair value of warrants liability line in the consolidated statement of operations. All unexercised 2011 warrants expired on May 2, 2017. See Note 3.
- j. STOCK-BASED COMPENSATION.** The Company recognizes expense in the consolidated statements of operations for the fair value of all stock-based payments to employees, directors, scientific advisors and consultants, 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 one to five years. Forfeitures are recognized as a reduction of stock-based compensation expense as they occur.

As of September 30, 2018, there were outstanding stock options to purchase 8,062,500 shares of common stock, of which stock options to purchase 4,011,663 shares of common stock were exercisable as of September 30, 2018.

For the three and nine-month periods ended September 30, 2018 and 2017, the Company recorded stock-based compensation expense as follows:

	Three months ended September 30,		Nine months ended September 30,	
	2018	2017	2018	2017
Research and development	\$ 240,122	\$ 192,796	\$ 820,062	\$ 622,700
General and administrative	518,054	336,942	1,700,961	1,299,751
<b>Total stock-based compensation</b>	<b>\$ 758,176</b>	<b>\$ 529,738</b>	<b>\$ 2,521,023</b>	<b>\$ 1,922,451</b>

- 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. The Company's comprehensive loss is shown on the Consolidated Statements of

Operations and Comprehensive Loss for the three and nine months ended September 30, 2018 and is comprised of net unrealized losses on the Company's available-for-sale securities. For December 31, 2017 and all prior periods, the Company's net loss equaled comprehensive loss, since the Company had no items which were considered other comprehensive income (loss).

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

- 1. NET LOSS PER SHARE.** Basic net 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:

	<b>September 30,</b>	
	<b>2018</b>	<b>2017</b>
Options to purchase common stock	8,062,500	6,085,000
Unvested restricted stock		26,667
Potential equivalent common stock excluded	8,062,500	6,111,667

Potentially dilutive options to purchase common stock as of September 30, 2018 have exercise prices ranging from \$0.79 to \$4.64. Potentially dilutive options to purchase common stock as of September 30, 2017 had exercise prices ranging from \$0.47 to \$4.64.

- m. RECENTLY ISSUED ACCOUNTING STANDARDS.** In February 2016, the FASB issued ASU No. 2016-02, *Leases (Topic 842)*, which requires an entity to recognize assets and liabilities arising from a lease for both financing and operating leases. The ASU will also require new qualitative and quantitative disclosures to help investors and other financial statement users better understand the amount, timing, and uncertainty of cash flows arising from leases. ASU 2016-02 is effective for fiscal years beginning after December 15, 2018, with early adoption permitted. The Company plans to adopt the standard as of January 1, 2019, the beginning of fiscal 2019, using the modified retrospective approach in which prior comparative periods are not adjusted. The Company will elect the package of practical expedients permitted under the transition guidance within the new standard, which among other things, allows the Company to carry forward historical lease classification. The Company has operating leases for its office facilities, which expire on November 30, 2022. The Company anticipates recognition of an additional asset and corresponding liability related to its facility lease on the consolidated balance sheet. This standard will have a material impact on the Company's consolidated financial statements.

In May 2017, the FASB issued ASU No. 2017-09, *Compensation - Stock Compensation (Topic 718): Scope of Modification Accounting* to clarify when to account for a change to the terms or conditions of a share-based payment award as a modification. Under this new guidance, modification accounting is required if the fair value, vesting conditions, or classification of the award changes as a result of the change in terms or conditions. ASU 2017-09 is effective for all entities for annual reporting periods beginning after December 15, 2017, including interim reporting periods within each annual reporting period, applied prospectively on or after the effective date. The Company adopted this standard in the first quarter of 2018. The adoption of this standard did not have a material impact on the Company's consolidated financial statements.

In June 2018, the FASB issued ASU No. 2018-07, *Compensation - Stock Compensation (Topic 718): Improvements to Nonemployee Share-Based Payment Accounting* that largely aligns the accounting for share-based payment awards issued to employees and nonemployees. Under this ASU, most of the guidance on such payments to nonemployees would be aligned with the requirements for share-based payments granted to employees. ASU 2018-07 is effective for all entities for annual reporting periods beginning after December 15, 2018, including interim reporting periods within each annual reporting period, with early adoption permitted. The Company is currently evaluating the impact this accounting standard will have on its consolidated financial statements.

- n. **RECLASSIFICATIONS.** Certain prior year amounts in the consolidated financial statements have been reclassified to conform to the current year presentation.

**Table of Contents****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 were classified as a liability (the 2011 warrants). The 2011 warrants were classified as a liability because of provisions in such warrants that allowed for the net cash settlement of such warrants in the event of certain fundamental transactions (as defined in the warrant agreement). During the period that the 2011 warrants were outstanding, the valuation of the 2011 warrants was 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 had 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; and the risk-free rate of return. Of those inputs, the exercise price of the warrants and the remaining term were readily observable in the warrant agreement. The annual rate of dividends was 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 was a Level 2 input, while the historical volatility was a Level 3 input in accordance with the fair value accounting guidance. Since the lowest level input was a Level 3, the Company determined the 2011 warrants liability were most appropriately classified within Level 3 of the fair value hierarchy. This liability was subject to a fair value mark-to-market adjustment each reporting period.

The following table rolls forward the fair value of the Company's warrants liability activity for the three and nine-month periods ended September 30, 2017:

	<b>Three months ended September 30, 2017</b>	<b>Nine months ended September 30, 2017</b>
Fair value, beginning of period	\$	\$ 122,226
Issuance of warrants		
Exercise of warrants		(309,130)
Change in fair value		186,904
Fair value, end of period	\$	\$

On May 2, 2017, the outstanding and unexercised 2011 warrants expired. During the nine months ended September 30, 2017, 613,913 of the 2011 warrants were exercised, with proceeds of \$798,087 to the Company.

**4. Investments.**

Available-for-sale investments by security type were as follows:

		Amortized cost	Gross Unrealized Gains	Gross Unrealized Losses	Estimated Fair Value
<b>At September 30, 2018:</b>					
U.S. Treasuries	ST	\$ 24,524,244	\$	\$ (10,754)	\$ 24,513,490
U.S. Treasuries	LT	5,054,051		(35,194)	5,018,857
Total		\$ 29,578,295	\$	\$ (45,948)	\$ 29,532,347

At December 31, 2017, the Company did not have any available-for-sale securities.

**Table of Contents****4. Investments (continued).**

In accordance with FASB ASC Topic 320, Investments – Debt and Equity Securities, or ASC 320, the Company has classified its U.S. Treasuries as available-for-sale securities with secondary or resale markets, and, as such, they are reported at fair value with unrealized gains and losses included in comprehensive loss in stockholders' equity and realized gain and losses, included in other income, net. There were no realized gains or losses from available-for-sale securities for the three or nine months ended September 30, 2018 or 2017.

Certain U.S. Treasuries at September 30, 2018 had fair values less than their amortized costs and, therefore, contained unrealized losses. Given that the Company has no intent to sell the U.S. Treasuries until a recovery of the fair value, which may be at maturity, and there are no current requirements to sell any of these securities, the Company did not consider these investments to be other-than-temporarily impaired as of September 30, 2018. The Company anticipates full recovery of amortized costs with respect to these investments at maturity. The duration of time the U.S. Treasuries have been in a continuous unrealized loss position as of September 30, 2018 was less than 9 months.

The estimated fair values of available-for-sale securities at September 30, 2018, by contractual maturity, are summarized as follows:

	<b>September 30, 2018</b>
Due in one year or less	\$ 24,513,490
Due after one year but within two years	5,018,857
	<b>\$ 29,532,347</b>

**5. Prepaid Expenses and Other Current Assets.**

Prepaid expenses and other current assets consist of the following:

	<b>September 30, 2018</b>	<b>December 31, 2017</b>
Prepaid research fees	\$ 301,837	\$ 388,977
Prepaid insurance	116,774	638,139
Prepaid pre-commercialization fees	121,706	65,000
Prepaid subscription fees	63,288	23,347
Prepaid rent	21,924	
Other	191,291	58,281
<b>Total prepaid expenses and other current assets</b>	<b>\$ 816,820</b>	<b>\$ 1,173,744</b>

**6. Property and Equipment, Net.**

Property and equipment, net consists of the following:

	<b>September 30, 2018</b>	<b>December 31, 2017</b>
Computer equipment	\$ 27,915	\$ 27,915
Furniture and equipment	191,835	169,931
Leasehold improvements	165,997	152,708
	385,747	350,554
Less: Accumulated depreciation	(184,654)	(159,169)
Total property and equipment, net	\$ 201,093	\$ 191,385

Depreciation expense was \$9,294 and \$25,485, respectively, for the three and nine-month periods ended September 30, 2018 and \$12,839 and \$38,754, respectively for the three and nine-month periods ended September 30, 2017.



**Table of Contents****7. Accrued Expenses and Other Liabilities.**

Accrued expenses and other liabilities consist of the following:

	<b>September 30,</b>	<b>December 31,</b>
	<b>2018</b>	<b>2017</b>
Accrued preclinical and clinical trial expenses	\$ 896,566	\$ 970,649
Accrued professional fees	429,073	227,457
Accrued compensation and benefits	740,939	821,935
Accrued license fees		252,500
Deferred rent and lease incentive	21,914	24,011
Other	38,958	24,035
<b>Current accrued expenses and other liabilities</b>	<b>2,127,450</b>	<b>2,320,587</b>
Deferred rent and lease incentive non-current	164,781	157,456
<b>Non-current accrued expenses and other liabilities</b>	<b>164,781</b>	<b>157,456</b>
<b>Total accrued expenses and other liabilities</b>	<b>\$ 2,292,231</b>	<b>\$ 2,478,043</b>

**8. Commitments and Contingencies.****a. LICENSE AGREEMENT WITH NORTHWESTERN UNIVERSITY (CPP-115).**

On August 27, 2009, the Company entered into a license agreement with Northwestern University (Northwestern), under which it acquired worldwide rights to CPP-115. As of September 30, 2018, the Company had paid \$424,885 in connection with the license and had accrued license fees of \$0 in the accompanying September 30, 2018 consolidated balance sheet for expenses, maintenance fees and milestones.

Recently, the Company became aware that certain patents granted to Northwestern in 2018 (which patents have been licensed by Northwestern to a third-party) for a new GABA aminotransferase inhibitor were derived from CPP-115. As a result, it is the Company's position that Northwestern has violated the license agreement based on its failure to transfer these new patent rights to the Company as part of the existing license agreement. It is also the Company's position that Northwestern's publication of information about the new patents in violation of the license agreement has damaged the Company. On October 26, 2018, because good faith efforts to resolve these disputes had not been successful, the Company notified Northwestern that it was terminating the license agreement and seeking damages for Northwestern's breach of the license agreement. Further, on the same date, the Company filed a claim for damages in arbitration against Northwestern for Northwestern's breaches of the license agreement.

On November 5, 2018, Northwestern advised the Company that in its view, Northwestern has a right to terminate the license agreement with the Company because the Company has allegedly breached the license agreement by failing to pay certain milestones and by allegedly failing to use commercially reasonable efforts to develop and commercialize any products. Northwestern has also advised the Company that, in its view, the Company has engaged in wrongful conduct and communications with the third party that licensed the new patents from Northwestern, and that such communications have damaged Northwestern's relationship with that third party. The Company disputes

Northwestern's allegations and intends to vigorously defend itself against any claims that Northwestern may bring against it in the arbitration proceedings relating to these allegations.

This matter is at a very early stage and there can be no assurance as to the outcome of this matter. See Note 12.

- 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 of 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.

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**8. Commitments and Contingencies (continued).**

- c. LICENSE AGREEMENT WITH BIOMARIN (FIRDAPSE).** On October 26, 2012, the Company entered into a license agreement with BioMarin Pharmaceutical, Inc. (BioMarin) for the North American rights to Firdapse®.

Under the License Agreement, the Company has agreed to pay: (i) royalties to BioMarin 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; and (ii) royalties to the third-party licensor of the rights sublicensed to the Company 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.

Additionally, the Company's license agreement with BioMarin requires the Company to pay certain milestone payments that BioMarin is obligated to pay to both a third-party licensor of the rights that have been sublicensed to the Company and to the former stockholders of Huxley Pharmaceuticals ( Huxley ) under an earlier stock purchase agreement between BioMarin and the former Huxley stockholders.

With respect to the third party licensor of the rights that have been sublicensed to the Company, the Company has agreed to pay: (i) \$150,000 upon acceptance by the FDA of a filing of an NDA for Firdapse® for the treatment of LEMS (which amount was paid during the second quarter of 2018 after acceptance by the FDA of the Company's NDA for Firdapse® for LEMS), and (ii) approximately \$3.0 million of which will be due upon the unconditional approval by the FDA of an NDA for Firdapse® for the treatment of LEMS (which milestone payment has not yet been earned).

With respect to the former Huxley stockholders, the Company had agreed that it would pay the following milestone payments if either of the following milestones were satisfied before April 20, 2018: (i) \$2,425,000 upon acceptance by the FDA of a filing of an NDA for Firdapse® for the treatment of LEMS, and (ii) approximately \$4,200,000 of which was to be paid upon the unconditional approval by the FDA of an NDA for Firdapse® for the treatment of LEMS. Since neither of these milestones were met on or before April 20, 2018, these milestone payments were never earned. However, prior to April 20, 2018, the Company was advised that the former Huxley stockholders intended to take legal action against BioMarin and the Company seeking payment of the milestone payments due to them even if the milestones were achieved after their expiration date (April 20, 2018). While the Company disputed its obligation to pay either of the above described milestone payments if these milestones were achieved after April 20, 2018, in an agreement which was executed and became effective on July 26, 2018, the Company, BioMarin and the former Huxley stockholders agreed to amicably resolve this matter. As part of the settlement, and without admitting any liability, the Company paid the former Huxley stockholders a \$1.0 million milestone payment upon execution of the settlement agreement, and agreed to pay a \$1.0 million payment upon the unconditional approval by the FDA of an NDA for Firdapse® for the treatment of LEMS (which milestone payment has not yet been earned). Further, as part of the settlement agreement, the Company, BioMarin and the former Huxley stockholders entered into mutual releases regarding these matters.

The Company also agreed to share in the cost of certain post-marketing studies being conducted by BioMarin, and, as of September 30, 2018, the Company had paid BioMarin \$3.8 million related to expenses in connection with Firdapse® studies and trials.



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**8. Commitments and Contingencies (continued).**

- 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.

**9. 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 2015. 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.

The Company's net deferred tax asset has a 100% valuation allowance at September 30, 2018 and December 31, 2017 as the Company believes that it is more likely than not that the deferred tax asset will not be realized.

**10. Stockholders Equity.**

***2016 Shelf Registration Statement***

On December 23, 2016, the Company filed a shelf Registration Statement on Form S-3 (the 2016 Shelf Registration Statement) with the SEC to sell up to approximately \$33.8 million of common stock. The 2016 Shelf Registration Statement (file No. 333-215315) was declared effective by the SEC on January 9, 2017. No sales have been conducted to date under the 2016 Shelf Registration Statement.

***2017 Shelf Registration Statement***

On July 12, 2017, the Company filed a universal shelf Registration Statement on Form S-3 (the 2017 Shelf Registration Statement) with the SEC to sell up to \$150 million of common stock, preferred stock, warrants to purchase common stock, or debt securities (including debt securities that may be convertible or exchangeable for common stock or other securities), which securities may be offered separately or together in units or multiple series. The 2017 Shelf Registration Statement (file No. 333-219259) was declared effective by the SEC on July 26, 2017.

On November 28, 2017, the Company filed a prospectus supplement and offered for sale 16,428,572 shares of its common stock at a price of \$3.50 per share in an underwritten public offering under the 2017 Shelf Registration. The Company received gross proceeds in the public offering of approximately \$57.5 million before underwriting commission and incurred expenses of approximately \$3.7 million.

At September 30, 2018, there is approximately \$92.5 million available for future sale under the 2017 Shelf Registration Statement.

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**11. Stock Compensation.**

*Stock Options*

During the three and nine-month periods ended September 30, 2018, the Company granted seven-year term options to purchase an aggregate of 550,000 shares and 3,267,500 shares, respectively, of the Company's common stock to employees and directors. The Company recorded stock-based compensation related to stock options totaling \$758,176 and \$2,506,026 respectively, during the three and nine-month periods ended September 30, 2018. During the three and nine-month periods ended September 30, 2018, 5,000 options and 1,314,998 options vested, respectively.

During the three and nine-month periods ended September 30, 2017, the Company granted seven-year term options to purchase an aggregate of 0 shares and 1,535,000 shares, respectively, of the Company's common stock to employees and directors. The Company recorded stock-based compensation related to stock options totaling \$510,715 and \$1,866,005 respectively, during the three and nine-month periods ended September 30, 2017. During the three and nine-month periods ended September 30, 2017, 261,668 options and 1,138,335 options vested, respectively.

During the three and nine-month periods ended September 30, 2018, options to purchase 89,999 shares and 136,665 shares, respectively, of the Company's common stock were exercised, with proceeds of \$104,532 and \$146,064, respectively, to the Company.

During the three and nine months ended September 30, 2017, options to purchase 5,000 shares of the Company's common stock were exercised, with proceeds of \$3,950 to the Company.

As of September 30, 2018, there was approximately \$6,204,000 of unrecognized compensation expense related to non-vested stock option awards granted under the 2014 and 2018 Stock Incentive Plans. The cost is expected to be recognized over a weighted average period of approximately 2.56 years.

*Restricted Stock Units*

No restricted stock units were granted during the three and nine-month periods ended September 30, 2018 and 2017. No stock-based compensation related to restricted stocks was recorded during the three and nine-months periods ended September 30, 2018. The Company recorded stock-based compensation related to restricted stock units totaling \$19,023 and \$56,446, respectively, during the three and nine-month periods ended September 30, 2017. All restricted stock units were vested as of December 31, 2017.

*Common Stock*

No shares of common stock were granted during the three-month period ended September 30, 2018. During the nine-month period ended September 30, 2018, the Company granted 3,094 net shares of common stock to employees as compensation. The Company recorded stock-based compensation related to common stock issued to employees totaling \$0 and approximately \$15,000, respectively, during the three and nine-month periods ended September 30, 2018. There were no grants of common stock to employees during the three and nine-month periods ended September 30, 2017.

**12. Subsequent Events.**

Subsequent to quarter end, the Company commenced an arbitration proceeding against Northwestern in regard to CPP-155. See Note 8.





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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 the current status of 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 consolidated financial statements for the third quarter of fiscal 2018.

*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 consolidated 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 nine-months ended September 30, 2018 as compared to the same periods ended September 30, 2017.

*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, chronic neuromuscular and neurological diseases. We currently have the following drug candidates in development:

Firdapse®

In October 2012, we licensed the North American rights to Firdapse<sup>®</sup>, 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<sup>®</sup> for the treatment of patients with Lambert-Eaton Myasthenic Syndrome, or LEMS, a rare and sometimes fatal autoimmune disease characterized by muscle weakness. Further, the FDA has granted Orphan Drug Designation for Firdapse<sup>®</sup> for the treatment of patients with LEMS, Congenital Myasthenic Syndromes, or CMS and Myasthenia Gravis (MG).

The chemical entity, amifampridine (3,4-diaminopyridine, or 3,4-DAP), has never been approved by the FDA for any indication. Because amifampridine phosphate (Firdapse<sup>®</sup>) has been granted Orphan Drug designation for the treatment of LEMS, CMS and MG by the FDA, the product is eligible to receive seven years of marketing exclusivity for either or all of these indications. Further, if we are the first pharmaceutical company to obtain approval for an amifampridine product, of which there can be no assurance, we will also be eligible to receive five years of marketing exclusivity with respect to the use of this product for any indication, running concurrently with the seven years of orphan marketing exclusivity described above (if both exclusivities are granted).

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We previously sponsored two multi-center, randomized, placebo-controlled Phase 3 trials evaluating Firdapse® for the treatment of LEMS, both of which were successful. On March 29, 2018, we reported that we had submitted an NDA to the FDA for Firdapse® for the symptomatic treatment of LEMS, and on May 29, 2018, we reported FDA acceptance of our NDA and Priority Review Status for Firdapse® for the symptomatic treatment of LEMS, with a Prescription Drug User Fee Act (PDUFA) goal date of November 28, 2018. There can be no assurance that our NDA for Firdapse® for LEMS will be approved by the FDA or the timing of any such approval.

We are currently conducting a Phase 3 clinical trial evaluating Firdapse® for the treatment of certain types of CMS. This trial, which will include approximately 23 adult and pediatric subjects, is being conducted at trial sites around the United States and Canada. We are also currently working to add one or more additional sites. Details of this trial are available on [www.clinicaltrials.gov](http://www.clinicaltrials.gov) (NCT02562066). Based on currently available information, we expect to report top-line results from this trial in the second half of 2019. There can be no assurance that any trial we conduct evaluating Firdapse® for the treatment of CMS will be successful or whether any NDA or NDA supplement that we may submit for Firdapse® for the treatment of CMS in the future will be filed by the FDA for review and approved.

We are currently conducting a Phase 3 clinical trial evaluating Firdapse® for the treatment of MuSK antibody positive myasthenia gravis (MuSK-MG) under a Special Protocol Assessment (SPA) with the FDA. The trial is a multi-site, international (U.S. and Italy), double-blind, placebo-controlled, clinical trial that is targeted to enroll approximately 60 subjects diagnosed with MuSK-MG. The trial will also enroll up to 10 generalized myasthenia gravis patients who will be assessed with the same clinical endpoints but achieving statistical significance in this subgroup of patients is not required and only summary statistics will be provided. We initiated this trial in January 2018 and are currently enrolling subjects. We currently expect to report top-line results from this trial in the second half of 2019. Details of this trial are available on [www.clinicaltrials.gov](http://www.clinicaltrials.gov) (NCT03304054).

We have begun conducting a Phase 3 proof-of-concept clinical study evaluating Firdapse® as a symptomatic treatment for patients with Spinal Muscular Atrophy (SMA) Type 3, ambulatory. The study is designed as a randomized (1:1), double-blind, 2-period, 2-treatment, crossover, outpatient proof-of-concept study to evaluate the safety, tolerability and potential efficacy of amifampridine in ambulatory patients diagnosed with SMA Type 3. The study is planned to include approximately 12 patients, and we currently expect to report top-line results from this study in the first half of 2020.

There can be no assurance that our currently ongoing trials evaluating Firdapse® for the treatment of CMS, MuSK-MG or SMA Type 3, or any trials we may undertake in the future to evaluate Firdapse® for the treatment of other rare, similar neuromuscular diseases, will be successful. Further, there can also be no assurance that the FDA will ever approve Firdapse® for any indication.

Now that our NDA for Firdapse® for the treatment of LEMS has been accepted for filing by the FDA, we are substantially increasing our activities to prepare for the potential marketing of Firdapse® in the United States as early as the beginning of 2019. During June 2018, we announced the appointment of Daniel J. Brennan as Chief Commercial Officer, and we are currently building our sales and marketing team to prepare for the launch of Firdapse® (assuming we obtain approval to commercialize the product), which will include our marketing and sales team, our patient education and support programs and our market access/reimbursement operations. We currently expect to launch and sell Firdapse®, and support Firdapse® patients and prescribers, through a field force of approximately 15-20 employees experienced in neurologic, central nervous system or rare diseases. At launch, the sales force will focus on the approximately 750 neuromuscular specialists who we believe most often treat neuromuscular diseases such as LEMS. We also expect to continue to work with several rare disease advocacy organizations to help increase awareness of LEMS, CMS and MuSK-MG and to provide education for the physicians who treat these rare diseases and the patients they treat, and we expect to have a field-based force of approximately 6

medical science liaisons who will help educate the medical communities and patients about these illnesses.

We currently intend to use a specialty pharmacy model to provide reimbursement, clinical and distribution support for Firdapse® and to offer cost-sharing and patient assistance programs to support qualified, commercially insured patients, and uninsured or under-insured patients. We may also donate money to independent charitable foundations dedicated to supporting LEMS patients as a means to potentially provide federal and state insured LEMS patients similar assistance. Our ultimate goal is to ensure that no LEMS patient is denied access to Firdapse® for financial reasons within existing legal restrictions.

Finally, if Firdapse® is approved for commercialization, we intend to take steps to seek approval for the product in Canada. We also intend as a longer term strategy to seek to develop a sustained release formulation for Firdapse®. There can be no assurance that we will be successful in these efforts.

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### **CPP-115**

We have been developing CPP-115, a GABA aminotransferase inhibitor that we licensed from Northwestern. Recently, we became aware that certain patents granted to Northwestern in 2018 (which patents have been licensed by Northwestern to a third-party) for a new GABA aminotransferase inhibitor were derived from CPP-115. As a result, it is our position that Northwestern has violated the license agreement based on its failure to transfer these new patent rights to us as part of the existing license agreement. It is also our position that Northwestern's publication of information about the new patents in violation of the license agreement has damaged us. On October 26, 2018, because good faith efforts to resolve these disputes had not been successful, we notified Northwestern that we were terminating the license agreement and seeking damages for Northwestern's breach of the license agreement. Further, on the same date, we filed a claim for damages in arbitration against Northwestern for Northwestern's breaches of the license agreement.

On November 5, 2018, Northwestern advised us that in its view, Northwestern has a right to terminate the license agreement with us because we allegedly breached the license agreement by failing to pay certain milestones and by allegedly failing to use commercially reasonable efforts to develop and commercialize any products. Northwestern has also advised us that, in its view, we have engaged in wrongful conduct and communications with the third party that licensed the new patents from Northwestern, and that such communications have damaged Northwestern's relationship with that third party. We dispute Northwestern's allegations and intend to vigorously defend ourselves against any claims that Northwestern may bring against us in the arbitration proceedings relating to these allegations.

This matter is at a very early stage and there can be no assurance as to the outcome of this matter.

### **Generic Sabril®**

In September 2015, we announced the initiation of a project to develop generic versions of Sabril® (vigabatrin). Sabril® is marketed by Lundbeck Inc. in the United States for the treatment of infantile spasms and refractory complex partial seizures. There can be no assurance that we will be successful in these efforts or that any abbreviated new drug applications (ANDAs) that we submit for vigabatrin will be accepted for review or approved.

We are also continuing our efforts to seek a partner to work with us in furthering the development of generic Sabril®. However, no agreements have been entered into to date.

There can be no assurance that we will ever successfully commercialize a generic version of Sabril®.

### **Available Capital Resources**

Based on forecasts of available cash, we believe that we have sufficient resources to support our currently anticipated operations through 2019 (without considering revenues and cash receipts that may be received in 2019 if we are successful in obtaining an approval for Firdapse® and launching the product in 2019, of which there can be no assurance). There can be no assurance that we will ever be in a position to commercialize any of our drug candidates or that we will obtain any additional funding that we may require in the future. 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 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 (our version of vigabatrin), CPP-115, and Firdapse<sup>®</sup>, and we expect this to continue for the foreseeable future.

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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 our 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 agreement, 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 consolidated 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. 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 currently incurring substantial costs to build our commercial team for a potential launch of Firdapse® in the first quarter of 2019. Such pre-commercialization costs are included in general and administrative expenses.

### *General and Administrative Expenses.*

Our general and administrative expenses consist primarily of salaries and personnel expenses for accounting, corporate, compliance and administrative functions. Other costs include administrative facility costs, regulatory fees, insurance, 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, 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 an equity financing that we completed in October 2011. In accordance with U.S. GAAP, we recorded the fair value of those warrants as a liability in the consolidated balance sheet using a Black-Scholes option-pricing model. We remeasured the fair value of this warrants liability at each reporting date until the warrants were exercised or until the unexercised warrants expired on May 2, 2017. Changes in the fair value of the warrants liability was reported in the consolidated statements of operations as income or expense. The fair value of the warrants liability was subject to significant fluctuation over the life of these warrants based on changes in the inputs to the Black-Scholes option-pricing model, including our common stock price, expected volatility, expected term, the risk-free interest rate and dividend yield.

### *Income Taxes.*

We have incurred operating losses since inception. Our net deferred tax asset has a 100% valuation allowance as of September 30, 2018 and December 31, 2017, 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 would 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 an audit. For tax positions meeting the more-likely-than-not threshold, the amount recognized in the financial statements



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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 consolidated financial statements included in this report.

### *Non-GAAP Financial Measures.*

We prepare our consolidated financial statements and footnotes thereto which accompany this report in accordance with U.S. GAAP (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 in our communications with investors. Non-GAAP measures are provided as additional information and not as an alternative to our consolidated financial statements presented in accordance with GAAP. Our non-GAAP financial measures are intended to enhance an overall understanding of our current financial performance. We believe that the non-GAAP financial measures that 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 have historically presented excludes from the calculation of net loss the expense (or the income) associated with the change in fair value of the liability-classified warrants. Further, we have historically reported non-GAAP net loss per share, which is calculated by dividing non-GAAP net loss by the weighted average common shares outstanding.

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 consolidated financial statements and notes thereto prepared in accordance with GAAP. Finally, the non-GAAP measures of net loss that 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 consolidated financial statements, which have been prepared in accordance with GAAP. The preparation of these consolidated 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 2017 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 2017 Annual Report on Form 10-K.

## **Results of Operations**

*Revenues.*

We had no revenues for the three and nine-month periods ended September 30, 2018 and 2017.

*Research and Development Expenses.*

Research and development expenses for the three and nine-month periods ended September 30, 2018 were \$4,538,369 and \$11,502,235, respectively, including stock-based compensation expense in each of the three and nine-month periods of \$240,122 and \$820,062, respectively. Research and development expenses for the three and nine-

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month periods ended September 30, 2017 were \$2,704,923 and \$7,970,603 respectively, including stock-based compensation expense in each of the three and nine-month periods of \$192,796 and \$622,700, respectively. Research and development expenses, in the aggregate, represented approximately 55% and 56% of total operating costs and expenses for the three and nine-month periods ended September 30, 2018 and 63% and 61% for the three and nine-month periods ended September 30, 2017, 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 nine months ended September 30, 2018, excluding stock-based compensation, increased compared to amounts expended in the same period in 2017. The increase in research and development expenses for the nine months ended September 30, 2018 when compared to the same period in 2017 is primarily due to increases in consulting expenses as we prepared to submit our NDA for Firdapse® during the first quarter of 2018, milestone expenses relating to the acceptance of our NDA submission in May 2018, expenses from our medical affairs program, and compensation and related personnel costs as we expand our headcount to support our currently ongoing trials and programs. We expect that research and development costs will continue to be substantial during the balance 2018 as we work towards completing trials evaluating Firdapse® for the treatment of CMS, MuSK-MG and SMA Type 3, continue our Expanded Access Program for Firdapse® and our other development programs, and prosecute our NDA submission for Firdapse® for LEMS.

*Selling and Marketing Expenses.*

We had no selling expenses for the three and nine-month periods ended September 30, 2018 and 2017.

During the fourth quarter of 2017, as we moved closer to submitting an NDA submission for Firdapse®, we re-started the development of our commercialization plans for Firdapse® and following the acceptance of our NDA for Firdapse® for the treatment of LEMS, we accelerated our efforts to build our commercial team for a potential launch of Firdapse® in the first quarter of 2019. Pre-commercialization costs are included in general and administrative expenses.

*General and Administrative Expenses.*

General and administrative expenses for the three and nine months ended September 30, 2018 were \$3,644,234 and \$8,949,663, respectively, including stock-based compensation expense in each of the three and nine-month periods ending September 30, 2018 of \$518,054 and \$1,700,961, respectively. General and administrative expenses for the three and nine months ended September 30, 2017 were \$1,601,785 and \$5,197,247, respectively, including stock-based compensation expense in each of the three and nine-month periods ending September 30, 2017 of \$336,942 and \$1,299,751, respectively. General and administrative expenses represented 45% and 44% of total operating costs and expenses for the three and nine months ended September 30, 2018 and 37% and 39% for the three and nine months ended September 30, 2017, respectively. The stock-based compensation is non-cash and relates to the expense of stock options awards and other non-cash stock compensation to certain employees. The increase in general and administrative expenses for the nine months ended September 30, 2018 when compared to the same period in 2017 is primarily due to increases in pre-commercialization expenses, headcount and corporate expenses as we build up our infrastructure and commercial programs in preparation for a potential Firdapse® launch in 2019. We expect that general and administrative costs, including pre-commercialization costs, will continue to increase in 2018 compared with the general and administrative costs incurred in 2017, as we continue to expand our operations in preparation for a potential launch of Firdapse® in early 2019.

As discussed above, pre-commercialization expenses are included in general and administrative expenses, and amounted to \$1,458,559 and \$2,921,758 respectively, for the three and nine-month periods ended September 30, 2018

and \$156,219 and \$563,508 respectively, for the three and nine-month periods ended September 30, 2017.

*Stock-Based Compensation.*

Total stock-based compensation for the three and nine-month periods ended September 30, 2018 were \$758,176 and \$2,521,023 and for the three and nine-month periods ended September 30, 2017 were \$529,738 and \$1,922,451, respectively. The increase in stock-based compensation for the nine-month periods ended September 30, 2018, when compared to the same period in 2017, is primarily due to the expense of options granted to employees and directors during the first quarter of 2018 in connection with 2017 year-end grants and the effect of grants to new

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employees as we increase our headcount to build up our infrastructure and commercial programs in preparation for a potential launch of Firdapse® in 2019.

### *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. As of May 2, 2017, all of the 2011 warrants were either exercised or had expired. During the period that the 2011 warrants were outstanding, the fair value of the warrants liability was 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 consolidated statements of operations.

No gain or loss was recognized for the three and nine months ended September 30, 2018, as all 2011 warrants that were not exercised expired on May 2, 2017. No gain or loss was recognized for the three months ended September 30, 2017. For the nine months ended September 30, 2017, we recognized a loss of \$186,904, due to the change in the fair value of the warrants liability. The loss during the nine months ended September 30, 2017 was principally a result of the increase of our stock price between December 31, 2016 and the warrants liability expiration date on May 2, 2017.

### *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 nine months ended September 30, 2018 when compared to the same period in 2017 is primarily due to higher yields on investments and higher invested balances. Other income, net, consists of interest income, dividend income, unrealized and realized gain (loss) on trading securities and realized gain (loss) on available-for-sale securities, if any. These proceeds are used to fund our drug 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 nine-month periods ended September 30, 2018 and 2017 we have applied a 100% valuation allowance against our deferred tax asset as we currently believe that it is more likely than not that the deferred tax asset will not be realized.

### *Net Loss.*

Our net loss was \$7,838,873 and \$19,503,905, respectively, for the three and nine months ended September 30, 2018 (\$0.08 and \$0.19, respectively, per basic and diluted share) as compared to a net loss of \$4,177,649 and \$13,024,679, respectively, for the three and nine months ended September 30, 2017 (\$0.05 and \$0.16, respectively, per basic and diluted share).

### *Non-GAAP Net Loss.*

Our non-GAAP net loss for the three and nine months ended September 30, 2018 was the same as our GAAP net loss, as there were no non-GAAP adjustments. Our non-GAAP net loss, which excludes for the three and nine months ended September 30, 2017 \$0 and a loss of \$186,904, respectively, associated with the change in the fair value of liability classified warrants, was \$4,177,649 and \$12,837,775, respectively, for the three and nine months ended September 30, 2017 (\$0.05 and \$0.15, 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 September 30, 2018, we had cash and investments aggregating \$66.7 million and working capital of \$59.0 million. At December 31, 2017, we had cash and investments aggregating \$84.0 million and working capital of \$80.9 million. At September 30, 2018, 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 continue our drug development programs and prepare for the commercialization of our drug candidates. We anticipate using current cash on hand to finance these activities.

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Based on forecasts of available cash, we believe that we have sufficient resources to support our currently anticipated operations through 2019 (without considering revenues and cash receipts that may be received in 2019 if we are successful in obtaining an approval for Firdapse® and launching the product in 2019, of which there can be no assurance). There can be no assurance that we will ever be in a position to commercialize any of our drug candidates or that we will obtain any additional funding that we may require in the future.

At the present time, we will require additional funding for future studies or trials, other than those described as being on-going in this report. We may also require additional working capital to support our operations beyond that time, depending on when and if we are able to launch Firdapse® and whether the results are cash flow positive. There can be no assurance as to the amount of any such funding that will be required for these purposes or whether any such funding will be available to us when it is required.

In that regard, our future funding requirements will depend on many factors, including:

the scope, rate of progress and cost of our clinical trials and other product development activities;

future clinical trial results;

the terms and timing of any collaborative, licensing and other arrangements that we may establish;

the cost and timing of regulatory approvals;

the cost and delays in product development as a result of any changes in regulatory oversight applicable to our products;

the cost and timing of establishing sales, marketing and distribution capabilities;

the effect of competition and market developments;

the cost of filing and potentially prosecuting, defending and enforcing any patent claims and other intellectual property rights; and

the extent to which we acquire or invest in other products.

We plan to raise additional funds that we may require in the future, through public or private equity offerings, debt financings, corporate collaborations or other means. We also may seek governmental grants for a portion of the required funding for our clinical trials and preclinical trials. We may also seek to raise capital to fund additional product development efforts or product acquisitions, even if we have sufficient funds for our planned operations. Any sale by us of additional equity or convertible debt securities could result in dilution to our stockholders. There can be

no assurance that any such required additional funding will be available to us at all or available on terms acceptable to us. Further, to the extent that we raise additional funds through collaborative arrangements, it may be necessary to relinquish some rights to our technologies or grant sublicenses on terms that are not favorable to us. If we are not able to secure additional funding when needed, we may have to delay, reduce the scope of or eliminate one or more research and development programs, which could have an adverse effect on our business.

On July 12, 2017, we filed a shelf registration statement with the SEC to sell up to \$150 million of common stock, preferred stock, warrants to purchase common stock, debt securities and units consisting of one or more of such securities (the 2017 Shelf Registration Statement ). The 2017 Shelf Registration Statement (file no. 333-219259) was declared effective by the SEC on July 26, 2017. We have completed one offering under the 2017 Shelf Registration Statement, raising net proceeds of approximately \$53.8 million from the sale of 16,428,572 shares of our common stock on November 28, 2017.

On December 23, 2016, we filed a shelf registration statement with the SEC to sell up to \$33.8 million of common stock (the 2016 Shelf Registration Statement ). This shelf registration statement was declared effective by the SEC on January 9, 2017. We have made no sales under the 2016 Shelf Registration Statement.

At September 30, 2018, the full amount of our 2016 Shelf Registration Statement and \$92.5 million of our 2017 Shelf Registration Statement remains available for future sales. However, if our public float (the market value



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of our common stock held by non-affiliate stockholders) were to fall below \$75 million, we would be subject to a further limitation under which we could sell no more than one-third (1/3) of our public float during any 12-month period. Further, the number of shares that we can sell at any one time may be limited under certain circumstances to 20% of the outstanding common stock under applicable NASDAQ marketplace rules.

*Cash Flows.*

Net cash used in operating activities was \$17,390,876 and \$9,712,705, respectively, for the nine-month periods ended September 30, 2018 and 2017. During the nine months ended September 30, 2018, net cash used in operating activities was primarily attributable to our net loss of \$19,503,905, decreases of \$604,591 in accounts payable, and \$185,812 in accrued expenses and other liabilities. This was partially offset by a \$356,924 decrease in prepaid expenses and other current assets and deposits, and \$2,546,508 of other non-cash expenses. During the nine months ended September 30, 2017, net cash used in operating activities was primarily attributable to our net loss of \$13,024,679. This was partially offset by a \$537,452 decrease in prepaid expenses and other current assets and deposits, a \$148,432 increase in accounts payable, a \$477,981 increase in accrued expenses and other liabilities, a \$186,904 non-cash change in fair value of warrants liability, and \$1,961,205 of other non-cash expenses.

Net cash used in investing activities was \$29,631,129 and \$64,748, respectively, for the nine-month periods ended September 30, 2018 and September 30, 2017. During the nine months ended September 30, 2018, net cash used in investing activities was primarily attributable to purchases of investments of \$37,141,968. This was partially offset by \$7,546,032 proceeds from maturities of investments. During the nine months ended September 30, 2017, net cash used in investing activities consisted of purchases of investments.

Net cash provided by financing activities during the nine-month period ended September 30, 2018 was \$141,616, consisting primarily of proceeds from the exercise of options to purchase common stock. Net cash provided by financing activities during the nine-month period ended September 30, 2017 was \$3,213,373, consisting primarily of the net proceeds from the exercise of warrants.

*Contractual Obligations.*

We have entered into the following contractual arrangements:

*Payments to BioMarin and others under our license agreement with BioMarin.* We have agreed to pay certain payments under our license agreement with BioMarin.

*Royalties:* We have agreed to pay (i) royalties to BioMarin 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; and (ii) royalties to the third-party licensor of the rights sublicensed to us 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.

*Milestone Payments:* Additionally, our license agreement with BioMarin requires that we pay certain milestone payments that BioMarin is obligated to pay to both a third-party licensor of the rights that have

been sublicensed to us and to the former stockholders of Huxley Pharmaceuticals ( Huxley ) under an earlier stock purchase agreement between BioMarin and the former Huxley stockholders.

With respect to the third party licensor of the rights that have been sublicensed to us, we have agreed to pay: (i) \$150,000 upon acceptance by the FDA of a filing of an NDA for Firdapse® for the treatment of LEMS (which amount was paid during the second quarter of 2018 after acceptance by the FDA of our NDA for Firdapse® for LEMS), and (ii) approximately \$3.0 million of which will be due upon the unconditional approval by the FDA of an NDA for Firdapse® for the treatment of LEMS (which milestone payment has not yet been earned).

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With respect to the former Huxley stockholders, we had agreed that we would pay the following milestone payments if either of the following milestones were satisfied before April 20, 2018: (i) \$2,425,000 upon acceptance by the FDA of a filing of an NDA for Firdapse<sup>®</sup> for the treatment of LEMS, and (ii) approximately \$4,200,000 of which was to be paid upon the unconditional approval by the FDA of an NDA for Firdapse<sup>®</sup> for the treatment of LEMS. Since neither of these milestones were met on or before April 20, 2018, these milestone payments were never earned. However, prior to April 20, 2018, we were advised that the former Huxley stockholders intended to take legal action against BioMarin and us seeking payment of the milestone payments due to them even if the milestones were achieved after their expiration date (April 20, 2018). While we disputed our obligation to pay either of the above described milestone payments if these milestones were achieved after April 20, 2018, in an agreement which was executed and became effective on July 26, 2018, we, BioMarin and the former Huxley stockholders agreed to amicably resolve this matter. As part of the settlement, and without admitting any liability, we paid the former Huxley stockholders a \$1.0 million milestone payment upon execution of the settlement agreement, and agreed to pay a \$1.0 million payment upon the unconditional approval by the FDA of an NDA for Firdapse<sup>®</sup> for the treatment of LEMS (which milestone payment has not yet been earned). Further, as part of the settlement agreement, we, BioMarin and the former Huxley stockholders entered into mutual releases regarding these matters.

*Cost Sharing Payments.* We have agreed to share in the cost of certain post-marketing studies conducted by BioMarin, and, as of September 30, 2018, we had paid BioMarin \$3.8 million related to expenses in connection with Firdapse<sup>®</sup> studies and trials.

*Payments to Northwestern University under our license agreement.* Under our license agreement with Northwestern, from which we derived our rights to CPP-115, through September 30, 2018, we had paid \$424,885 and had accrued liabilities of \$0 in the accompanying consolidated balance sheet. See [Overview-PPP-115](#) above for a description of the current status of our license agreement with Northwestern.

*Employment agreements.* We have entered into an employment agreement with our Chief Executive Officer that requires us to make base salary payments of approximately \$525,000 in 2018. The agreement expires in November 2020.

*Lease for office space.* We operate our business in leased office space in Coral Gables, Florida. We currently lease approximately 7,800 square feet of office space for which we pay annual rent of approximately \$330,000.

*Off-Balance Sheet Arrangements.*

We currently have no debt or capital leases. We have operating leases for our office facilities. We do not have any off-balance sheet arrangements as such term is defined in rules promulgated by the SEC.

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**Caution Concerning Forward-Looking Statements**

This Current Report on Form 10-Q contains forward-looking statements, as that term is defined in the Private Securities Litigation Reform Act of 1995. These include statements regarding our expectations, beliefs, plans or objectives for future operations and anticipated results of operations. For this purpose, any statements contained herein that are not statements of historical fact may be deemed to be forward-looking statements. Without limiting the foregoing, believes, anticipates, proposes, plans, expects, intends, may, and other similar expressions are intended to identify forward-looking statements. Such statements involve known and unknown risks, uncertainties and other factors that may cause our actual results, performance or achievements to be materially different from any future results, performance or achievements expressed or implied by such forward looking statements. The forward-looking statements made in this report are based on current expectations that involve numerous risks and uncertainties.

The successful development and commercialization of our current drug candidates 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:

our estimates regarding anticipated capital requirements and our need for additional financing;

the risk that another pharmaceutical company will receive an approval for its formulation of 3,4-DAP for the treatment of LEMS, CMS, or any other indication, before we do;

whether Firdapse® will be determined to be safe and effective and approved for commercialization for any indication;

whether the receipt of breakthrough therapy designation for Firdapse® for LEMS will affect the likelihood that the product will be found to be safe and effective;

whether as part of the FDA review of any NDA that we may submit for filing for Firdapse®, the tradename Firdapse®, which is the tradename used for the same product in Europe, will be approved for use for the product in the United States;

whether, assuming Firdapse® is approved for commercialization, we will be able to develop a sales and marketing organization that can successfully market Firdapse® while maintaining full compliance with applicable federal and state laws, rules and regulations;

whether our estimates of the size of the market for our drug candidates will turn out to be accurate;

the pricing of our products that we may be able to achieve if we are granted the ability to commercialize our drug candidates;

assuming Firdapse® is approved for commercialization, the timing of third party payor coverage and reimbursement for the product;

whether, even if Firdapse® is approved for commercialization, we will be successful in commercializing Firdapse®;

changes in the healthcare industry and the effect of political pressure from President Trump, Congress and medical professionals seeking to reduce prescription drug costs;

changes to the healthcare industry occasioned by any future repeal and replacement of the Affordable Care Act, in laws relating to the pricing of drug products, or changes in the healthcare industry generally;

the scope, rate of progress and expense of our clinical trials and studies, pre-clinical studies, proof-of-concept studies, and our other drug development activities, and whether our trials and studies will be successful;

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our ability to complete our trials and studies on a timely basis and within the budgets we establish for such trials and studies;

whether the trials that we are currently undertaking to evaluate Firdapse<sup>®</sup> for the treatment of CMS, MuSK-MG and SMA Type 3 or any other trials that we undertake in the future will be successful;

the result of our dispute with Northwestern regarding our license for CPP-115 and whether we will recover damages from Northwestern for their alleged breach of their license agreement with us;

whether we can successfully design and complete bioequivalence studies of our versions of vigabatrin compared to Sabril<sup>®</sup> that are acceptable to the FDA;

whether any ANDAs that we submit for a generic version of Sabril<sup>®</sup> will be accepted for filing and approved (and the timing of any such acceptances and approvals); and

the ability of our third-party suppliers and contract manufacturers to maintain compliance with current Good Manufacturing Practices (cGMP).

Our current plans and objectives are based on assumptions relating to the development of our current drug candidates. Although we believe that our assumptions are reasonable, any of our assumptions could prove inaccurate. In light of the significant uncertainties inherent in the forward-looking statements we have made herein, which reflect our views only as of the date of this report, you should not place undue reliance upon such statements. We undertake no obligation to update or revise publicly any forward-looking statements, whether as a result of new information, future events or otherwise.

## **ITEM 3. QUANTITATIVE AND QUALITATIVE DISCLOSURE ABOUT MARKET RISK**

Our market risks during the nine months ended September 30, 2018 have not materially changed from those discussed in Item 7A of our Annual Report on Form 10-K for the year ended December 31, 2017.

## **ITEM 4. CONTROLS AND PROCEDURES**

- a.** We have carried out an evaluation, under the supervision and with the participation of our management, including our principal executive officer and principal financial officer, of the effectiveness of the design and operation of our disclosure controls and procedures (as defined in Rules 13a-15(e) and 15d-15(e) of the Securities Exchange Act of 1934, as amended (the Exchange Act)). Based on such evaluation, our principal executive officer and principal financial officer have concluded that as of September 30, 2018, our disclosure controls and procedures were effective to ensure that the information required to be disclosed by us in the reports filed or submitted by us under the Exchange Act, was recorded, processed, summarized or reported within the time periods specified in the rules and regulations of the SEC, and include controls and procedures designed to ensure that information required to be disclosed by us in such reports was accumulated and communicated to management, including our principal executive officer and principal

financial officer, as appropriate to allow timely decisions regarding required disclosures.

- b.** During the three months ended September 30, 2018, there were no changes in our internal controls or in other factors that could have a material effect, or are reasonably likely to have a material effect, on our internal control over financial reporting.

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**PART II. OTHER INFORMATION**

**ITEM 1. LEGAL PROCEEDINGS**

The Company is not a party to any material legal proceedings.

**ITEM 1A. RISK FACTORS**

There are many factors that affect our business, our financial condition, and the results of our operations. In addition to the information set forth in this quarterly report, you should carefully read and consider Item 1A. Risk Factors in Part I, and Item 7. Management's Discussion and Analysis of Financial Condition and Results of Operations in Part II, of our 2017 Annual Report on Form 10-K filed with the SEC, which contain a description of significant factors that might cause our actual results of operations in future periods to differ materially from those currently expected or desired.

**ITEM 2. UNREGISTERED SALES OF EQUITY SECURITIES AND USE OF PROCEEDS**

None.

**ITEM 3. DEFAULTS UPON SENIOR SECURITIES**

None.

**ITEM 4. MINE SAFETY DISCLOSURE**

Not applicable.

**ITEM 5. OTHER INFORMATION**

None.

**ITEM 6. EXHIBITS**

- 10.1 Settlement Agreement, effective as of July 26, 2018, among the Company, BioMarin Pharmaceutical Inc. and Aceras BioMedical LLC, in its capacity as stockholder representative for the former stockholders of Huxley Pharmaceuticals, Inc.
- 10.2 Fourth Amendment to Lease, dated effective as of August 13, 2018, between the Company and PRII 355 Alhambra Circle LLC (portions of this exhibit have been omitted and filed separately with the SEC



pursuant to a request for confidential treatment)

31.1	<u>Certification of Principal Executive Officer under Section 302 of the Sarbanes-Oxley Act of 2002</u>
31.2	<u>Certification of Principal Financial Officer under Section 302 of the Sarbanes-Oxley Act of 2002</u>
32.1	<u>Certification of Principal Executive Officer under Section 906 of the Sarbanes-Oxley Act of 2002</u>
32.2	<u>Certification of Principal Financial Officer under Section 906 of the Sarbanes-Oxley Act of 2002</u>
101.INS	XBRL Instance Document
101.SCH	XBRL Taxonomy Extension Schema
101.CAL	XBRL Taxonomy Extension Calculation Linkbase
101.DEF	XBRL Taxonomy Extension Definition Linkbase
101.LAB	XBRL Taxonomy Extension Label Linkbase
101.PRE	XBRL Taxonomy Extension Presentation Linkbase

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**SIGNATURES**

Pursuant to the Securities Exchange Act of 1934, the Registrant has caused this report to be signed on its behalf by the undersigned, thereunto duly authorized.

**Catalyst Pharmaceuticals, Inc.**

By: /s/ Alicia Grande  
Alicia Grande  
Vice President, Treasurer and Chief Financial  
Officer

Date: November 7, 2018