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**UNITED STATES  
SECURITIES AND EXCHANGE COMMISSION**  
Washington, D.C. 20549

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**FORM 10-Q**

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[Mark One]

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

For the Quarterly Period Ended March 31, 2018

OR

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

Commission File No. 001-33057

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**CATALYST PHARMACEUTICALS, INC.**

(Exact name of registrant as specified in its charter)

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**Delaware**  
(State or other jurisdiction of  
incorporation or organization)

**76-0837053**  
(IRS Employer  
Identification No.)

**355 Alhambra Circle**  
**Suite 1250**  
**Coral Gables, Florida**  
(Address of principal executive offices)

**33134**  
(Zip Code)

**Registrant's telephone number, including area code: (305) 420-3200**

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

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

Indicate by check mark whether the registrant is a large accelerated filer, an accelerated filer, a non-accelerated filer, 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 (Check one):

|                         |  |                           |                                     |
|-------------------------|--|---------------------------|-------------------------------------|
| Large accelerated filer | <input type="checkbox"/>   | Accelerated Filer         | <input checked="" type="checkbox"/> |
| Non-accelerated filer   | <input type="checkbox"/> (Do not check if a smaller reporting company) | Smaller reporting company | <input type="checkbox"/>            |
|                         |  | Emerging growth company   | <input type="checkbox"/>            |

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 102,596,164 shares of common stock, \$0.001 par value per share, were outstanding as of May 4, 2018.

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CATALYST PHARMACEUTICALS, INC.

INDEX

**PART I. FINANCIAL INFORMATION**

|         |   |    |
|---------|---|----|
| Item 1. | <a href="#">FINANCIAL STATEMENTS</a>  |    |
|         | <a href="#">Consolidated balance sheets at March 31, 2018 (unaudited) and December 31, 2017</a>   | 3  |
|         | <a href="#">Consolidated statements of operations and comprehensive loss for the three months ended March 31, 2018 and 2017 (unaudited)</a> | 4  |
|         | <a href="#">Consolidated statement of stockholders' equity for the three months ended March 31, 2018 (unaudited)</a>                        | 5  |
|         | <a href="#">Consolidated statements of cash flows for the three months ended March 31, 2018 and 2017 (unaudited)</a>                        | 6  |
|         | <a href="#">Notes to unaudited consolidated financial statements</a>  | 7  |
| Item 2. | <a href="#">MANAGEMENT'S DISCUSSION AND ANALYSIS OF FINANCIAL CONDITION AND RESULTS OF OPERATIONS</a>                                       | 17 |
| Item 3. | <a href="#">QUANTITATIVE AND QUALITATIVE DISCLOSURE ABOUT MARKET RISK</a>   | 29 |
| Item 4. | <a href="#">CONTROLS AND PROCEDURES</a>   | 29 |

**PART II. OTHER INFORMATION**

|          |   |    |
|----------|---|----|
| Item 1.  | <a href="#">LEGAL PROCEEDINGS</a>   | 30 |
| Item 1A. | <a href="#">RISK FACTORS</a>  | 30 |
| Item 2.  | <a href="#">UNREGISTERED SALES OF EQUITY SECURITIES AND USE OF PROCEEDS</a> | 30 |
| Item 3.  | <a href="#">DEFAULTS UPON SENIOR SECURITIES</a>                             | 30 |
| Item 4.  | <a href="#">MINE SAFETY DISCLOSURE</a>                                      | 30 |
| Item 5.  | <a href="#">OTHER INFORMATION</a>   | 30 |
| Item 6.  | <a href="#">EXHIBITS</a>  | 30 |
|          | <a href="#">SIGNATURES</a>  | 32 |

**CATALYST PHARMACEUTICALS, INC.**  
**CONSOLIDATED BALANCE SHEETS**

|  | <u>March 31,</u><br><u>2018</u> | <u>December 31,</u><br><u>2017</u> |
|--|---------------------------------|------------------------------------|
| <b>ASSETS</b>  |                                 |                                    |
| Current Assets:  |                                 |                                    |
| Cash and cash equivalents  | \$ 19,600,591                   | \$ 57,496,702                      |
| Short-term investments   | 58,342,578                      | 26,516,711                         |
| Prepaid expenses and other current assets  | 992,828                         | 1,173,744                          |
| Total current assets   | 78,935,997                      | 85,187,157                         |
| Property and equipment, net  | 183,370                         | 191,385                            |
| Deposits   | 8,888                           | 8,888                              |
| Total assets   | <u>\$ 79,128,255</u>            | <u>\$ 85,387,430</u>               |
| <b>LIABILITIES AND STOCKHOLDERS' EQUITY</b>  |                                 |                                    |
| Current Liabilities:   |                                 |                                    |
| Accounts payable   | \$ 805,023                      | \$ 1,945,575                       |
| Accrued expenses and other liabilities   | 1,926,371                       | 2,320,587                          |
| Total current liabilities  | 2,731,394                       | 4,266,162                          |
| Accrued expenses and other liabilities, non-current  | 150,395                         | 157,456                            |
| Total liabilities  | 2,881,789                       | 4,423,618                          |
| Commitments and contingencies  |                                 |                                    |
| Stockholders' equity:  |                                 |                                    |
| Preferred stock, \$0.001 par value, 5,000,000 shares authorized: none issued and outstanding at March 31, 2018 and December 31, 2017   | —                               | —                                  |
| Common stock, \$0.001 par value, 150,000,000 shares authorized; 102,586,164 shares and 102,549,498 shares issued and outstanding at March 31, 2018 and December 31, 2017, respectively | 102,586                         | 102,549                            |
| Additional paid-in capital   | 208,426,045                     | 207,421,710                        |
| Accumulated deficit  | (132,260,339)                   | (126,560,447)                      |
| Accumulated other comprehensive loss   | (21,826)                        | —                                  |
| Total stockholders' equity   | 76,246,466                      | 80,963,812                         |
| Total liabilities and stockholders' equity   | <u>\$ 79,128,255</u>            | <u>\$ 85,387,430</u>               |

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

**CATALYST PHARMACEUTICALS, INC.**  
**CONSOLIDATED STATEMENTS OF OPERATIONS AND COMPREHENSIVE LOSS (unaudited)**

|   | For the Three Months Ended |                       |
|---|----------------------------|-----------------------|
|   | March 31,                  |                       |
|   | 2018                       | 2017                  |
| Operating costs and expenses:                           |                            |                       |
| Research and development                                | \$ 3,259,042               | \$ 2,813,929          |
| General and administrative                              | 2,674,398                  | 1,865,942             |
| Total operating costs and expenses                      | <u>5,933,440</u>           | <u>4,679,871</u>      |
| Loss from operations                                    | (5,933,440)                | (4,679,871)           |
| Other income, net                                       | 233,548                    | 109,977               |
| Change in fair value of warrants liability              | —                          | (397,235)             |
| Loss before income taxes                                | (5,699,892)                | (4,967,129)           |
| Provision for income taxes                              | —                          | —                     |
| Net loss  | <u>\$ (5,699,892)</u>      | <u>\$ (4,967,129)</u> |
| Net loss per share – basic and diluted                  | <u>\$ (0.06)</u>           | <u>\$ (0.06)</u>      |
| Weighted average shares outstanding – basic and diluted | <u>102,557,350</u>         | <u>82,972,316</u>     |
| Other comprehensive loss:                               |                            |                       |
| Unrealized gain (loss) on available-for-sale securities | (21,826)                   | —                     |
| Comprehensive loss                                      | <u>\$ (5,721,718)</u>      | <u>\$ (4,967,129)</u> |

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

**CATALYST PHARMACEUTICALS, INC.**  
**CONSOLIDATED STATEMENT OF STOCKHOLDERS' EQUITY (unaudited)**  
**For the three months ended March 31, 2018**

|  | Preferred<br>Stock | Common<br>Stock  | Additional<br>Paid-in<br>Capital | Accumulated<br>Deficit | Accumulated<br>Other<br>Comprehensive<br>Loss | Total               |
|--|--------------------|------------------|----------------------------------|------------------------|---|---------------------|
| <b>Balance at December 31, 2017</b>        | \$ —               | \$102,549        | \$207,421,710                    | \$(126,560,447)        | \$ —  | \$80,963,812        |
| Issuance of stock options for services     | —                  | —                | 971,340                          | —                      | —   | 971,340             |
| Exercise of stock options for common stock | —                  | 37               | 32,995                           | —                      | —   | 33,032              |
| Other comprehensive loss                   | —                  | —                | —                                | —                      | (21,826)                                      | (21,826)            |
| Net loss                                   | —                  | —                | —                                | (5,699,892)            | —   | (5,699,892)         |
| <b>Balance at March 31, 2018</b>           | <u>\$ —</u>        | <u>\$102,586</u> | <u>\$208,426,045</u>             | <u>\$(132,260,339)</u> | <u>\$ (21,826)</u>                            | <u>\$76,246,466</u> |

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

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

|   | For the Three Months Ended |                     |
|---|----------------------------|---------------------|
|   | March 31,                  |                     |
|   | 2018                       | 2017                |
| <b>Operating Activities:</b>  |                            |                     |
| Net loss  | \$ (5,699,892)             | \$ (4,967,129)      |
| Adjustments to reconcile net loss to net cash used in operating activities: |                            |                     |
| Depreciation  | 8,015                      | 12,958              |
| Stock-based compensation  | 971,340                    | 754,144             |
| Change in fair value of warrants liability                                  | —                          | 397,235             |
| (Increase) decrease in:   |                            |                     |
| Prepaid expenses and other current assets and deposits                      | 180,916                    | 118,082             |
| Increase (decrease) in:   |                            |                     |
| Accounts payable  | (1,140,552)                | (15,248)            |
| Accrued expenses and other liabilities                                      | (401,277)                  | (178,917)           |
| Net cash used in operating activities                                       | (6,081,450)                | (3,878,875)         |
| <b>Investing Activities:</b>  |                            |                     |
| Purchase of investments   | (31,847,693)               | (33,145)            |
| Net cash provided by (used in) investing activities                         | (31,847,693)               | (33,145)            |
| <b>Financing Activities:</b>  |                            |                     |
| Proceeds from exercise of stock options                                     | 33,032                     | —                   |
| Net cash provided by (used in) financing activities                         | 33,032                     | —                   |
| Net increase (decrease) in cash and cash equivalents                        | (37,896,111)               | (3,912,020)         |
| Cash and cash equivalents - beginning of period                             | 57,496,702                 | 13,893,064          |
| Cash and cash equivalents - end of period                                   | <u>\$ 19,600,591</u>       | <u>\$ 9,981,044</u> |
| <b>Non-cash investing activities:</b>                                       |                            |                     |
| Unrealized loss on available-for-sale securities                            | \$ 21,826                  | \$ —                |

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

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), MuSK antibody positive myasthenia gravis, and infantile spasms.

Since inception, the Company has devoted substantially all of its efforts to business planning, research and development, recruiting management and technical staff, acquiring operating assets and raising capital. The Company's primary focus is on the development and commercialization of its drug candidates. The Company has incurred operating losses in each period from inception through March 31, 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 three months ended March 31, 2018 are not necessarily indicative of the results to be expected for any future period or for the full 2018 fiscal year.

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. **SHORT-TERM INVESTMENTS.** The Company invests in short-term high credit-quality funds in order to obtain higher yields on its cash available for investments. At March 31, 2018 short-term investments consisted of a short-term bond fund and U.S. Treasuries. At December 31, 2017 short-term 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 March 31, 2018 and December 31, 2017, the only investment classified as trading securities was the short-term bond fund. Unrealized gain (loss) for the three months ended March 31, 2018 and 2017 for trading securities was (\$58,861) and \$29,430, respectively, 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 from the date of purchase as short-term investments. At March 31, 2018, all U.S. Treasuries were due within one year. 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 gain 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 statement of operations. The Company recognizes a charge when the declines in the fair value below the amortized cost basis of our 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.

- 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.



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 March 31, 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.

|                                   | <b>Fair Value Measurements at Reporting Date Using</b> |  |   |  |
|-----------------------------------|--|--|---|--|
|                                   | Balances as of<br>March 31,<br>2018                    | Quoted Prices in<br>Active Markets<br>for Identical<br>Assets/Liabilities<br>(Level 1) | Significant<br>Other<br>Observable<br>Inputs<br>(Level 2) | Significant<br>Unobservable<br>Inputs<br>(Level 3) |
| <i>Cash and cash equivalents:</i> |  |  |   |  |
| Money market funds                | \$19,343,497   | \$ 19,343,497  | \$ —  | \$ —   |
| <i>Short-term investments:</i>    |  |  |   |  |
| Short-term bond fund              | \$26,496,992   | \$ 26,496,992  | \$ —  | \$ —   |
| U.S. Treasuries                   | \$31,845,586   | \$ —   | \$31,845,586  | \$ —   |
|                                   | Balances as of<br>December 31,<br>2017                 | Quoted Prices in<br>Active Markets<br>for Identical<br>Assets/Liabilities<br>(Level 1) | Significant<br>Other<br>Observable<br>Inputs<br>(Level 2) | Significant<br>Unobservable<br>Inputs<br>(Level 3) |
| <i>Cash and cash equivalents:</i> |  |  |   |  |
| Money market funds                | \$56,820,688   | \$ 56,820,688  | \$ —  | \$ —   |
| <i>Short-term investments:</i>    |  |  |   |  |
| Short-term bond fund              | \$26,516,711   | \$ 26,516,711  | \$ —  | \$ —   |

**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 periods 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. At March 31, 2017, 763,913 of the 2011 warrants remained outstanding. All unexercised 2011 warrants expired on May 2, 2017. See Note 3.

j. **STOCK-BASED COMPENSATION.** The Company recognizes expense in the consolidated statement of operations for the fair value of all stock-based payments to employees, directors 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 three years. Forfeitures are recognized as a reduction of share-based compensation expense as they occur.

As of March 31, 2018, there were outstanding stock options to purchase 6,902,500 shares of common stock, of which stock options to purchase 3,616,662 shares of common stock were exercisable as of March 31, 2018.

For the three-month periods ended March 31, 2018 and 2017, the Company recorded stock-based compensation expense as follows:

|                                | <u>Three months ended March 31,</u> |                   |
|--------------------------------|-------------------------------------|-------------------|
|                                | <u>2018</u>                         | <u>2017</u>       |
| Research and development       | \$ 294,315                          | \$ 206,352        |
| General and administrative     | 677,025                             | 547,792           |
| Total stock-based compensation | <u>\$ 971,340</u>                   | <u>\$ 754,144</u> |

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 Statement of Operations and Comprehensive Loss as of March 31, 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).

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

- i. NET LOSS PER SHARE.** Basic loss per share is computed by dividing net loss for the period by the weighted average number of common shares outstanding during the period. The calculation of basic and diluted net loss per share is the same for all periods presented, as the effect of potential common stock equivalents is anti-dilutive due to the Company's net loss position for all periods presented. The potential shares, which are excluded from the determination of basic and diluted net loss per share as their effect is anti-dilutive, are as follows:

|  | March 31,        |                  |
|--|------------------|------------------|
|  | 2018             | 2017             |
| Options to purchase common stock           | 6,902,500        | 6,088,333        |
| Warrants to purchase common stock          | —                | 2,407,663        |
| Unvested restricted stock                  | —                | 26,667           |
| Potential equivalent common stock excluded | <u>6,902,500</u> | <u>8,522,663</u> |

Potentially dilutive options to purchase common stock as of March 31, 2018 have exercise prices ranging from \$0.79 to \$4.64. Potentially dilutive options to purchase common stock as of March 31, 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 is currently evaluating the impact this accounting standard will have on its 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.

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

### 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 warrants 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. All unexercised 2011 warrants expired on May 2, 2017.

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

|                          | <b>March 31, 2017</b> |
|--------------------------|-----------------------|
| Risk free interest rate  | 1.03%                 |
| Expected term            | 0.09 years            |
| Expected volatility      | 110%                  |
| Expected dividend yield  | 0%                    |
| Expected forfeiture rate | 0%                    |

The following table rolls forward the fair value of the Company's warrants liability activity for the three-month period ended March 31, 2017:

|                                 | <b>Three months ended<br/>March 31, 2017</b> |
|---------------------------------|--|
| Fair value, beginning of period | \$ 122,226                                   |
| Issuance of warrants            | —  |
| Exercise of warrants            | —  |
| Change in fair value            | 397,235                                      |
| Fair value, end of period       | <u>\$ 519,461</u>                            |

On May 2, 2017, the outstanding and unexercised 2011 warrants expired. During the three months ended March 31, 2017, none of the 2011 warrants were exercised.

**4. Short-term Investments.**

Short-term investments by security type were as follows:

|                              | Amortized<br>cost   | Gross<br>Unrealized<br>Gains | Gross<br>Unrealized<br>Losses | Estimated<br>Fair Value |
|------------------------------|---------------------|------------------------------|-------------------------------|-------------------------|
| <b>At March 31, 2018:</b>    |                     |                              |                               |                         |
| Short-term bond fund         | \$26,555,853        | \$ —                         | \$ (58,861)                   | \$26,496,992            |
| U.S. Treasuries              | 31,867,412          | —                            | (21,826)                      | 31,845,586              |
| Total                        | <u>\$58,423,265</u> | <u>\$ —</u>                  | <u>\$ (80,687)</u>            | <u>\$58,342,578</u>     |
| <b>At December 31, 2017:</b> |                     |                              |                               |                         |
| Short-term bond fund         | <u>\$26,487,281</u> | <u>\$ 29,430</u>             | <u>\$ —</u>                   | <u>\$26,516,711</u>     |

The Company has classified its U.S. Treasuries as available-for-sale securities and, as such, they are reported at fair value with unrealized gain 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 months ended March 31, 2018 or 2017.

Certain U.S. Treasuries at March 31, 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 its 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 March 31, 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 March 31, 2018 was less than 3 months.

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

Prepaid expenses and other current assets consist of the following:

|   | March 31, 2018    | December 31, 2017   |
|---|-------------------|---------------------|
| Prepaid research fees                           | \$ 249,094        | \$ 388,977          |
| Prepaid insurance                               | 501,181           | 638,139             |
| Prepaid pre-commercialization fees              | 76,900            | 65,000              |
| Prepaid subscription fees                       | 92,916            | 23,347              |
| Prepaid rent                                    | 20,981            | —                   |
| Other   | 51,756            | 58,281              |
| Total prepaid expenses and other current assets | <u>\$ 992,828</u> | <u>\$ 1,173,744</u> |

**6. Property and Equipment, net.**

Property and equipment, net consists of the following:

|                                   | March 31, 2018    | December 31, 2017 |
|-----------------------------------|-------------------|-------------------|
| Computer equipment                | \$ 27,915         | \$ 27,915         |
| Furniture and equipment           | 169,931           | 169,931           |
| Leasehold improvements            | 152,708           | 152,708           |
|                                   | 350,554           | 350,554           |
| Less: Accumulated depreciation    | (167,184)         | (159,169)         |
| Total property and equipment, net | <u>\$ 183,370</u> | <u>\$ 191,385</u> |

Depreciation expense was \$8,015 and \$12,958, respectively, for the three-month periods ended March 31, 2018 and 2017 respectively.

## 7. Accrued Expenses and Other Liabilities.

Accrued expenses and other liabilities consist of the following:

|  | <u>March 31, 2018</u> | <u>December 31, 2017</u> |
|--|-----------------------|--------------------------|
| Accrued preclinical and clinical trial expenses    | \$ 990,080            | \$ 970,649               |
| Accrued professional fees                          | 558,260               | 227,457                  |
| Accrued compensation and benefits                  | 54,548                | 821,935                  |
| Accrued license fees                               | 278,750               | 252,500                  |
| Deferred rent and lease incentive                  | 25,597                | 24,011                   |
| Other  | 19,136                | 24,035                   |
| Current accrued expenses and other liabilities     | <u>1,926,371</u>      | <u>2,320,587</u>         |
| Deferred rent and lease incentive - non-current    | 150,395               | 157,456                  |
| Non-current accrued expenses and other liabilities | <u>150,395</u>        | <u>157,456</u>           |
| Total accrued expenses and other liabilities       | <u>\$ 2,076,766</u>   | <u>\$ 2,478,043</u>      |

## 8. Commitments and Contingencies.

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

Under the license agreement with Northwestern, the Company is responsible for continued research and development of any resulting product candidates. As of March 31, 2018, the Company has paid \$424,885 in connection with the license and has accrued license fees of \$278,750 in the accompanying March 31, 2018 consolidated balance sheet for expenses, maintenance fees and milestones. In addition, the Company is obligated to pay certain milestone payments in future years relating to clinical development activities with respect to CPP-115, and royalties on any products resulting from the license agreement, if the Company does not cancel the license agreement. The next milestone payment of \$300,000 is due on the earlier of successful completion of the first Phase 3 clinical trial for CPP-115 or August 27, 2018.

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

- c. **LICENSE AGREEMENT WITH BIOMARIN.** 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.

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

Under the Company's license agreement with BioMarin, the Company has agreed 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. These milestones aggregate (i) approximately \$2.6 million due upon acceptance by the FDA of a filing of an NDA for Firdapse® for the treatment of LEMS or CMS (approximately \$150,000 of which will be due to the third party licensor and approximately \$2,425,000 of which will be due to the former Huxley stockholders), and (ii) approximately \$7.2 million due upon the unconditional approval by the FDA of an NDA for Firdapse® for the treatment of LEMS (approximately \$3.0 million of which will be due to the third party licensor and approximately \$4.2 million of which will be due to the former Huxley stockholders). However, under BioMarin's agreement with the former Huxley stockholders (and under the Company's license agreement with BioMarin), BioMarin's obligation to pay the milestone payments due to the former Huxley stockholders (and the Company's corresponding obligation to pay such milestone payments) expired because the milestones were not satisfied by April 20, 2018.

BioMarin has recently advised the Company that the former Huxley stockholders may take legal action seeking payment of the milestone payments due to them from BioMarin if these milestones are achieved after April 20, 2018, notwithstanding the express termination date in the agreements. BioMarin has also advised the Company that the Company could become involved in any such legal action. While it is too early to determine how this matter will affect the Company, based on currently available information the Company does not believe that this matter will have a material adverse effect on the Company's financial position or results of operations.

The Company also agreed to share in the cost of certain post-marketing studies being conducted by BioMarin, and, as of March 31, 2018, the Company had paid BioMarin \$3.8 million related to expenses in connection with Firdapse® studies and trials.

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

**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 2014. 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 March 31, 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. The Company has to date conducted the following sales of its securities under the 2017 Shelf Registration Statement:

- (a) 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. 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.

As of March 31, 2018, there is approximately \$92.5 million available for future sale under the 2017 Shelf Registration Statement.

**11. Stock Compensation.**

*Stock Options*

During the three-month periods ended March 31, 2018 and 2017, the Company granted seven-year term options to purchase an aggregate of 1,772,500 and 1,520,000 shares, respectively, of the Company's common stock to employees and directors. Option awards generally vest over a period of 1 to 3 years of continuous service. The Company recorded stock-based compensation related to stock options totaling \$971,340 and \$735,536 respectively, during the three-month periods ended March 31, 2018 and 2017. During the three-month periods ended March 31, 2018 and 2017, respectively, 744,998 and 255,000 options vested.

During the three months ended March 31, 2018, options to purchase 36,666 shares of the Company's common stock were exercised, with proceeds of \$33,032 to the Company. No options were exercised during the three months ended March 31, 2017.

As of March 31, 2018, there was approximately \$4,810,000 of unrecognized compensation expense related to non-vested stock option awards granted under the 2006 and 2014 Stock Incentive Plans. The cost is expected to be recognized over a weighted average period of approximately 2.34 years.

*Restricted Stock Units*

No restricted stock units were granted during the three months ended March 31, 2018 and 2017. The Company recorded stock-based compensation related to restricted stock units totaling \$0 and \$18,608, during the three-month periods ended March 31, 2018 and 2017. All restricted stock units were vested as of December 31, 2017.



## 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 first 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 months ended March 31, 2018 as compared to the same period ended March 31, 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 three drug candidates in development:

- Firdapse®

In October 2012, we licensed the North American rights to Firdapse®, a proprietary form of amifampridine phosphate, or chemically known as 3,4-diaminopyridine phosphate, from BioMarin Pharmaceutical Inc. (BioMarin). In August 2013, we were granted "breakthrough therapy designation" by the U.S. Food & Drug Administration (FDA) for Firdapse® for the treatment of patients with Lambert-Eaton Myasthenic Syndrome, or LEMS, a rare and sometimes fatal autoimmune disease characterized by muscle weakness. Further, the FDA has granted Orphan Drug Designation for Firdapse® 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®) has been granted Orphan Drug designation for the treatment of LEMS, CMS and MG by the FDA, the product is also 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 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).

We previously sponsored a multi-center, randomized, placebo-controlled Phase 3 trial evaluating Firdapse® for the treatment of LEMS. This Phase 3 trial, which involved 38 subjects, was designed as a randomized “withdrawal” trial in which all patients were treated with Firdapse® during a 7 to 91-day run-in-period followed by treatment with either Firdapse® or placebo over a two-week randomization period. The co-primary endpoints for this Phase 3 trial were the comparison of changes in patients randomized to continue Firdapse® versus those who transitioned to placebo that occurred in both the quantitative myasthenia gravis score (QMG), which measures muscle strength, and subject global impression score (SGI), on which the subjects rate their global impression of the effects of a study treatment during the two-week randomization period. In September 2014, we reported positive top-line results from this Phase 3 trial, and the successful results of this study were published in 2016 in *Muscle & Nerve* (Muscle Nerve, 2016, 53(5):717-725).

During 2014, we established an expanded access program (EAP) to make Firdapse® available to any patients diagnosed with LEMS, CMS, or Downbeat Nystagmus in the United States, who meet the inclusion and exclusion criteria, with Firdapse® being provided to patients for free until sometime after new drug application (NDA) approval, should we receive such approval (of which there can be no assurance). We continue to inform neuromuscular physicians as to the availability of the Firdapse® EAP and also to work with various rare disease advocacy organizations to inform patients and physicians about the program.

On December 17, 2015, we announced completion of the submission of an NDA for Firdapse® for the treatment of LEMS and CMS. However, on February 17, 2016, we announced that we had received a “refusal to file” (RTF) letter from the FDA regarding our NDA submission. In early April 2016, we met with the FDA to obtain greater clarity regarding what would be required by the FDA to accept the Firdapse® NDA for filing. Following the receipt of the formal minutes of that meeting, on April 26, 2016, we issued a press release reporting that the FDA had advised us that in addition to the results of our previously submitted multi-center, randomized, placebo-controlled Phase 3 trial, we would need to submit positive results from a second adequate and well-controlled study in patients with LEMS. Additionally, there was a requirement for us to perform several abuse liability studies for Firdapse®.

In October 2016, we announced that we had reached an agreement with the FDA under a Special Protocol Assessment (SPA) for the protocol design, clinical endpoints, and statistical analysis approach to be taken in our second Phase 3 study evaluating Firdapse® (amifampridine phosphate) for the symptomatic treatment of LEMS. A SPA is a process by which sponsors ask the FDA to evaluate the protocol of a proposed clinical trial to determine whether it adequately addresses scientific and regulatory requirements for the purpose identified by the sponsor. A SPA agreement indicates FDA concurrence with the adequacy and acceptability of specific critical elements of protocol design, endpoints and analysis. Additionally, it provides a binding agreement with FDA’s review division that a pivotal trial design, conduct, and planned analysis adequately addresses the scientific and regulatory objectives in support of a regulatory submission for drug approval. However, the FDA may rescind a SPA agreement when the division director determines that a substantial scientific issue essential to determining the safety or efficacy of the product has been identified after the trial has begun.

Our second Phase 3 trial evaluating Firdapse® for the treatment of LEMS (designated as LMS-003) was conducted at sites in Miami, Florida and Los Angeles, California. This double-blind, placebo-controlled withdrawal trial had the same co-primary endpoints as our first Phase 3 trial evaluating Firdapse® for the treatment of LEMS. Further, the FDA allowed us to enroll patients from our expanded access program as study subjects in this second trial. Enrollment in this trial, which included 26 subjects, was completed in October 2017. Details of the Phase 3 clinical trial are available on [www.clinicaltrials.gov](http://www.clinicaltrials.gov) (NCT02970162).

On November 27, 2017, we reported positive top-line results from the LMS-003 trial. This trial had two prospectively defined co-primary endpoints. The first of these, quantitative myasthenia gravis score (QMG), achieved a statistically significant p-value of 0.0004, and the second, subject global impression (SGI), achieved a statistically significant p-value of 0.0003. More importantly, a clinically significant difference of 6.4 points was observed between the Firdapse® and placebo groups for the QMG endpoint. Firdapse® was well tolerated and showed a similar safety profile to that seen in earlier studies. All p-values reported are based on the entire intent to treat (ITT) population of patients that enrolled in this trial.

The prospectively defined secondary endpoint for the physician's clinical global impression of improvement (CGI-I) achieved statistical significance (p-value 0.0020). Further, the exploratory endpoints of triple timed up and go (3TUG, p-value 0.0112) and the evaluation of the QMG-Limb domains endpoint (p-value 0.0010) were also statistically significant. The exploratory endpoint of most bothersome symptom (MBS) (p-value 0.0572) was not significant, but showed a trend.

We were also required to conduct three pre-clinical abuse liability studies under the FDA guidance for "Assessment of Abuse Potential of Drugs" that was finalized in January 2017 (Self-Administration, Physical Dependence and Drug Discrimination). All three studies have been completed, and top-line results indicate that amifampridine phosphate does not exhibit abuse potential in these assessment models.

On February 12, 2018, after receipt of the minutes of our recently held Type C meeting with the FDA, we issued a press release reporting on the results of the meeting. Prior to the meeting, we had provided the FDA with our preliminary data package for our proposed NDA resubmission, including the positive top-line results from our LMS-003 trial, as well as the FDA-required abuse liability studies that we recently completed demonstrating that Firdapse® does not have abuse liability potential. The minutes of the meeting reflect the FDA's advice to us that our proposed filing package will be sufficient for resubmission of an NDA for Firdapse®.

On March 29, 2018, we reported that we had resubmitted an NDA to the FDA for Firdapse® for the symptomatic treatment of LEMS. There can be no assurance that our resubmitted NDA will be accepted for filing or approved.

Our original NDA submission for Firdapse® included data and information (including data from a currently ongoing investigator treatment IND) providing evidence supporting the benefits of Firdapse® for treating certain types of CMS, and requested that CMS be included in our initial label for Firdapse®. To provide additional support for our submission of an NDA for Firdapse® for the treatment of CMS, in October 2015 we initiated a small blinded clinical trial at four academic centers of up to 10 subjects in the pediatric CMS population, ages 2 to 17. However, after considering comments from the FDA, we determined to enroll both adult and pediatric subjects with CMS in this trial and to expand the number of subjects to be evaluated in the trial to an aggregate of approximately 20 subjects. We are currently conducting this study at trial sites around the United States and Canada, and we are currently working to add one or more additional sites outside the United States. Details of this trial are available on [www.clinicaltrials.gov](http://www.clinicaltrials.gov) (NCT02562066).

Based on currently available information, we expect to complete enrollment in this trial before the end of 2018 and to report top-line results from this trial in the first quarter of 2019. There can be no assurance that any trial we perform for 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 considered adding to the proposed label for our recently resubmitted NDA for Firdapse® those limited types of congenital myasthenic syndromes (CMS) that are considered mechanistically similar to LEMS. However, after discussion with the FDA at the Type C meeting, and further consideration, we elected not to complicate the review of our NDA submission for Firdapse® for the treatment of LEMS with this second indication. Once our currently ongoing CMS clinical trial is completed, and assuming the trial is successful, we plan to seek to add CMS to our label for Firdapse® for a much broader population of CMS patients.

In February 2016, we announced the initiation of an investigator-sponsored, randomized, double-blind, placebo-controlled, crossover Phase 2/3 clinical trial evaluating the safety, tolerability and potential efficacy of Firdapse® as a symptomatic treatment for patients with MuSK-MG. MuSK-MG is a particularly severe form of myasthenia gravis that affects about 3,000 to 4,800 patients in the U.S., for which there are no approved effective therapies and is therefore an unmet medical need. Seven patients participated in this proof-of-concept trial. We provided study drug, placebo and financial support for this study.

On March 15, 2017, we reported top-line results from this trial. Both of the co-primary efficacy endpoints of change from baseline (CFB) in total Quantitative Myasthenia Gravis (QMG) score (p=0.0003) and CFB in total Myasthenia Gravis Activities of Daily Living (MG-ADL) score (p=0.0006) were statistically and clinically significant in this trial. Several secondary efficacy measures also achieved statistical significance. Amifampridine phosphate was well tolerated in this population of patients.

## [Table of Contents](#)

On August 30, 2017, we announced that we had reached an agreement with the FDA on a SPA for the protocol design, clinical endpoints, and statistical analysis approach to be taken in our proposed Phase 3 registration trial evaluating the safety and efficacy of amifampridine phosphate treatment in patients with MuSK-MG. The protocol that the FDA has reviewed is for 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 employ a primary endpoint of Myasthenia Gravis Activities of Daily Living (MG-ADL) and a secondary endpoint of Quantitative Myasthenia Gravis Score (QMG). At the FDA's request, 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 recently began enrolling subjects. We anticipate that it will take about 12 months to complete the enrollment for the trial and we expect to report top-line results from this trial in the first half of 2019. Details of this trial are available on [www.clinicaltrials.gov](http://www.clinicaltrials.gov) (NCT03304054).

On November 21, 2017, we announced the initiation of a company-sponsored, proof-of-concept clinical trial evaluating safety, tolerability and efficacy of Firdapse® as a symptomatic treatment for patients with Spinal Muscular Atrophy (SMA) Type 3. The study is being conducted by a team of researchers led by Lorenzo Maggi, MD, and Giovanni Baranello, MD, of the Fondazione Istituto Neurologico Carlo Besta in Milan, Italy, a major referral center for SMA patients. 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 anticipate reporting top-line results from the study in the second half of 2019.

There can be no assurance that any trial that we initiate to evaluate Firdapse® for MuSK-MG or SMA Type 3 will be successful. Further, there can also be no assurance that the FDA will ever approve Firdapse® for these indications.

Finally, we may seek to evaluate Firdapse® for the treatment of other rare, similar neuromuscular diseases, although we have not yet begun to develop clinical programs for these other indications, and all such programs are subject to the availability of funding. There can be no assurance that Firdapse® will be an effective treatment for any other rare, similar neuromuscular diseases.

Prior to the receipt of the RTF letter, we had actively been taking steps to prepare for the commercialization of Firdapse® in the United States. However, in light of the receipt of the RTF letter, in the first quarter of 2016 we put most of our commercialization activities on hold in order to conserve cash. During the fourth quarter of 2017, as we moved closer to the filing of an NDA for Firdapse® for LEMS, we recommenced the development of our commercialization plans for Firdapse®. We are also continuing to work with several rare disease advocacy organizations to help increase awareness of LEMS, CMS and MUSK-MG and to provide awareness and outreach support for the physicians who treat these rare diseases and the patients they treat.

### CPP-115

We are developing CPP-115, a GABA aminotransferase inhibitor that, based on our preclinical studies to date, we believe is a more potent form of vigabatrin, and may have fewer side effects (e.g., visual field defects) than those associated with vigabatrin. We are hoping to develop CPP-115 for the treatment of refractory infantile spasms and possibly for the treatment of adult refractory patients with Tourette's Disorder. CPP-115 has been granted Orphan Drug Designation by the FDA for the treatment of infantile spasms and Orphan Medicinal Product Designation in the European Union, or E.U., for West syndrome (a form of infantile spasms).

We are currently refining our development plans for this product. We are also working with one or more potential investigators who have expressed an interest in evaluating our product for particular indications (particularly infantile spasms).

We are also continuing our efforts to seek a partner to work with us in furthering the development of CPP-115. However, no agreements have been entered into to date.

## [Table of Contents](#)

There can be no assurance that we will ever successfully commercialize CPP-115.

- **Generic Sabril®**

In September 2015, we announced the initiation of a project to develop generic versions of Sabril® (vigabatrin) in two dosage forms: tablets and powder sachets. Sabril® is marketed by Lundbeck Inc. in the United States in both dosage forms 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®, and we expect this to continue for the foreseeable future.

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.

## [Table of Contents](#)

### *Selling and Marketing Expenses.*

We do not currently have any selling or marketing expenses. We had been incurring costs tied to our future sales and marketing efforts for Firdapse®. However, during the first quarter of 2016, following the receipt of the RTF letter, we put most of these activities on hold in order to conserve cash. In the fourth quarter of 2017, we recommenced the developing of our commercialization plans for Firdapse® as we moved closer to the submission of an NDA for Firdapse®. 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 were 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 March 31, 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 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.

## [Table of Contents](#)

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-month periods ended March 31, 2018 and 2017.

#### *Research and Development Expenses.*

Research and development expenses for the three-month periods ended March 31, 2018 and 2017 were \$3,259,042 and \$2,813,929, respectively, including stock-based compensation expense in each of the three-month periods of \$294,315 and \$206,352, respectively. Research and development expenses, in the aggregate, represented approximately 55% and 60% of total operating costs and expenses for the three-month periods ended March 31, 2018 and 2017. 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 three months ended March 31, 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 three months ended March 31, 2018 when compared to the same period in 2017 is primarily due to an increase in consulting expenses as we prepared to submit our NDA for Firdapse® during March 2018, offset by decreases in clinical expenses, including related drug costs. We expect that research and development costs will continue to be substantial during the balance 2018 as we complete trials evaluating Firdapse® for the treatment of CMS, MuSK-MG and SMA Type 3, continue our Expanded Access Program for Firdapse®, continue our other development programs and prosecute our recently submitted NDA for Firdapse® for LEMS, assuming it is filed by the FDA.



## [Table of Contents](#)

### *Selling and Marketing Expenses.*

We had no selling expenses for the three-month periods ended March 31, 2018 and 2017. In 2016, we had been incurring costs tied to our future sales and marketing efforts for Firdapse®. However, during the first quarter of 2016, following the receipt of the RTF letter, we put most of these activities on hold in order to conserve cash. During the fourth quarter of 2017, as we moved closer to an NDA submission for Firdapse®, we restarted the development of our commercialization plans for Firdapse®. Pre-commercialization costs are included in general and administrative expenses.

### *General and Administrative Expenses.*

General and administrative expenses for the three months ended March 31, 2018 and 2017 were \$2,674,398 and \$1,865,942, respectively, including stock-based compensation expense in each of the three-month periods ending March 31, 2018 and 2017 of \$677,025 and \$547,792, respectively. General and administrative expenses represented 45% and 40% of total operating costs and expenses for the three months ended March 31, 2018 and 2017, respectively. The increase in general and administrative expenses for the three months ended March 31, 2018 when compared to the same period in 2017 is primarily due to increase in pre-commercialization expenses. 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 expand our operations and headcount to build up our infrastructure and commercial programs in preparation for a potential Firdapse® launch in 2019.

As discussed above, pre-commercialization expenses are included in general and administrative expenses, and amounted to \$630,991 and \$166,205 respectively, for the three months ended March 31, 2018 and 2017.

### *Stock-Based Compensation.*

Total stock-based compensation for the three-month periods ended March 31, 2018 and 2017 were \$971,340 and \$754,144, respectively. The increase in stock-based compensation for the three-month period ended March 31, 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.

### *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. The fair value of the portion of these warrants which remain outstanding was recorded in the liability section of the consolidated balance sheet and was estimated at \$519,461 at March 31, 2017. The fair value of the warrants liability is determined at the end of each reporting period with the resulting gains or losses recorded as the change in fair value of warrants liability in the consolidated statements of operations.

No gain or loss was recognized for the three months ended March 31, 2018, as all 2011 warrants that were not exercised expired on May 2, 2017. For the three months ended March 31, 2017, we recognized a loss of \$397,235, due to the change in the fair value of the warrants liability. The loss during the three months ended March 31, 2017 was principally a result of the increase of our stock price between December 31, 2016 and March 31, 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 three months ended March 31, 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 and unrealized and realized gain (loss) on trading securities. 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-month periods ended March 31, 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.



## [Table of Contents](#)

### *Net Loss.*

Our net loss was \$5,699,892 and \$4,967,129, respectively, for the three months ended March 31, 2018 and 2017 (\$0.06 and \$0.06, respectively, per basic and diluted share).

### *Non-GAAP Net Loss.*

Our non-GAAP net loss, which excludes for the three months ended March 31, 2018 and 2017 \$0 and a loss of \$397,235, respectively, associated with the change in the fair value of liability classified warrants, was \$5,699,892 and \$4,569,894 (\$0.06 and \$0.06 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 March 31, 2018, we had cash and cash equivalents and short-term investments aggregating \$77.9 million and working capital of \$76.2 million. At December 31, 2017, we had cash and cash equivalents and short-term investments aggregating \$84.0 million and working capital of \$80.9 million. At March 31, 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. It will likely be some time before we obtain the necessary regulatory approvals to commercialize one or more of our product candidates in the United States.

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.

## [Table of Contents](#)

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 \$150,000,000 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:

- On November 28, 2017, we raised net proceeds of approximately \$53.8 million from the sale of 16,428,572 shares of our common stock.

On December 23, 2016, we filed a shelf registration statement with the SEC to sell up to \$33,842,512 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 March 31, 2018, the full amount of our 2016 Shelf Registration Statement and \$92,499,998 of our 2017 Shelf Registration Statement remains available for future sales. However, if our public float (the market value 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 \$6,081,450 and \$3,878,875, respectively, for the three-month periods ended March 31, 2018 and 2017. During the three months ended March 31, 2018, net cash used in operating activities was primarily attributable to our net loss of \$5,699,892, and decreases of \$1,140,552 in accounts payable, and \$401,277 in accrued expenses and other liabilities. This was partially offset by a \$180,916 decrease in prepaid expenses and other current assets and deposits, and \$979,355 of other non-cash expenses. During the three months ended March 31, 2017, net cash used in operating activities was primarily attributable to our net loss of \$4,967,129 and decreases of \$15,248 in accounts payable and \$178,917 in accrued expenses and other liabilities. This was partially offset by a \$118,082 decrease in prepaid expenses and other current assets and deposits, \$397,235 of non-cash change in fair value of warrants liability and \$767,102 of other non-cash expenses. Such additional non-cash expenses consist of depreciation and stock-based compensation expense.

Net cash used in investing activities was \$31,847,693 and \$33,145, respectively, for the three-month periods ended March 31, 2018 and March 31, 2017, consisting primarily of purchases of short-term investments.

Net cash provided by financing activities during the three-month period ended March 31, 2018 was \$33,032, consisting of proceeds from the exercise of options to purchase common stock. No cash was used in or provided by financing activities during the three-month period ended March 31, 2017.

### *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 to our license agreement with BioMarin.

## Table of Contents

- *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:* Under our license agreement with BioMarin, we have agreed 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 us and to the former stockholders of Huxley Pharmaceuticals (“Huxley”) under an earlier stock purchase agreement between BioMarin and the former Huxley stockholders. These milestones aggregate (i) approximately \$2.6 million due upon acceptance by the FDA of a filing of an NDA for Firdapse® for the treatment of LEMS or CMS (approximately \$150,000 of which will be due to the third party licensor and approximately \$2,425,000 of which will be due to the former Huxley stockholders), and (ii) approximately \$7.2 million due upon the unconditional approval by the FDA of an NDA for Firdapse® for the treatment of LEMS (approximately \$3.0 million of which will be due to the third party licensor and approximately \$4.2 million of which will be due to the former Huxley stockholders). However, under BioMarin’s agreement with the former Huxley stockholders (and under our license agreement with BioMarin), BioMarin’s obligation to pay the milestone payments due to the former Huxley stockholders (and our corresponding obligation to pay such milestone payments) expired because these milestones were not satisfied by April 20, 2018.

BioMarin has recently advised us that the former Huxley stockholders may take legal action seeking payment of the milestone payments due to them from BioMarin if these milestones are achieved after April 20, 2018, notwithstanding the express termination date in the agreements. BioMarin has also advised us that we could become involved in any such legal action. While it is too early to determine how this matter will affect us, based on currently available information we do not believe that this matter will have a material adverse effect on our financial position or results of operations.

- *Cost Sharing Payments.* We have agreed to share in the cost of certain post-marketing studies conducted by BioMarin, and, as of March 31, 2018, we had paid BioMarin \$3.8 million related to expenses in connection with Firdapse® studies and trials.
- *Payments to Northwestern University under our license agreement.* Under our license agreement with Northwestern, we have paid to date \$424,885, had accrued liabilities of \$278,750, at March 31, 2018 in the accompanying consolidated balance sheet, and owe certain milestone payments in future years if we do not cancel the license agreement. The next milestone payment of \$300,000 is due on the earlier of successful completion of the first Phase 3 clinical trial of CPP-115 or August 27, 2018.
- *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 2018.
- *Lease for office space.* We operate our business in leased office space in Coral Gables, Florida. We currently lease approximately 5,200 square feet of office space for which we pay annual rent of approximately \$200,000.

### *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.

### 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-diaminopyridine (3,4-DAP) for the treatment of Lambert-Eaton Myasthenic Syndrome (LEMS), Congenital Myasthenic Syndromes (CMS), or any other indication, before we do;
- whether the NDA that we recently submitted for Firdapse® will be accepted for filing by the FDA, and, if accepted, whether it will be granted a priority review;
- whether, even if the FDA accepts an NDA submission for Firdapse®, such product will be determined to be safe and effective and approved for commercialization for any of the submitted indications;
- whether the receipt of breakthrough therapy designation for Firdapse® for LEMS will result in an expedited review of Firdapse® by the FDA or 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 or contract with a sales and marketing organization that can successfully market Firdapse® while maintaining full compliance with applicable federal and state laws, rules and regulations;
- whether the trials that we are undertaking to evaluate Firdapse® for the treatment of CMS, MuSK-MG and SMA Type 3 or any other trials that we undertake in the future will be successful;
- whether CPP-115 will be determined to be safe for humans;
- whether CPP-115 will be determined to be effective for the treatment of infantile spasms, or possibly Tourette’s Disorder;
- whether we can successfully design and complete bioequivalence studies of our versions of vigabatrin compared to Sabril® that are acceptable to the FDA;
- whether any ANDA that we submit for a generic version of Sabril® will be accepted by the FDA for review and approved (and the timing of any such approval);
- 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;

## Table of Contents

- our ability to complete our trials and studies on a timely basis and within the budgets we establish for such trials and studies;
- the scope, rate of progress and expense of our clinical trials and studies, pre-clinical studies, proof-of-concept studies, and our other development activities;
- the ability of our third-party suppliers and contract manufacturers to maintain compliance with cGMP;
- 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; and
- changes in 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;

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 three months ended March 31, 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 March 31, 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 March 31, 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.

**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**

|         |  |
|---------|--|
| 31.1    | Certification of Principal Executive Officer under Section 302 of the Sarbanes-Oxley Act of 2002 |
| 31.2    | Certification of Principal Financial Officer under Section 302 of the Sarbanes-Oxley Act of 2002 |
| 32.1    | Certification of Principal Executive Officer under Section 906 of the Sarbanes-Oxley Act of 2002 |
| 32.2    | Certification of Principal Financial Officer under Section 906 of the Sarbanes-Oxley Act of 2002 |
| 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  |

**Exhibit Index**

| <u>Exhibit<br/>Number</u> | <u>Description</u>   |
|---------------------------|--|
| 31.1                      | <a href="#">Certification of Principal Executive Officer under Section 302 of the Sarbanes-Oxley Act of 2002</a> |
| 31.2                      | <a href="#">Certification of Principal Financial Officer under Section 302 of the Sarbanes-Oxley Act of 2002</a> |
| 32.1                      | <a href="#">Certification of Principal Executive Officer under Section 906 of the Sarbanes-Oxley Act of 2002</a> |
| 32.2                      | <a href="#">Certification of Principal Financial Officer under Section 906 of the Sarbanes-Oxley Act of 2002</a> |
| 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: May 9, 2018



## Certification of Principal Executive Officer

I, Patrick J. McEnany, certify that:

1. I have reviewed this quarterly report on Form 10-Q of Catalyst Pharmaceuticals, Inc.;
2. Based on my knowledge, this quarterly report does not contain any untrue statement of a material fact or omit to state a material fact necessary to make the statements made, in light of the circumstances under which such statements were made, not misleading with respect to the period covered by this report;
3. Based on my knowledge, the financial statements, and other financial information included in this report, fairly present in all material respects the financial condition, results of operations and cash flows of the registrant as of, and for, the periods presented in this report;
4. The registrant's other certifying officer and I are responsible for establishing and maintaining disclosure controls and procedures (as defined in Exchange Act Rules 13a-15(e) and 15d-15(e)) and internal control over financial reporting (as defined in Exchange Act Rules 13a-15(f) and 15(d)-15(f)) for the registrant and have:
  - a) Designed such disclosure controls and procedures, or caused such disclosure controls and procedures to be designed under our supervision, to ensure that material information relating to the registrant, including its consolidated subsidiary, is made known to us by others within those entities, particularly during the period in which this report is being prepared;
  - b) Designed such internal control over financial reporting, or caused such internal control over financial reporting to be designed under our supervision, to provide reasonable assurance regarding the reliability of financial reporting and the preparation of financial statements for external purposes in accordance with generally accepted accounting principles;
  - c) Evaluated the effectiveness of the registrant's disclosure controls and procedures and presented in this report our conclusions about the effectiveness of the disclosure controls and procedures, as of the end of the period covered by this report based on such evaluation; and
  - d) Disclosed in this report any change in the registrant's internal control over financial reporting that occurred during the registrant's most recent fiscal quarter that has materially affected, or is reasonably likely to materially affect, the registrant's internal control over financial reporting; and
5. The registrant's other certifying officer and I have disclosed, based on our most recent evaluation of internal control over financial reporting, to the registrant's auditors and the audit committee of registrant's board of directors (or persons performing the equivalent functions):
  - a) all significant deficiencies and material weaknesses in the design or operation of internal control over financial reporting which are reasonably likely to adversely affect the registrant's ability to record, process, summarize and report financial information; and
  - b) any fraud, whether or not material, that involves management or other employees who have a significant role in the registrant's internal control over financial reporting.

Date: May 9, 2018

/s/ Patrick J. McEnany

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Patrick J. McEnany  
Chief Executive Officer  
(Principal Executive Officer)

**Certification of Principal Financial Officer**

I, Alicia Grande, certify that:

1. I have reviewed this quarterly report on Form 10-Q of Catalyst Pharmaceuticals, Inc.;
2. Based on my knowledge, this quarterly report does not contain any untrue statement of a material fact or omit to state a material fact necessary to make the statements made, in light of the circumstances under which such statements were made, not misleading with respect to the period covered by this report;
3. Based on my knowledge, the financial statements, and other financial information included in this report, fairly present in all material respects the financial condition, results of operations and cash flows of the registrant as of, and for, the periods presented in this report;
4. The registrant's other certifying officer and I are responsible for establishing and maintaining disclosure controls and procedures (as defined in Exchange Act Rules 13a-15(e) and 15d-15(e)) and internal control over financial reporting (as defined in Exchange Act Rules 13a-15(f) and 15(d)-15(f)) for the registrant and have:
  - a) Designed such disclosure controls and procedures, or caused such disclosure controls and procedures to be designed under our supervision, to ensure that material information relating to the registrant, including its consolidated subsidiary, is made known to us by others within those entities, particularly during the period in which this report is being prepared;
  - b) Designed such internal control over financial reporting, or caused such internal control over financial reporting to be designed under our supervision, to provide reasonable assurance regarding the reliability of financial reporting and the preparation of financial statements for external purposes in accordance with generally accepted accounting principles;
  - c) Evaluated the effectiveness of the registrant's disclosure controls and procedures and presented in this report our conclusions about the effectiveness of the disclosure controls and procedures, as of the end of the period covered by this report based on such evaluation; and
  - d) Disclosed in this report any change in the registrant's internal control over financial reporting that occurred during the registrant's most recent fiscal quarter that has materially affected, or is reasonably likely to materially affect, the registrant's internal control over financial reporting; and
5. The registrant's other certifying officer and I have disclosed, based on our most recent evaluation of internal control over financial reporting, to the registrant's auditors and the audit committee of registrant's board of directors (or persons performing the equivalent functions):
  - a) all significant deficiencies and material weaknesses in the design or operation of internal control over financial reporting which are reasonably likely to adversely affect the registrant's ability to record, process, summarize and report financial information; and
  - b) any fraud, whether or not material, that involves management or other employees who have a significant role in the registrant's internal control over financial reporting.

Date: May 9, 2018

/s/ Alicia Grande

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Alicia Grande  
Chief Financial Officer  
(Principal Financial Officer)

**Certification Required by 18 U.S.C. Section 1350  
(as adopted pursuant to Section 906 of the Sarbanes-Oxley Act of 2002)**

I, Patrick J. McEnany as Principal Executive Officer of Catalyst Pharmaceuticals, Inc. (the "Company"), certify, pursuant to 18 U.S.C. Section 1350 (as adopted pursuant to Section 906 of the Sarbanes-Oxley Act of 2002), that to my knowledge:

1. the accompanying Quarterly Report on Form 10-Q of the Company for the quarterly period ended March 31, 2018 (the "Report"), filed with the U.S. Securities and Exchange Commission, fully complies with the requirements of Section 13(a) or 15(d) of the Securities Exchange Act of 1934, as amended; and
2. the information contained in the Report fairly presents, in all material respects, the financial condition and results of operations of the Company.

Date: May 9, 2018

/s/ Patrick J. McEnany

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Patrick J. McEnany  
Chief Executive Officer  
(Principal Executive Officer)

**Certification Required by 18 U.S.C. Section 1350  
(as adopted pursuant to Section 906 of the Sarbanes-Oxley Act of 2002)**

I, Alicia Grande as Principal Financial Officer of Catalyst Pharmaceuticals, Inc. (the "Company"), certify, pursuant to 18 U.S.C. Section 1350 (as adopted pursuant to Section 906 of the Sarbanes-Oxley Act of 2002), that to my knowledge:

1. the accompanying Quarterly Report on Form 10-Q of the Company for the quarterly period ended March 31, 2018 (the "Report"), filed with the U.S. Securities and Exchange Commission, fully complies with the requirements of Section 13(a) or 15(d) of the Securities Exchange Act of 1934, as amended; and
2. the information contained in the Report fairly presents, in all material respects, the financial condition and results of operations of the Company.

Date: May 9, 2018

/s/ Alicia Grande

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Alicia Grande  
Chief Financial Officer  
(Principal Financial Officer)