
**UNITED STATES
SECURITIES AND EXCHANGE COMMISSION
WASHINGTON, DC 20549**

FORM 10-Q

(Mark One)

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

FOR THE QUARTERLY PERIOD ENDED SEPTEMBER 30, 2017

OR

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

FOR THE TRANSITION PERIOD FROM TO

Commission file number: 001-36003

CONATUS PHARMACEUTICALS INC.

(Exact name of registrant as specified in its charter)

Delaware
(State or Other Jurisdiction of
Incorporation or Organization)

16745 W. Bernardo Dr., Suite 200
San Diego, CA
(Address of Principal Executive Offices)

20-3183915
(I.R.S. Employer
Identification No.)

92127
(Zip Code)

(858) 376-2600

(Registrant's Telephone Number, Including Area Code)

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

Indicate by check mark whether the registrant has submitted electronically and posted on its corporate website, if any, every Interactive Data File required to be submitted and posted pursuant to Rule 405 of Regulation S-T (§ 232.405 of this chapter) during the preceding 12 months (or for such shorter period that the registrant was required to submit and post such files). Yes 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 the definitions of "large accelerated filer," "accelerated filer," "smaller reporting company," and "emerging growth company" in Rule 12b-2 of the Exchange Act.

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

As of October 30, 2017, the registrant had 30,005,462 shares of common stock (\$0.0001 par value) outstanding.

CONATUS PHARMACEUTICALS INC.

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PART I. FINANCIAL INFORMATION

ITEM 1. FINANCIAL STATEMENTS

Conatus Pharmaceuticals Inc.

Condensed Balance Sheets
(Unaudited)

| | September 30, 2017 | December 31, 2016 |
|--|-----------------------|----------------------|
| Assets | | |
| Current assets: | | |
| Cash and cash equivalents | \$ 14,360,452 | \$ 58,083,409 |
| Marketable securities | 70,823,435 | 18,931,715 |
| Other receivables | — | 2,500,000 |
| Prepaid and other current assets | 1,339,208 | 937,436 |
| Total current assets | 86,523,095 | 80,452,560 |
| Property and equipment, net | 204,914 | 261,446 |
| Other assets | 2,538,211 | 1,609,834 |
| Total assets | <u>\$ 89,266,220</u> | <u>\$ 82,323,840</u> |
| Liabilities and stockholders' equity | | |
| Current liabilities: | | |
| Accounts payable and accrued expenses | \$ 10,937,851 | \$ 5,311,093 |
| Accrued compensation | 1,810,919 | 2,351,703 |
| Current portion of deferred revenue | 19,454,795 | 30,897,192 |
| Note payable | — | 1,000,000 |
| Total current liabilities | 32,203,565 | 39,559,988 |
| Deferred revenue, less current portion | 12,673,762 | 20,803,762 |
| Convertible note payable | 12,968,493 | — |
| Deferred rent | 139,392 | 171,544 |
| Stockholders' equity: | | |
| Preferred stock, \$0.0001 par value; 10,000,000 shares authorized; no shares issued and outstanding | — | — |
| Common stock, \$0.0001 par value; 200,000,000 shares authorized; 30,005,462 shares issued and outstanding at September 30, 2017; 26,118,722 shares issued and outstanding at December 31, 2016 | 3,001 | 2,612 |
| Additional paid-in capital | 194,951,562 | 172,424,531 |
| Accumulated other comprehensive loss | (23,093) | (6,145) |
| Accumulated deficit | (163,650,462) | (150,632,452) |
| Total stockholders' equity | 31,281,008 | 21,788,546 |
| Total liabilities and stockholders' equity | <u>\$ 89,266,220</u> | <u>\$ 82,323,840</u> |

See accompanying notes to condensed financial statements.

Conatus Pharmaceuticals Inc.

**Condensed Statements of Operations and Comprehensive Loss
(Unaudited)**

| | Three Months Ended September 30, | | Nine Months Ended September 30, | |
|---|----------------------------------|-----------------------|---------------------------------|------------------------|
| | 2017 | 2016 | 2017 | 2016 |
| Revenues: | | | | |
| Collaboration revenue | \$ 9,565,890 | \$ — | \$ 26,572,397 | \$ — |
| Total revenues | 9,565,890 | — | 26,572,397 | — |
| Operating expenses: | | | | |
| Research and development | 11,165,150 | 4,825,421 | 32,308,786 | 13,770,371 |
| General and administrative | 2,449,382 | 2,069,447 | 7,406,591 | 6,883,708 |
| Total operating expenses | 13,614,532 | 6,894,868 | 39,715,377 | 20,654,079 |
| Loss from operations | (4,048,642) | (6,894,868) | (13,142,980) | (20,654,079) |
| Other income (expense): | | | | |
| Interest income | 258,623 | 33,640 | 648,103 | 94,995 |
| Interest expense | (189,041) | (17,500) | (473,354) | (52,500) |
| Other (expense) income | (21,318) | 11,818 | (71,779) | 1,645 |
| Total other income | 48,264 | 27,958 | 102,970 | 44,140 |
| Net loss | (4,000,378) | (6,866,910) | (13,040,010) | (20,609,939) |
| Other comprehensive income (loss): | | | | |
| Net unrealized (losses) gains on marketable securities | (455) | (9,999) | (16,948) | 12,885 |
| Comprehensive loss | \$ (4,000,833) | \$ (6,876,909) | \$ (13,056,958) | \$ (20,597,054) |
| Net loss per share, basic and diluted | \$ (0.13) | \$ (0.31) | \$ (0.46) | \$ (0.96) |
| Weighted average shares outstanding used in computing net loss per share, basic and diluted | 30,004,037 | 22,410,702 | 28,104,199 | 21,527,993 |

See accompanying notes to condensed financial statements.

Conatus Pharmaceuticals Inc.
Condensed Statements of Cash Flows
(Unaudited)

| | Nine Months Ended September 30, | |
|---|--|----------------------|
| | 2017 | 2016 |
| Operating activities | | |
| Net loss | \$ (13,040,010) | \$ (20,609,939) |
| Adjustments to reconcile net loss to net cash used in operating activities: | | |
| Depreciation | 81,023 | 79,877 |
| Stock-based compensation expense | 3,049,181 | 2,456,196 |
| Amortization of premiums and discounts on marketable securities, net | (13,260) | 6,323 |
| Accrued interest included in convertible note payable | 468,493 | — |
| Changes in operating assets and liabilities: | | |
| Other receivables | 2,500,000 | — |
| Prepaid and other current assets | (458,038) | 844,647 |
| Other assets | (872,111) | — |
| Accounts payable and accrued expenses | 5,617,193 | (97,396) |
| Accrued compensation | (540,784) | 175,197 |
| Deferred revenue | (19,572,397) | — |
| Deferred rent | (22,587) | (13,313) |
| Net cash used in operating activities | (22,803,297) | (17,158,408) |
| Investing activities | | |
| Maturities of marketable securities | 57,257,000 | 30,847,000 |
| Purchase of marketable securities | (109,152,408) | (24,958,424) |
| Capital expenditures | (24,491) | (107,165) |
| Net cash (used in) provided by investing activities | (51,919,899) | 5,781,411 |
| Financing activities | | |
| Proceeds from issuance of convertible promissory note, net | 12,500,000 | — |
| Principal payment on promissory note | (1,000,000) | — |
| Proceeds from issuance of common stock, net | 30,609,789 | 11,781,626 |
| Repurchase of common stock | (11,202,542) | — |
| Proceeds from stock issuances related to exercise of stock options and employee stock purchase plan | 92,992 | 32,898 |
| Net cash provided by financing activities | 31,000,239 | 11,814,524 |
| Net (decrease) increase in cash and cash equivalents | (43,722,957) | 437,527 |
| Cash and cash equivalents at beginning of period | 58,083,409 | 13,876,090 |
| Cash and cash equivalents at end of period | <u>\$ 14,360,452</u> | <u>\$ 14,313,617</u> |
| Supplemental disclosure of cash flow information: | | |
| Cash paid for interest | <u>\$ 4,861</u> | <u>\$ 52,500</u> |

See accompanying notes to condensed financial statements.

Conatus Pharmaceuticals Inc.

**Notes to Condensed Financial Statements
(Unaudited)**

1. Organization and Basis of Presentation

Conatus Pharmaceuticals Inc. (the Company) was incorporated in the state of Delaware on July 13, 2005. The Company is a biotechnology company focused on the development and commercialization of novel medicines to treat liver disease.

As of September 30, 2017, the Company has devoted substantially all of its efforts to product development and has not realized product sales revenues from its planned principal operations.

The Company has a limited operating history, and the sales and income potential of the Company's business and market are unproven. The Company has experienced net losses since its inception and, as of September 30, 2017, had an accumulated deficit of \$163.7 million. The Company expects to continue to incur net losses for at least the next several years. Successful transition to attaining profitable operations is dependent upon achieving a level of revenues adequate to support the Company's cost structure. If the Company is unable to generate revenues adequate to support its cost structure, the Company may need to raise additional equity or debt financing.

The accompanying unaudited interim condensed financial statements have been prepared in accordance with U.S. generally accepted accounting principles (GAAP) and the rules and regulations of the Securities and Exchange Commission (SEC) related to a quarterly report on Form 10-Q. Certain information and note disclosures normally included in annual financial statements prepared in accordance with GAAP have been condensed or omitted pursuant to those rules and regulations. The unaudited interim condensed financial statements reflect all adjustments which, in the opinion of management, are necessary for a fair statement of the results for the periods presented. All such adjustments are of a normal and recurring nature. The operating results presented in these unaudited interim condensed financial statements are not necessarily indicative of the results that may be expected for any future periods. These unaudited interim condensed financial statements should be read in conjunction with the audited financial statements and the notes thereto for the year ended December 31, 2016 included in the Company's annual report on Form 10-K filed with the SEC on March 16, 2017.

2. Summary of Significant Accounting Policies

Use of Estimates

The preparation of condensed financial statements in conformity with GAAP requires management to make estimates and assumptions that affect the reported amounts of assets and liabilities and disclosure of contingent assets and liabilities at the date of the financial statements and the reported amounts of expenses during the reporting period. Actual results could differ from those estimates.

Concentrations of Credit Risk

Financial instruments that potentially subject the Company to significant concentrations of credit risk consist primarily of cash, cash equivalents and marketable securities. The Company maintains deposits in federally insured financial institutions in excess of federally insured limits. The Company has not experienced any losses in such accounts and believes it is not exposed to significant risk on its cash. Additionally, the Company established guidelines regarding approved investments and maturities of investments, which are designed to maintain safety and liquidity.

Cash and Cash Equivalents

The Company considers all highly liquid investments with an original maturity from the date of purchase of three months or less to be cash equivalents. Cash and cash equivalents include cash in readily available checking and money market accounts.

Marketable Securities

The Company classifies its marketable securities as available-for-sale and records such assets at estimated fair value in the condensed balance sheets, with unrealized gains and losses, if any, reported as a component of other comprehensive income (loss) within the condensed statements of operations and comprehensive loss and as a separate component of stockholders' equity. The Company classifies marketable securities with remaining maturities greater than one year as current assets because such marketable

securities are available to fund the Company's current operations. The Company invests its excess cash balances primarily in corporate debt securities and money market funds with strong credit ratings. Realized gains and losses are calculated on the specific identification method and recorded as interest income. There were no realized gains and losses for the nine-month periods ended September 30, 2017 and 2016.

At each balance sheet date, the Company assesses available-for-sale securities in an unrealized loss position to determine whether the unrealized loss is other-than-temporary. The Company considers factors including: the significance of the decline in value compared to the cost basis, underlying factors contributing to a decline in the prices of securities in a single asset class, the length of time the market value of the security has been less than its cost basis, the security's relative performance versus its peers, sector or asset class, expected market volatility and the market and economy in general. When the Company determines that a decline in the fair value below its cost basis is other-than-temporary, the Company recognizes an impairment loss in the period in which the other-than-temporary decline occurred. There have been no other-than-temporary declines in the value of marketable securities, as it is more likely than not the Company will hold the securities until maturity or a recovery of the cost basis.

Fair Value of Financial Instruments

The carrying amounts of prepaid and other current assets, accounts payable and accrued expenses are reasonable estimates of their fair value because of the short maturity of these items.

Stock-Based Compensation

Stock-based compensation expense for stock option grants under the Company's stock option plans is recorded at the estimated fair value of the award as of the grant date and is recognized as expense on a straight-line basis over the requisite service period of the stock-based award. Stock-based compensation expense for employee stock purchases under the Company's 2013 Employee Stock Purchase Plan (the ESPP) is recorded at the estimated fair value of the purchase as of the plan enrollment date and is recognized as expense on a straight-line basis over the applicable six-month ESPP offering period. The estimation of stock option and ESPP fair value requires management to make estimates and judgments about, among other things, employee exercise behavior, forfeiture rates and volatility of the Company's common stock. The judgments directly affect the amount of compensation expense that will be recognized.

Property and Equipment

Property and equipment, which consists of furniture and fixtures, computers and office equipment and leasehold improvements, are stated at cost and depreciated over the estimated useful lives of the assets (three to five years) using the straight-line method. Leasehold improvements are amortized over the shorter of their estimated useful lives or the lease term.

Long-Lived Assets

The Company regularly reviews the carrying value and estimated lives of all of its long-lived assets, including property and equipment, to determine whether indicators of impairment may exist which warrant adjustments to carrying values or estimated useful lives. The determinants used for this evaluation include management's estimate of the asset's ability to generate positive income from operations and positive cash flow in future periods, as well as the strategic significance of the assets to the Company's business objective. Should an impairment exist, the impairment loss would be measured based on the excess of the carrying amount of the asset's fair value. The Company has not recognized any impairment losses through September 30, 2017.

Revenue Recognition

The Company recognizes revenue when each of the following four criteria is met: (i) persuasive evidence of an arrangement exists; (ii) products are delivered or as services are rendered; (iii) the sales price is fixed or determinable; and (iv) collectability is reasonably assured.

The Company recognizes revenue under its Option, Collaboration and License Agreement (the Collaboration Agreement) with Novartis Pharma AG (Novartis) based on the relevant accounting literature. Under this guidance, multiple elements or deliverables may include (i) grants of licenses, or options to obtain licenses, to intellectual property, (ii) research and development services, (iii) participation on joint research and/or joint development committees, and/or (iv) manufacturing or supply services. The payments entities may receive under these arrangements typically include one or more of the following: non-refundable, upfront license fees; option exercise fees; funding of research and/or development efforts; amounts due upon the achievement of specified objectives; and/or royalties on future product sales.

Multiple-element arrangements require the separability of deliverables included in an arrangement into different units of accounting and the allocation of arrangement consideration to the units of accounting. The evaluation of multiple-element arrangements requires management to make judgments about (i) the identification of deliverables, (ii) whether such deliverables are separable from the other aspects of the contractual relationship, (iii) the estimated selling price of each deliverable, and (iv) the expected period of performance for each deliverable.

To determine the units of accounting under a multiple-element arrangement, management evaluates certain separation criteria, including whether the deliverables have stand-alone value, based on the relevant facts and circumstances for each arrangement. Management then estimates the selling price for each unit of accounting and allocates the arrangement consideration to each unit using the relative selling price method. The allocated consideration for each unit of accounting is recognized based on the method most appropriate for that unit of account and in accordance with the revenue recognition criteria detailed above.

If there are deliverables in an arrangement that are not separable from other aspects of the contractual relationship, they are treated as a combined unit of accounting, with the allocated revenue for the combined unit recognized in a manner consistent with the revenue recognition applicable to the final deliverable in the combined unit. Payments received prior to satisfying the relevant revenue recognition criteria are recorded as deferred revenue in the accompanying balance sheets and recognized as revenue when the related revenue recognition criteria are met.

The Collaboration Agreement provides for non-refundable milestone payments. The Company recognizes revenue that is contingent upon the achievement of a substantive milestone in its entirety in the period in which the milestone is achieved. A milestone is considered substantive when the consideration payable to the Company for such milestone (i) is consistent with the Company's performance necessary to achieve the milestone or the increase in value to the collaboration resulting from the Company's performance, (ii) relates solely to the Company's past performance and (iii) is reasonable relative to all of the other deliverables and payments within the arrangement. In making this assessment, the Company considers all facts and circumstances relevant to the arrangement, including factors such as the scientific, regulatory, commercial and other risks that must be overcome to achieve the milestone, the level of effort and investment required to achieve the milestone and whether any portion of the milestone consideration is related to future performance or deliverables.

The Company periodically reviews the estimated performance periods under the Collaboration Agreement, which provides for non-refundable upfront payments and fees. The Company will adjust the periods over which revenue should be recognized when appropriate to reflect changes in assumptions relating to the estimated performance periods. The Company could accelerate revenue recognition in the event of early termination of programs or if the Company's expectations change. Alternatively, the Company could decelerate revenue recognition if programs are extended or delayed. While such changes to the Company's estimates have no impact on the Company's reported cash flows, the amount of revenue recorded in future periods could be materially impacted.

The Company records revenues related to the reimbursement of costs incurred under the Collaboration Agreement where the Company acts as a principal, controls the research and development activities and bears credit risk. Under the Collaboration Agreement, the Company is reimbursed for associated out-of-pocket costs and for a certain amount of the Company's full-time equivalent (FTE) costs based on an agreed-upon FTE rate. The gross amount of these pass-through reimbursed costs is reported as revenue in the accompanying statements of operations and comprehensive loss, while the actual expenses for which the Company is reimbursed are reflected as research and development costs.

See Note 8 – Collaboration and License Agreements for further information.

Research and Development Expenses

All research and development costs are expensed as incurred.

Income Taxes

The Company's policy related to accounting for uncertainty in income taxes prescribes a recognition threshold and measurement attribute criteria for the financial statement recognition and measurement of tax positions taken or expected to be taken in a tax return. For those benefits to be recognized, a tax position must be more likely than not to be sustained upon examination by taxing authorities. As of December 31, 2016, there are no unrecognized tax benefits included in the condensed balance sheet that would, if recognized, affect the Company's effective tax rate, and the Company has noted no material changes through September 30, 2017. The Company has not recognized interest and penalties in the condensed balance sheets or condensed statements of operations and comprehensive loss. The Company is subject to U.S. and California taxation. As of December 31, 2016, the Company's tax years beginning 2005 to date are subject to examination by taxing authorities.

Comprehensive Loss

The Company is required to report all components of comprehensive loss, including net loss, in the condensed financial statements in the period in which they are recognized. Comprehensive loss is defined as the change in equity during a period from transactions and other events and circumstances from nonowner sources, including unrealized gains and losses on marketable securities. Comprehensive gains (losses) have been reflected in the condensed statements of operations and comprehensive loss for all periods presented.

Segment Reporting

Operating segments are identified as components of an enterprise about which separate discrete financial information is used in making decisions regarding resource allocation and assessing performance. To date, the Company has viewed its operations and managed its business as one segment operating primarily in the United States.

Net Loss Per Share

Basic net loss per share is calculated by dividing the net loss by the weighted average number of common shares outstanding during the period. Diluted net loss per share is computed by dividing the net loss by the weighted average number of common shares and common share equivalents outstanding for the period. Common stock equivalents are only included when their effect is dilutive. The Company's potentially dilutive securities have been excluded from the computation of diluted net loss per share in the periods in which they would be anti-dilutive. For all periods presented, there is no difference in the number of shares used to compute basic and diluted shares outstanding due to the Company's net loss position.

The following table sets forth the outstanding potentially dilutive securities that have been excluded in the calculation of diluted net loss per share because to do so would be anti-dilutive.

| | September 30, | |
|---|------------------|------------------|
| | 2017 | 2016 |
| Warrants to purchase common stock | 149,704 | 149,704 |
| Common stock options issued and outstanding | 4,910,557 | 3,496,826 |
| Shares issuable upon conversion of convertible note payable | 2,297,138 | — |
| Common stock subject to repurchase | — | 6,316 |
| ESPP shares pending issuance | 7,514 | 7,415 |
| Total | <u>7,364,913</u> | <u>3,660,261</u> |

Recent Accounting Pronouncements

In May 2014, the Financial Accounting Standards Board (FASB) issued Accounting Standards Update (ASU) No. 2014-09, *Revenue from Contracts with Customers (Topic 606)*. This guidance requires that an entity recognize revenue to depict the transfer of goods or services to customers in an amount that reflects the consideration to which the entity expects to be entitled in exchange for those goods or services. For public companies, ASU No. 2014-09 is effective for annual reporting periods beginning after December 15, 2017 and interim periods within that reporting period. Early adoption is permitted for annual reporting periods beginning after December 15, 2016. The Company is continuing to assess the impact of the new guidance on its accounting policies and procedures and is evaluating the new requirements as applied to existing revenue contracts. While this assessment is still in progress, the Company believes the most significant impact will relate to the timing of collaboration revenues, where the recognition of variable consideration such as milestone payments may be accelerated. In conjunction with its continuing assessment of the impact of the new guidance, the Company is also evaluating its method of adoption and reviewing and updating its internal controls over financial reporting to ensure that information required to implement the new standard is appropriately captured and recorded. The Company will implement any changes as required to facilitate adoption of the new guidance beginning in the first quarter of 2018.

In February 2016, the FASB issued ASU No. 2016-02, *Leases (Topic 842)*. This guidance requires organizations that lease assets with lease terms of more than 12 months to recognize on the balance sheet the assets and liabilities for the rights and obligations created by those leases. The ASU also requires disclosures to give financial statement users information on the amount, timing and uncertainty of cash flows arising from leases, including qualitative and quantitative information. For public companies, ASU No. 2016-02 is effective for fiscal years, and interim periods within those fiscal years, beginning after December 15, 2018. Early adoption is permitted. The Company is currently evaluating the impact of the pending adoption of ASU No. 2016-02 on its financial statements and related disclosures.

In March 2016, the FASB issued ASU No. 2016-09, *Compensation—Stock Compensation (Topic 718)*. This guidance changes the accounting for certain aspects of stock-based compensation, including income taxes, forfeitures, tax withholding and classification on the statement of cash flows. For public companies, ASU No. 2016-09 is effective for annual and interim periods beginning after

December 15, 2016. The Company adopted this guidance effective March 31, 2017, as required. The adoption of this guidance had an immaterial impact on the Company's financial statements and related disclosures.

In August 2016, the FASB issued ASU No. 2016-15, *Statement of Cash Flows (Topic 230): Classification of Certain Cash Receipts and Cash Payments*. This guidance addresses the presentation and classification of certain cash flow items, including the classification of cash receipts and payments that have aspects of more than one class of cash flows, to reduce the existing diversity in practice. ASU 2016-15 is effective for fiscal years, and interim periods within those fiscal years, beginning after December 15, 2017. Early adoption is permitted. The Company early adopted this guidance effective June 30, 2017. The adoption of this guidance had no impact on the Company's financial statements and related disclosures.

In May 2017, the FASB issued ASU No. 2017-09, *Compensation—Stock Compensation (Topic 718)*. This guidance amends the scope of modification accounting for share-based payment arrangements and addresses the types of changes to the terms or conditions of share-based payment awards to which an entity would be required to apply modification accounting under Topic 718. For public companies, ASU No. 2017-09 is effective for annual periods, and interim periods within those annual periods, beginning after December 15, 2017. Early adoption is permitted. The Company early adopted this guidance effective June 30, 2017. The adoption of this guidance had no impact on the Company's financial statements and related disclosures.

3. Fair Value Measurements

The accounting guidance defines fair value, establishes a consistent framework for measuring fair value and expands disclosure for each major asset and liability category measured at fair value on either a recurring or nonrecurring basis. Fair value is defined as an exit price, representing the amount that would be received to sell an asset or paid to transfer a liability in an orderly transaction between market participants. As such, fair value is a market-based measurement that should be determined based on assumptions that market participants would use in pricing an asset or liability. As a basis for considering such assumptions, the accounting guidance establishes a three-tier fair value hierarchy, which prioritizes the inputs used in measuring fair value as follows:

- Level 1: Includes financial instruments for which quoted market prices for identical instruments are available in active markets.
- Level 2: Includes financial instruments for which there are inputs other than quoted prices for similar instruments in active markets, quoted prices for identical or similar instruments in markets with insufficient volume or infrequent transaction (less active markets) or model-driven valuations in which significant inputs are observable or can be derived principally from, or corroborated by, observable market data.
- Level 3: Includes financial instruments for which fair value is derived from valuation techniques in which one or more significant inputs are unobservable, including management's own assumptions.

Below is a summary of assets measured at fair value as of September 30, 2017 and December 31, 2016.

| | September 30, 2017 | Fair Value Measurements Using | | |
|---------------------------|-----------------------|---|--|--|
| | | Quoted Prices in Active Markets for Identical Assets (Level 1) | Significant Other Observable Inputs (Level 2) | Significant Unobservable Inputs (Level 3) |
| Assets | | | | |
| Money market funds | \$ 12,875,586 | \$ 12,875,586 | \$ — | \$ — |
| Corporate debt securities | 70,823,435 | — | 70,823,435 | — |
| Total | <u>\$ 83,699,021</u> | <u>\$ 12,875,586</u> | <u>\$ 70,823,435</u> | <u>\$ —</u> |
| | | | | |
| | December 31, 2016 | Fair Value Measurements Using | | |
| | | Quoted Prices in Active Markets for Identical Assets (Level 1) | Significant Other Observable Inputs (Level 2) | Significant Unobservable Inputs (Level 3) |
| Assets | | | | |
| Money market funds | \$ 45,523,208 | \$ 45,523,208 | \$ — | \$ — |
| Corporate debt securities | 27,702,317 | — | 27,702,317 | — |
| Total | <u>\$ 73,225,525</u> | <u>\$ 45,523,208</u> | <u>\$ 27,702,317</u> | <u>\$ —</u> |

The Company's marketable securities, consisting principally of debt securities, are classified as available-for-sale, are stated at fair value, and consist of Level 2 financial instruments in the fair value hierarchy. The Company determines the fair value of its debt security holdings based on pricing from a service provider. The service provider values the securities based on using market prices from a variety of industry-standard independent data providers. Such market prices may be quoted prices in active markets for identical assets (Level 1 inputs) or pricing determined using inputs other than quoted prices that are observable either directly or indirectly (Level 2 inputs), such as yield curve, volatility factors, credit spreads, default rates, loss severity, current market and contractual prices for the underlying instruments or debt, broker and dealer quotes, as well as other relevant economic measures.

4. Marketable Securities

The Company invests its excess cash in money market funds and debt instruments of financial institutions, corporations, government sponsored entities and municipalities. The following tables summarize the Company's marketable securities:

| As of September 30, 2017 | Maturity (in years) | Amortized Cost | Unrealized Gains | Unrealized Losses | Estimated Fair Value |
|---------------------------|------------------------|-------------------|---------------------|----------------------|-------------------------|
| Corporate debt securities | 1 or less | \$ 70,846,528 | \$ 1,235 | \$ (24,328) | \$ 70,823,435 |
| Total | | \$ 70,846,528 | \$ 1,235 | \$ (24,328) | \$ 70,823,435 |

| As of December 31, 2016 | Maturity (in years) | Amortized Cost | Unrealized Gains | Unrealized Losses | Estimated Fair Value |
|---------------------------|------------------------|-------------------|---------------------|----------------------|-------------------------|
| Corporate debt securities | 1 or less | \$ 18,937,860 | \$ 901 | \$ (7,046) | \$ 18,931,715 |
| Total | | \$ 18,937,860 | \$ 901 | \$ (7,046) | \$ 18,931,715 |

5. Property and Equipment

Property and equipment consist of the following:

| | September 30, 2017 | December 31, 2016 |
|--|-----------------------|----------------------|
| Furniture and fixtures | \$ 333,670 | \$ 333,670 |
| Computer equipment and office equipment | 142,045 | 119,354 |
| Leasehold improvements | 152,217 | 152,217 |
| | 627,932 | 605,241 |
| Less accumulated depreciation and amortization | (423,018) | (343,795) |
| Total | \$ 204,914 | \$ 261,446 |

6. Note Payable

In July 2010, the Company issued to Pfizer Inc. (Pfizer) a \$1.0 million promissory note (the Pfizer Note). The Pfizer Note bore interest at a rate of 7% per annum and was scheduled to mature on July 29, 2020. Interest was payable on a quarterly basis. In July 2013, the Pfizer Note was amended to become convertible into shares of the Company's common stock following the completion of the Company's initial public offering (IPO), at the option of the holder, at a price per share equal to the fair market value of the common stock on the date of conversion. On January 24, 2017, the Company voluntarily prepaid the entire balance of the outstanding principal and accrued and unpaid interest of the Pfizer Note in the amount of \$1,004,861.

Prior to the prepayment of the Pfizer Note, the Company recorded the Pfizer Note on the balance sheet at face value. Based on borrowing rates available to the Company for loans with similar terms, the Company believed that the fair value of the Pfizer Note approximated its carrying value. The fair value measurement was categorized within Level 3 of the fair value hierarchy.

On February 15, 2017, the Company issued a convertible promissory note (the Novartis Note) in the principal amount of \$15.0 million, pursuant to the Investment Agreement entered into between the Company and Novartis on December 19, 2016 (the Investment Agreement). The Novartis Note bears interest on the unpaid principal balance at a rate of 6% per annum and has a scheduled maturity date of December 31, 2019. The Company may prepay or convert all or part of the Novartis Note into shares of the Company's common stock, at its option, until December 31, 2019. Novartis has the option to convert all or part of the Novartis Note into shares of the Company's common stock upon a change in control of the Company or termination of the Collaboration Agreement by Novartis pursuant to certain provisions. If converted, the principal and accrued interest under the Novartis Note will convert into the Company's common stock at a conversion price equal to 120% of the 20-day trailing average closing price per share of the common stock immediately prior to the conversion date. In the event the aggregate number of shares of common stock issued upon the

conversion would exceed the lesser of 19.0% of the Company's outstanding shares on a fully-diluted basis (i) at the inception of the Investment Agreement or (ii) on the conversion date, then only the lesser amount shall convert into shares of common stock and Novartis shall be repaid in cash for any remaining principal and unpaid interest after such conversion. Upon the occurrence of certain events of default, the Novartis Note requires the Company to repay the principal balance of the Novartis Note and any unpaid accrued interest. The ability to borrow and repay the debt at a discount using shares of the Company's common stock was deemed to be additional, foregone revenue attributable to the Collaboration Agreement, which the Company imputed and recorded as both a receivable from Novartis and a liability (deferred revenue) of \$2.5 million at the inception of the Collaboration Agreement and the Investment Agreement. On February 15, 2017, the Company recorded the \$15.0 million proceeds from the issuance of the Novartis Note as a convertible note payable in the amount of \$12.5 million and a reduction of the outstanding receivable from Novartis of \$2.5 million. The convertible note payable, along with the related accrued interest, totaled \$13.0 million as of September 30, 2017.

The Company elected to account for the Novartis Note under the fair value option. At September 30, 2017, the Company concluded that the fair value of the Novartis Note remained at \$13.0 million due to its conversion features. The fair value measurement is categorized within Level 3 of the fair value hierarchy.

7. Stockholders' Equity

Common Stock

In May 2017, the Company completed a public offering of 5,980,000 shares of its common stock at a public offering price of \$5.50 per share. The shares were registered pursuant to a registration statement on Form S-3 filed on August 14, 2014. The Company received net proceeds of \$30.6 million, after deducting underwriting discounts and commissions and offering-related transaction costs. Immediately following the offering, the Company used \$11.2 million of the net proceeds to repurchase and retire 2,166,836 shares of its common stock from funds affiliated with Advent Private Equity (collectively Advent) at a price of \$5.17 per share, which is equal to the net proceeds per share that the Company received from the offering, before expenses, pursuant to a stock purchase agreement the Company entered into with Advent in May 2017.

Warrants

In 2013, the Company issued warrants exercisable for 1,124,026 shares of Series B preferred stock, at an exercise price of \$0.90 per share, to certain existing investors in conjunction with a private placement (the 2013 Warrants) and warrants exercisable for 111,112 shares of Series B preferred stock, at an exercise price of \$0.90 per share, to Oxford Finance LLC and Silicon Valley Bank in conjunction with the Company's entry into a loan and security agreement (the Lender Warrants). Upon completion of the IPO, the 2013 Warrants and the Lender Warrants became exercisable for 136,236 and 13,468 shares of common stock, respectively, at an exercise price of \$7.43 per share. The 2013 Warrants and the Lender Warrants will expire on May 30, 2018 and July 3, 2023, respectively.

Stock Options

The following table summarizes the Company's stock option activity under all stock option plans for the nine months ended September 30, 2017:

| | Total Options | Weighted- Average Exercise Price |
|-------------------------------|--------------------------|---|
| Balance at December 31, 2016 | 3,393,813 | \$ 5.10 |
| Granted | 1,702,600 | 4.91 |
| Exercised | (62,944) | 1.19 |
| Cancelled | (122,912) | 4.67 |
| Balance at September 30, 2017 | <u>4,910,557</u> | <u>\$ 5.10</u> |

On August 31, 2017, in connection with the appointment of its new Executive Vice President, Chief Operating Officer and Chief Financial Officer, the Company granted stock options to purchase 525,000 shares of the Company's common stock outside of its stock option plans. The grant was made as an inducement that was a material component of the executive's compensation and acceptance of employment with the Company and was granted as an employment inducement award pursuant to NASDAQ Listing Rule 5635(c)(4). While granted outside of the Company's stock option plans, the terms and conditions of this award are consistent with awards granted to the Company's executive officers pursuant to the Company's 2013 Incentive Award Plan. The stock options

will vest over a four-year period, with a quarter of the options vesting on the first anniversary of the date of grant and the remainder of the options vesting monthly over the subsequent three years.

Stock-Based Compensation

The Company recorded stock-based compensation of \$1.0 million and \$0.7 million for the three months ended September 30, 2017 and 2016, respectively, and \$3.0 million and \$2.5 million for the nine months ended September 30, 2017 and 2016, respectively.

Common Stock Reserved for Future Issuance

The following shares of common stock were reserved for future issuance at September 30, 2017:

| | |
|---|------------------|
| Warrants to purchase common stock | 149,704 |
| Common stock options issued and outstanding | 4,910,557 |
| Common stock authorized for future option grants | 545,503 |
| Common stock authorized for the ESPP | 544,578 |
| Shares issuable upon conversion of convertible note payable | 2,297,138 |
| Total | <u>8,447,480</u> |

8. Collaboration and License Agreements

In December 2016, the Company entered into the Collaboration Agreement with Novartis, pursuant to which the Company granted Novartis an exclusive option to collaborate with the Company to develop products containing emricasan. Pursuant to the Collaboration Agreement, the Company received a non-refundable upfront payment of \$50.0 million from Novartis.

In May 2017, Novartis exercised its option under the Collaboration Agreement. In July 2017, the Company received a \$7.0 million option exercise payment, at which time the license under the Collaboration Agreement became effective (the License Effective Date). Under the Collaboration Agreement, the Company will be eligible to receive up to an aggregate of \$650.0 million in milestone payments over the term of the Collaboration Agreement, contingent on the achievement of certain development, regulatory and commercial milestones, as well as royalties or profit and loss sharing on future product sales in the United States, if any.

Pursuant to the Collaboration Agreement, the Company is responsible for completing its four ongoing Phase 2b trials. Novartis will generally pay 50% of the Company's Phase 2b emricasan development costs pursuant to an agreed upon budget. Novartis will assume full responsibility for emricasan's Phase 3 development and all combination product development.

Unless terminated earlier, the Collaboration Agreement will remain in effect on a product-by-product and country-by-country basis until Novartis' royalty obligations expire. Novartis has certain termination rights in the event of a mandated clinical trial hold for any product containing emricasan as its sole active ingredient. Additionally, Novartis has the right to terminate the Collaboration Agreement without cause upon 180 days prior written notice to the Company. In such event, the license granted to Novartis will be terminated and revert to the Company. In the event Novartis terminates the Collaboration Agreement due to the Company's uncured material breach or insolvency, the license granted to Novartis pursuant to the Collaboration Agreement will become irrevocable, and Novartis will be required to continue to make all milestone and royalty payments otherwise due to the Company under the Collaboration Agreement, provided that if the Company materially breaches the Collaboration Agreement such that the rights licensed to Novartis or the commercial prospects of the emricasan products are seriously impaired, the milestone and royalty payments will be reduced by 50%.

Under the relevant accounting literature, the Collaboration Agreement meets the definition of a collaborative arrangement and a multiple-element arrangement. The Company concluded that there were two significant deliverables under the Collaboration Agreement – the option to obtain the license and the research and development services – but that the license does not have stand-alone value as Novartis cannot obtain value from the license without the research and development services, which the Company is uniquely able to perform. As such, the Company expects to recognize as collaboration revenue a portion of the upfront payment received of \$50.0 million, the option exercise fee of \$7.0 million, and the imputed income from the Investment Agreement as described below on a straight-line basis between the inception of the agreement (or with respect to the option exercise fee, upon exercise of the option) through mid-2019 – the estimated period over which the Company expects to perform the research and development services. Due to the inherently unpredictable nature of product development activities, the Company periodically reviews the performance period of the research and development services and will adjust the period over which revenue is recognized when appropriate. The Company could accelerate revenue recognition in the event of early termination of programs or if the Company's expectations change. Alternatively, the Company could decelerate revenue recognition if programs are extended or delayed. While

such changes to the Company's estimates have no impact on the Company's reported cash flows, the amount of revenue recorded in future periods could be materially impacted. Expense reimbursements for the Company's Phase 2b emricasan development costs will be recognized as collaboration revenue when the related expenses are incurred.

Under the Investment Agreement, the Company is able to borrow up to \$15.0 million at a rate of 6% per annum, under one or two notes, which will mature on December 31, 2019. The Company may elect at its sole discretion to convert all or part of the outstanding principal and accrued interest into fully paid shares of common stock, at 120% of the 20-day trailing average closing price per share of the common stock immediately prior to the conversion date. Novartis has the option to convert all or part of the note(s) into shares of the Company's common stock upon a change in control of the Company or termination of the Collaboration Agreement by Novartis pursuant to certain provisions. In the event the conversion of the notes would exceed the lesser of 19.0% of the Company's outstanding shares on a fully-diluted basis (i) at the inception of the Investment Agreement or (ii) on the conversion date, then only the lesser amount shall convert into shares of common stock and Novartis shall be repaid in cash for any remaining principal and unpaid interest after such conversion. This ability to borrow and repay the debt at a discount using shares of the Company's common stock was deemed to be additional, foregone revenue attributable to the Collaboration Agreement, which the Company imputed and recorded as both a receivable from Novartis and a liability (deferred revenue) of \$2.5 million at the inception of the Collaboration Agreement and the Investment Agreement. On February 15, 2017, the Company issued the Novartis Note in the principal amount of \$15.0 million and recorded the \$15.0 million proceeds as a convertible note payable in the amount of \$12.5 million and a reduction of the outstanding receivable from Novartis of \$2.5 million.

9. Commitments

In February 2014, the Company entered into a noncancelable operating lease agreement (the Lease) for certain office space with a lease term from July 2014 through December 2019 and a renewal option for an additional five years. In May 2015, the Company entered into a first amendment to the Lease (the First Lease Amendment) for additional office space starting in September 2015 through September 2020. The First Lease Amendment also extended the term of the Lease to September 2020. The monthly base rent under the Lease and the First Lease Amendment increases approximately 3% annually from \$32,784 in 2015 to \$39,268 in 2020. Future minimum payments under this noncancelable operating lease total \$1.3 million at September 30, 2017.

Rent expense was \$94,501 for each of the three-month periods ended September 30, 2017 and 2016 and \$283,504 for each of the nine-month periods ended September 30, 2017 and 2016.

In July 2010, the Company entered into a stock purchase agreement with Pfizer, pursuant to which the Company acquired all of the outstanding stock of Idun Pharmaceuticals, Inc., which was subsequently spun off to the Company's stockholders in January 2013. Under the stock purchase agreement, the Company may be required to make payments to Pfizer totaling \$18.0 million upon the achievement of specified regulatory milestones.

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

The following discussion and analysis and the unaudited interim condensed financial statements included in this quarterly report on Form 10-Q should be read in conjunction with the financial statements and notes thereto for the year ended December 31, 2016 and the related Management's Discussion and Analysis of Financial Condition and Results of Operations, both of which are contained in our annual report on Form 10-K filed with the Securities and Exchange Commission, or SEC, on March 16, 2017.

Forward-Looking Statements

This quarterly report on Form 10-Q contains forward-looking statements within the meaning of Section 21E of the Securities Exchange Act of 1934, as amended, or the Exchange Act. All statements other than statements of historical facts contained in this quarterly report, including statements regarding our future results of operations and financial position, business strategy, prospective products, product approvals, research and development costs, timing and likelihood of success, plans and objectives of management for future operations and future results of anticipated products, are forward-looking statements. These statements involve known and unknown risks, uncertainties and other important 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 the forward-looking statements.

In some cases, you can identify forward-looking statements by terms such as "may," "will," "should," "expect," "plan," "anticipate," "could," "intend," "target," "project," "contemplate," "believe," "estimate," "predict," "potential" or "continue" or the negative of these terms or other similar expressions. The forward-looking statements in this quarterly report are only predictions. We have based these forward-looking statements largely on our current expectations and projections about future events and financial trends that we believe may affect our business, financial condition and results of operations. These forward-looking statements speak only as of the date of this quarterly report and are subject to a number of risks, uncertainties and assumptions, including those described in Part II, Item 1A, "Risk Factors." The events and circumstances reflected in our forward-looking statements may not be achieved or occur, and actual results could differ materially from those projected in the forward-looking statements. Moreover, we operate in an evolving environment. New risk factors and uncertainties may emerge from time to time, and it is not possible for management to predict all risk factors and uncertainties. Except as required by applicable law, we do not plan to publicly update or revise any forward-looking statements contained herein, whether as a result of any new information, future events, changed circumstances or otherwise.

Overview

We are a biotechnology company focused on the development and commercialization of novel medicines to treat liver disease. We are developing emricasan, a first-in-class, orally active pan-caspase protease inhibitor, for the treatment of patients with chronic liver disease. Emricasan is designed to reduce the activities of human caspases, which are enzymes that mediate inflammation and apoptosis. We believe that by reducing the activity of these enzymes, caspase inhibitors have the potential to interrupt the progression of a variety of diseases.

We plan to continue advancing toward initial registration of emricasan for patients with cirrhosis due to nonalcoholic steatohepatitis, or NASH, with parallel development toward registration of emricasan for patients with NASH fibrosis. Our current clinical program for emricasan includes the following randomized, double-blind, placebo-controlled Phase 2b clinical trials:

- *Phase 2b ENCORE-PH (Portal Hypertension) Clinical Trial:* In November 2016, we initiated a clinical trial to evaluate the effect of emricasan in approximately 240 compensated or early decompensated NASH cirrhosis patients with severe portal hypertension. Top-line results are expected in the second half of 2018.
- *Phase 2b ENCORE-LF (Liver Function) Clinical Trial:* In May 2017, we initiated a clinical trial to evaluate emricasan in approximately 210 patients with decompensated NASH cirrhosis. Top-line results are expected in the second half of 2019.
- *Phase 2b ENCORE-NF (NASH Fibrosis) Clinical Trial:* In January 2016, we initiated a clinical trial to evaluate emricasan in approximately 330 patients with liver fibrosis resulting from NASH. Top-line results are expected in the first half of 2019.
- *Phase 2b POLT-HCV-SVR Clinical Trial:* In May 2014, we initiated a clinical trial in approximately 60 post-orthotopic liver transplant, or POLT, recipients with reestablished liver fibrosis post-transplant as a result of recurrent hepatitis C virus, or HCV, infection who have successfully achieved a sustained viral response, or SVR, following HCV antiviral therapy, or POLT-HCV-SVR, patients with residual fibrosis or cirrhosis, classified as Ishak Fibrosis Score 2-6. Top-line results are expected in the second quarter of 2018.

In addition, we plan to initiate a post-treatment observational study pursuant to which subjects from the four trials above will be followed for long-term three-year safety follow-up.

In May 2017, Novartis Pharma AG, or Novartis, exercised its option under the Option, Collaboration and License Agreement, or the Collaboration Agreement, we entered into with Novartis in December 2016. Pursuant to such exercise, we granted Novartis an exclusive, worldwide license to our intellectual property rights relating to emricasan to collaborate with us and develop and commercialize emricasan products, containing emricasan either as a single active ingredient or in combination with other Novartis compounds for liver cirrhosis or liver fibrosis, for the treatment, diagnosis and prevention of disease in all indications in humans. The license became effective upon our receipt of a \$7.0 million option exercise payment in July 2017.

Pursuant to the Collaboration Agreement, we are responsible for completing the three ENCORE trials and the POLT-HCV-SVR trial described above. We and Novartis will generally share the costs of these four Phase 2b trials and the planned observational study equally. Upon completion of the four Phase 2b trials, Novartis will assume 100% of the observational study costs. Novartis is responsible for 100% of certain expenses for required registration-supportive nonclinical activities. Novartis is also responsible for the development of emricasan beyond the four Phase 2b trials and planned observational study described above, including the Phase 3 development of emricasan single agent products and all development for emricasan combination products, and Novartis has agreed to use commercially reasonable efforts to develop and commercialize emricasan products. A joint steering committee comprised of representatives from our company and Novartis oversees the collaboration, development and commercialization of emricasan products.

Under the Collaboration Agreement, Novartis paid us an upfront payment of \$50.0 million and an option exercise payment of \$7.0 million. In addition, we are eligible to receive up to an aggregate of \$650.0 million in milestone payments, as well as royalties.

In June 2017, the U.S. Food and Drug Administration granted Orphan Drug Designation to our preclinical product candidate IDN-7314, a pan-caspase inhibitor, for the treatment of primary sclerosing cholangitis, or PSC, a disease affecting bile ducts in the liver, which can lead to cirrhosis and liver failure. In October 2017, the European Medicines Agency granted orphan designation to IDN-7314 for the treatment of PSC. We believe these orphan designations provide an opportunity to address an important unmet medical need and expand our development pipeline beyond emricasan. Pursuant to the Collaboration Agreement, Novartis will have a right of first negotiation prior to any offer for IDN-7314 by us to a third party, and we may not develop IDN-7314 in any pivotal registration clinical trials or commercialize IDN-7314 in liver disease until the earlier of five years after the first commercial sale of an emricasan product in the United States or major European market or ten years from the execution date of the Collaboration Agreement. We will continue to evaluate the potential of IDN-7314 as a product candidate as a component of our pipeline expansion plans. We also plan to expand our development pipeline by developing new product candidates based on our expertise in caspase inhibition or purchasing or in-licensing product candidates. In addition to liver disease, we may pursue the development of product candidates in other disease areas.

Since our inception, our primary activities have been organizational activities, including recruiting personnel, conducting research and development, including clinical trials, and raising capital. We have no products approved for sale, and we have not generated any revenues from product sales to date. We have funded our operations since inception primarily through sales of equity securities and convertible promissory notes and payments made under the Collaboration Agreement, and we have incurred significant operating losses since our inception. We have never been profitable and have incurred net losses of \$29.7 million and \$24.1 million for the years ended December 31, 2016 and 2015, respectively, and \$13.0 million for the nine months ended September 30, 2017. As of September 30, 2017, we had an accumulated deficit of \$163.7 million.

We expect to continue to incur significant operating losses and negative cash flows from operating activities for the foreseeable future as we continue the clinical development of emricasan and seek regulatory approval for and, if approved, pursue commercialization of emricasan. In May 2017, we completed a public offering of 5,980,000 shares of our common stock at a public offering price of \$5.50 per share. We received net proceeds of \$30.6 million, after deducting underwriting discounts and commissions and offering-related transaction costs. Immediately following the offering, we used \$11.2 million of the net proceeds to repurchase and retire 2,166,836 shares of our common stock from funds affiliated with Advent Private Equity, or Advent, at a price of \$5.17 per share.

As of September 30, 2017, we had cash, cash equivalents and marketable securities of \$85.2 million. Although it is difficult to predict future liquidity requirements, we believe that our existing cash, cash equivalents and marketable securities will be sufficient to fund our operations for at least the next 12 months from the date of the filing of this Form 10-Q. We will need to raise additional capital to fund further operations, including the development of product candidates other than emricasan. We may obtain additional financing in the future through the issuance of our common stock in future public offerings, through other equity or debt financings or through collaborations or partnerships with other companies.

Successful transition to profitability is dependent upon achieving a level of revenues adequate to support our cost structure. We cannot assure you that we will ever be profitable or generate sustained positive cash flow from operating activities and, unless and until we do, we will need to raise substantial additional capital through equity or debt financings or through collaborations or partnerships with other companies. We may not be able to raise additional capital on terms acceptable to us, or at all, and any failure to raise capital as and when needed could have a material adverse effect on our results of operations, financial condition and our ability to execute on our business plan.

JOBS Act

In April 2012, the Jumpstart Our Business Startups Act of 2012, or the JOBS Act, was signed into law. The JOBS Act contains provisions that, among other things, reduce certain reporting requirements for an “emerging growth company.” As an “emerging growth company,” we are electing not to take advantage of the extended transition period afforded by the JOBS Act for the implementation of new or revised accounting standards, and as a result, we will comply with new or revised accounting standards on the relevant dates on which adoption of such standards is required for non-emerging growth companies. Section 107 of the JOBS Act provides that our decision not to take advantage of the extended transition period is irrevocable. In addition, we are in the process of evaluating the benefits of relying on the other exemptions and reduced reporting requirements provided by the JOBS Act. Subject to certain conditions set forth in the JOBS Act, if as an “emerging growth company” we choose to rely on such exemptions, we may not be required to, among other things, (i) provide an auditor’s attestation report on our system of internal control over financial reporting pursuant to Section 404 of the Sarbanes-Oxley Act of 2002, (ii) provide all of the compensation disclosure that may be required of non-emerging growth public companies under the Dodd-Frank Wall Street Reform and Consumer Protection Act, (iii) comply with any requirement that may be adopted by the Public Company Accounting Oversight Board regarding mandatory audit firm rotation or a supplement to the auditor’s report providing additional information about the audit and the financial statements (auditor discussion and analysis) and (iv) disclose certain executive compensation-related items such as the correlation between executive compensation and performance and comparisons of the Chief Executive Officer’s compensation to median employee compensation. These exemptions will apply for a period of five years following the completion of our initial public offering, or IPO, or until we no longer meet the requirements of being an “emerging growth company,” whichever is earlier.

Financial Overview

Revenues

Our revenues to date have been generated primarily from the Collaboration Agreement with Novartis. Under the terms of the Collaboration Agreement, we received an upfront payment of \$50.0 million. In May 2017, Novartis exercised its option, and we received a \$7.0 million option exercise payment in July 2017. We are eligible to receive up to \$650.0 million in additional payments for development, regulatory and commercial sales milestones, as well as royalties or profit and loss sharing on future product sales in the United States, if any.

We currently have no products approved for sale, and we have not generated any revenues from product sales to date. We have not submitted any product candidate for regulatory approval. If we fail to achieve clinical success in the development of emricasan in a timely manner and/or obtain regulatory approval for this product candidate, or to successfully develop other product candidates, our ability to generate future revenues would be materially adversely affected.

Research and Development Expenses

The majority of our operating expenses to date have been incurred in research and development activities. Starting in late 2011, research and development expenses have been focused on the development of emricasan. Since acquiring emricasan in 2010, we have incurred \$98.5 million in the development of emricasan through September 30, 2017. Our business model is currently focused on the development of emricasan in various liver diseases and is dependent upon our continuing to conduct research and a significant amount of clinical development. Our research and development expenses consist primarily of:

- expenses incurred under agreements with contract research organizations, or CROs, investigative sites and consultants that conduct our clinical trials and our preclinical studies;
- employee-related expenses, which include salaries and benefits;
- the cost of finalizing our chemistry, manufacturing and controls, or CMC, capabilities and providing clinical trial materials; and
- costs associated with other research activities and regulatory approvals.

Research and development costs are expensed as incurred.

At this time, due to the inherently unpredictable nature of preclinical and clinical development, we are unable to estimate with any certainty the costs we will incur in the continued development of emricasan. Clinical development timelines, the probability of success and development costs can differ materially from expectations.

The costs of clinical trials may vary significantly over the life of a project owing to factors that include but are not limited to the following:

- per patient trial costs;
- the number of patients that participate in the clinical trials;
- the number of sites included in the clinical trials;
- the countries in which the clinical trials are conducted;
- the length of time required to enroll eligible patients;
- the number of doses that patients receive;
- the drop-out or discontinuation rates of patients;
- potential additional safety monitoring or other studies requested by regulatory agencies;
- the duration of patient follow-up; and
- the efficacy and safety profile of the product candidate.

We are currently focused on advancing emricasan in multiple indications, and our future research and development expenses will depend on its clinical success. In addition, we cannot forecast with any degree of certainty and to what extent Novartis will develop and commercialize emricasan.

Research and development expenditures will continue to be significant and will increase as we continue clinical development of emricasan over at least the next several years. We expect to incur significant development costs as we conduct our ongoing Phase 2b trials of emricasan and develop product candidates other than emricasan.

We do not expect emricasan to be commercially available, if at all, for at least the next several years.

General and Administrative Expenses

General and administrative expenses consist principally of salaries and related costs for personnel in executive, finance, business development and administrative functions. Other general and administrative expenses include costs related to being a public company, as well as insurance, facilities, travel, patent filing and maintenance, legal and consulting expenses.

If we exercise our option to co-commercialize emricasan pursuant to the Collaboration Agreement, we may incur expenses associated with activities related to commercializing emricasan. Some expenses may be incurred prior to receiving regulatory approval of emricasan. We do not expect to receive any such regulatory approval for at least the next several years.

Interest Income

Interest income consists primarily of interest income earned on our cash, cash equivalents and marketable securities.

Interest Expense

Interest expense consists of accrued interest on our \$15.0 million convertible promissory note payable to Novartis and coupon interest on our \$1.0 million promissory note payable to Pfizer Inc.

Other Income (Expense)

Other income (expense) includes non-operating transactions such as those caused by currency fluctuations between transaction dates and settlement dates and the conversion of account balances held in foreign currencies to U.S. dollars.

Critical Accounting Policies and Significant Judgments and Estimates

Our management's discussion and analysis of financial condition and results of operations is based on our condensed financial statements, which have been prepared in accordance with accounting principles generally accepted in the United States. The preparation of these financial statements requires us to make estimates and assumptions that affect the reported amounts of assets and liabilities and the disclosure of contingent assets and liabilities at the date of the financial statements, as well as the reported revenues and expenses during the reporting periods. These items are monitored and analyzed by us for changes in facts and circumstances, and material changes in these estimates could occur in the future. We base our estimates on historical experience and on various other factors that we believe are reasonable under the circumstances, the results of which form the basis for making judgments about the carrying value of assets and liabilities that are not readily apparent from other sources. Changes in estimates are reflected in reported results for the period in which they become known. Actual results may differ materially from these estimates under different assumptions or conditions.

There were no significant changes during the nine months ended September 30, 2017 to the critical accounting policies described in "Item 7 – Management's Discussion and Analysis of Financial Condition and Results of Operations – Critical Accounting Policies and Significant Judgments and Estimates" in our Annual Report on Form 10-K for the fiscal year ended December 31, 2016 filed with the SEC on March 16, 2017.

Results of Operations

Comparison of the Three Months Ended September 30, 2017 and 2016

Total Revenues

Total revenues were \$9.6 million for the three months ended September 30, 2017, as compared to \$0.0 million for the same period in 2016. For the three months ended September 30, 2017, total revenues consisted of collaboration revenue related to the Collaboration Agreement with Novartis, which was executed in December 2016.

We currently recognize collaboration revenue on the license portion of deferred revenue on a straight-line basis between the inception of the agreement (or with respect to the option exercise fee, upon exercise of the option) through mid 2019 – the estimated period over which we expect to perform the research and development services. Due to the inherently unpredictable nature of product development activities, we periodically review the performance period of the research and development services and will adjust the period over which revenue is recognized when appropriate. Changes in the performance period could materially impact the timing of future revenue recognition.

Research and Development Expenses

Research and development expenses were \$11.2 million for the three months ended September 30, 2017, as compared to \$4.8 million for the same period in 2016. The increase of \$6.4 million was primarily due to the ramp up of our ENCORE-NF, ENCORE-PH and ENCORE-LF clinical trials.

General and Administrative Expenses

General and administrative expenses were \$2.4 million for the three months ended September 30, 2017, as compared to \$2.1 million for the same period in 2016. The increase of \$0.3 million was primarily due to higher personnel costs and professional fees.

Changes in components of Other Income (Expense) were as follows:

Interest Income

Interest income was \$259,000 for the three months ended September 30, 2017, as compared to \$34,000 for the same period in 2016. Interest income consisted of interest earned on our cash, cash equivalents and marketable securities and fluctuates based on changes in investment balances and interest rates.

Interest Expense

Interest expense was \$189,000 for the three months ended September 30, 2017, as compared to \$18,000 for the same period in 2016. The increase was due to higher interest expense related to the \$15.0 million convertible promissory note issued to Novartis in February 2017, partially offset by lower interest expense related to the \$1.0 million promissory note payable to Pfizer Inc., which was voluntarily prepaid in January 2017.

Other (Expense) Income

Other expense was \$21,000 for the three months ended September 30, 2017, as compared to other income of \$12,000 for the same period in 2016. Other expense represents non-operating transactions such as those caused by currency fluctuations between transaction dates and settlement dates and the conversion of account balances held in foreign currencies to U.S. dollars.

Comparison of the Nine Months Ended September 30, 2017 and 2016

Total Revenues

Total revenues were \$26.6 million for the nine months ended September 30, 2017, as compared to \$0.0 million for the same period in 2016. For the nine months ended September 30, 2017, total revenues consisted of collaboration revenue related to the Collaboration Agreement with Novartis, which was executed in December 2016.

Research and Development Expenses

Research and development expenses were \$32.3 million for the nine months ended September 30, 2017, as compared to \$13.8 million for the same period in 2016. The increase of \$18.5 million was primarily due to the ramp up of our ENCORE-NF, ENCORE-PH and ENCORE-LF clinical trials.

General and Administrative Expenses

General and administrative expenses were \$7.4 million for the nine months ended September 30, 2017, as compared to \$6.9 million for the same period in 2016. The increase of \$0.5 million was primarily due to higher personnel costs and professional fees.

Changes in components of Other Income (Expense) were as follows:

Interest Income

Interest income was \$648,000 for the nine months ended September 30, 2017, as compared to \$95,000 for the same period in 2016. Interest income consisted of interest earned on our cash, cash equivalents and marketable securities and fluctuates based on changes in investment balances and interest rates.

Interest Expense

Interest expense was \$473,000 for the nine months ended September 30, 2017, as compared to \$53,000 for the same period in 2016. The increase was due to higher interest expense related to the \$15.0 million convertible promissory note issued to Novartis in February 2017, partially offset by lower interest expense related to the \$1.0 million promissory note payable to Pfizer Inc., which was voluntarily prepaid in January 2017.

Other (Expense) Income

Other expense was \$72,000 for the nine months ended September 30, 2017, as compared to other income of \$2,000 for the same period in 2016. Other expense represents non-operating transactions such as those caused by currency fluctuations between transaction dates and settlement dates and the conversion of account balances held in foreign currencies to U.S. dollars.

Liquidity and Capital Resources

We have incurred losses since inception and negative cash flows from operating activities through December 31, 2015. For the year ended December 31, 2016, we had positive net cash flows from operating activities due to the upfront payment related to the Collaboration Agreement with Novartis. As of September 30, 2017, we had an accumulated deficit of \$163.7 million. We anticipate that we will continue to incur net losses for the foreseeable future as we continue the development and potential commercialization of emricasan.

Prior to our IPO in July 2013, we funded our operations primarily through private placements of equity and convertible debt securities. In July 2013, we completed our IPO of 6,000,000 shares of common stock at an offering price of \$11.00 per share. We received net proceeds of \$58.6 million, after deducting underwriting discounts and commissions and offering-related transaction costs.

In August 2014, we entered into an At Market Issuance Sales Agreement, or the Sales Agreement, with MLV & Co. LLC, or MLV, pursuant to which we could sell from time to time, at our option, up to an aggregate of \$50.0 million of shares of our common stock through MLV, as sales agent. We terminated the Sales Agreement in December 2016. We sold 6,305,526 shares of our common stock pursuant to the Sales Agreement at a weighted average price per share of \$2.35 and received net proceeds of \$14.2 million, after deducting offering-related transaction costs and commissions.

In April 2015, we completed a public offering of 4,025,000 shares of our common stock at a public offering price of \$5.75 per share. We received net proceeds of \$21.4 million, after deducting underwriting discounts and commissions and offering-related transaction costs. In May 2017, we completed a public offering of 5,980,000 shares of our common stock at a public offering price of \$5.50 per share. We received net proceeds of \$30.6 million, after deducting underwriting discounts and commissions and offering-related transaction costs. Immediately following the offering, we used \$11.2 million of the net proceeds to repurchase and retire 2,166,836 shares of our common stock from Advent at a price of \$5.17 per share, which is equal to the net proceeds per share we received from the offering, before expenses, pursuant to a stock purchase agreement we entered into with Advent in May 2017.

In December 2016, we entered into the Collaboration Agreement with Novartis pursuant to which we granted Novartis an exclusive option to collaborate with us for the global development and commercialization of emricasan. Under the Collaboration Agreement, Novartis paid us an upfront payment of \$50.0 million. In May 2017, Novartis exercised its option, and we received a \$7.0 million option exercise payment in July 2017. Concurrent with the entry into the Collaboration Agreement, we entered into an Investment Agreement with Novartis whereby we agreed to sell and Novartis agreed to purchase, convertible promissory notes, in one or two closings, for an aggregate principal amount of up to \$15.0 million. In February 2017, we issued to Novartis a convertible promissory note, or the Novartis Note, in the principal amount of \$15.0 million. The maturity date of the Novartis Note is December 31, 2019. The Novartis Note bears interest on the unpaid principal balance at a rate of 6% per annum. We may prepay or convert the Novartis Note into shares of our common stock, at our option, until December 31, 2019. Novartis may convert the Novartis Note into shares of our common stock upon a change of control of our company or termination of the Collaboration Agreement by Novartis pursuant to certain provisions. If converted, the principal and accrued interest under the Novartis Note will convert into shares of our common stock at a conversion price equal to 120% of the 20-day trailing average closing price per share of the common stock immediately prior to the conversion date. Upon the occurrence of certain events of default, the Novartis Note requires us to repay the principal balance and any unpaid accrued interest.

At September 30, 2017, we had cash, cash equivalents and marketable securities of \$85.2 million. We believe our existing cash, cash equivalents and marketable securities will be sufficient to fund our operations for at least the next 12 months from the date of the filing of this Form 10-Q. To fund further operations, we will need to raise additional capital. We plan to continue to fund losses from operations and capital funding needs through future equity and debt financing, as well as potential collaborations. The sale of additional equity or convertible debt could result in additional dilution to our stockholders. The incurrence of indebtedness would result in debt service obligations and could result in operating and financing covenants that would restrict our operations. No assurances can be provided that financing will be available in the amounts we need or on terms acceptable to us, if at all. If we are not able to secure adequate additional funding, we may be forced to make reductions in spending, extend payment terms with suppliers, liquidate assets where possible, and/or suspend or curtail planned programs. Any of these actions could materially harm our business, results of operations and future prospects.

The following table sets forth a summary of the net cash flow activity for each of the periods set forth below:

| | Nine Months Ended September 30, | |
|--|--|-------------------|
| | 2017 | 2016 |
| Net cash used in operating activities | \$ (22,803,297) | \$ (17,158,408) |
| Net cash (used in) provided by investing activities | (51,919,899) | 5,781,411 |
| Net cash provided by financing activities | 31,000,239 | 11,814,524 |
| Net (decrease) increase in cash and cash equivalents | <u>\$ (43,722,957)</u> | <u>\$ 437,527</u> |

Net cash used in operating activities was \$22.8 million and \$17.2 million for the nine months ended September 30, 2017 and 2016, respectively. The primary use of cash was to fund our operations related to the development of emricasan.

Net cash used in investing activities was \$51.9 million for the nine months ended September 30, 2017, which consisted primarily of cash used to purchase marketable securities, partially offset by proceeds from maturities of marketable securities. Net cash provided by investing activities was \$5.8 million for the nine months ended September 30, 2016, which consisted primarily of proceeds from maturities of marketable securities, partially offset by cash used to purchase marketable securities.

Net cash provided by financing activities was \$31.0 million for the nine months ended September 30, 2017, which consisted primarily of net proceeds from our public offering in May 2017 and proceeds from the issuance of the \$15.0 million Novartis Note in February 2017, partially offset by the repurchase of shares from Advent in May 2017 and voluntary prepayment of the \$1.0 million promissory note payable to Pfizer Inc. in January 2017. Net cash provided by financing activities was \$11.8 million for the nine months ended September 30, 2016, which consisted primarily of net proceeds from sales of common stock pursuant to the Sales Agreement.

Contractual Obligations and Commitments

As of September 30, 2017, there have been no material changes outside the ordinary course of our business to the contractual obligations we reported in “Item 7 – Management’s Discussion and Analysis of Financial Condition and Results of Operations – Contractual Obligations and Commitments” in our annual report on Form 10-K for the year ended December 31, 2016 filed with the SEC on March 16, 2017.

Off-Balance Sheet Arrangements

We do not have any off-balance sheet arrangements (as defined by applicable regulations of the SEC) that are reasonably likely to have a current or future material effect on our financial condition, results of operations, liquidity, capital expenditures or capital resources.

ITEM 3. QUANTITATIVE AND QUALITATIVE DISCLOSURES ABOUT MARKET RISK

As of September 30, 2017, there have been no material changes in our market risk from that described in “Item 7A. Quantitative and Qualitative Disclosures About Market Risk” in our annual report on Form 10-K for the year ended December 31, 2016 filed with the Securities and Exchange Commission on March 16, 2017.

ITEM 4. CONTROLS AND PROCEDURES

Conclusion Regarding the Effectiveness of Disclosure Controls and Procedures

Our management, with the participation of our principal executive officer and principal financial officer, has evaluated the effectiveness of our disclosure controls and procedures as defined in Rules 13a-15(e) and 15d-15(e) under the Securities Exchange Act of 1934, as amended, as of the end of the period covered by this quarterly report on Form 10-Q. Based on such evaluation, our principal executive officer and principal financial officer have concluded that, as of such date, our disclosure controls and procedures were effective.

Inherent Limitations of Disclosure Controls and Procedures and Internal Control Over Financial Reporting

Our management, including our principal executive officer and our principal financial officer, does not expect that our disclosure controls and procedures or our internal control over financial reporting will prevent all error and all fraud. A control system, no matter how well conceived and operated, can provide only reasonable, not absolute, assurance that the objectives of the control system are met. Because of the inherent limitations in all control systems, no evaluation of controls can provide absolute assurance that all control issues and instances of fraud, if any, within our company have been detected. These inherent limitations include the realities that judgments in decision-making can be faulty and that breakdowns can occur because of a simple error or mistake. Additionally, controls can be circumvented by the individual acts of some persons, by collusion of two or more people, or by management override of the control. The design of any system of controls also is based in part upon certain assumptions about the likelihood of future events, and there can be no assurance that any design will succeed in achieving its stated goals under all potential future conditions. Over time, controls may become inadequate because of changes in conditions, or the degree of compliance with the policies or procedures may deteriorate. Because of the inherent limitations in a cost-effective control system, misstatements due to error or fraud may occur and not be detected.

Changes in Internal Control Over Financial Reporting

There has been no change in our internal control over financial reporting during the quarter ended September 30, 2017, that has materially affected, or is reasonably likely to materially affect, our internal control over financial reporting.

PART II. OTHER INFORMATION

ITEM 1. LEGAL PROCEEDINGS

We are currently not a party to any material legal proceedings.

ITEM 1A. RISK FACTORS

There have been no material changes to the risk factors included in “Item 1A. Risk Factors” in our annual report on Form 10-K for the year ended December 31, 2016 filed with the Securities and Exchange Commission on March 16, 2017, other than the risk factors below.

We may not be able to obtain orphan drug exclusivity for emricasan or IDN-7314 for any indication.

In the United States, under the Orphan Drug Act, the U.S. Food and Drug Administration, or the FDA, may grant orphan designation to a drug or biological product intended to treat a rare disease or condition. Such diseases and conditions are those that affect fewer than 200,000 individuals in the United States, or if they affect more than 200,000 individuals in the United States, there is no reasonable expectation that the cost of developing and making a drug product available in the United States for these types of diseases or conditions will be recovered from sales of the product. Orphan Drug Designation must be requested before submitting an NDA. If the FDA grants Orphan Drug Designation, the identity of the therapeutic agent and its potential orphan use are disclosed publicly by that agency. Orphan Drug Designation does not convey any advantage in or shorten the duration of the regulatory review and approval process, but it can lead to financial incentives, such as opportunities for grant funding toward clinical trial costs, tax advantages and user-fee waivers.

If a drug that has orphan designation subsequently receives the first FDA approval for the disease or condition for which it has such designation, the drug is entitled to orphan drug marketing exclusivity for a period of seven years. Orphan drug marketing exclusivity generally prevents the FDA from approving another application, including a full NDA, to market the same drug or biological product for the same indication for seven years, except in limited circumstances, including if the FDA concludes that the later drug is safer, more effective or makes a major contribution to patient care. For purposes of small molecule drugs, the FDA defines “same drug” as a drug that contains the same active chemical entity and is intended for the same use as the drug in question. A designated orphan drug may not receive orphan drug marketing exclusivity if it is approved for a use that is broader than the indication for which it received orphan designation. Orphan drug marketing exclusivity rights in the United States may be lost if the FDA later determines that the request for designation was materially defective or if the manufacturer is unable to assure sufficient quantity of the drug to meet the needs of patients with the rare disease or condition.

The criteria for designating an orphan medicinal product in the European Union, or the EU, are similar in principle to those in the United States. Under Article 3 of Regulation (EC) 141/2000, a medicinal product may be designated as orphan if (1) it is intended for the diagnosis, prevention or treatment of a life-threatening or chronically debilitating condition; (2) either (a) such condition affects no more than five in 10,000 persons in the EU when the application is made, or (b) the product, without the benefits derived from orphan status, would not generate sufficient return in the EU to justify investment; and (3) there exists no satisfactory method of diagnosis, prevention or treatment of such condition authorized for marketing in the EU, or if such a method exists, the product will be of significant benefit to those affected by the condition, as defined in Regulation (EC) 847/2000. Orphan medicinal products are eligible for financial incentives such as reduction of fees or fee waivers and are, upon grant of a marketing authorization, entitled to ten years of market exclusivity for the approved therapeutic indication. The application for orphan designation must be submitted before the application for marketing authorization. The applicant will receive a fee reduction for the marketing authorization application if the orphan designation has been granted, but not if the designation is still pending at the time the marketing authorization is submitted. Orphan designation does not convey any advantage in, or shorten the duration of, the regulatory review and approval process.

The ten-year market exclusivity in the EU may be reduced to six years if, at the end of the fifth year, it is established that the product no longer meets the criteria for orphan designation, for example, if the product is sufficiently profitable not to justify maintenance of market exclusivity. Additionally, marketing authorization may be granted to a similar product for the same indication at any time if:

- the second applicant can establish that its product, although similar, is safer, more effective or otherwise clinically superior;
- the applicant consents to a second orphan medicinal product application; or
- the applicant cannot supply enough orphan medicinal product.

We originally applied for Orphan Drug Designation for emricasan for the treatment of fibrosis in HCV-POLT patients in the United States and the EU. In late 2013, the FDA granted an Orphan Drug Designation for emricasan for the treatment of POLT patients with reestablished fibrosis to delay the progression to cirrhosis and end-stage liver disease. In the EU, we withdrew the application based on feedback from the applicable regulatory body that emricasan may have efficacy in fibrosis outside of the HCV-POLT patient population. In June 2017, the FDA granted Orphan Drug Designation for IDN-7314 for the treatment of primary sclerosing cholangitis, or PSC. In October 2017, the European Medicines Agency, or EMA, granted orphan designation to IDN-7314 for the treatment of PSC. We cannot assure you that we will be able to obtain orphan drug exclusivity for emricasan or IDN-7314 in any jurisdiction for the target indications in a timely manner or at all or that a competitor will not obtain orphan drug exclusivity that could block the regulatory approval of emricasan or IDN-7314 for several years. If we are unable to obtain Orphan Drug Designation in the United States or in the EU, we will not receive market exclusivity, which might affect our ability to generate sufficient revenues. If a competitor is able to obtain orphan exclusivity that would block emricasan's or IDN-7314's regulatory approval, our ability to generate revenues could be significantly reduced, which could harm our business prospects, financial condition and results of operations.

We may be unable to maintain or effectively utilize orphan drug exclusivity for emricasan or IDN-7314 for any indication.

We received Orphan Drug Designation from the FDA for emricasan for the treatment of POLT patients with reestablished fibrosis to delay the progression to cirrhosis and end-stage liver disease. We also received Orphan Drug Designation from the FDA and orphan designation from the EMA for IDN-7314 for the treatment of PSC. We may be unable to obtain regulatory approval for emricasan or IDN-7314 for these orphan populations or any other orphan population, or we may be unable to successfully commercialize emricasan or IDN-7314 for such orphan populations due to risks that include:

- the orphan patient populations may change in size;
- there may be changes in the treatment options for patients that may provide alternative treatments to emricasan or IDN-7314;
- the development costs may be greater than projected revenue of drug sales for the orphan indications;
- the FDA may disagree with the design or implementation of our clinical trials;
- there may be difficulties in enrolling patients for clinical trials;
- emricasan or IDN-7314 may not prove to be efficacious in the orphan patient populations;
- clinical trial results may not meet the level of statistical significance required by the FDA or the EMA; and
- emricasan or IDN-7314 may not have a favorable risk/benefit assessment in the orphan indication.

If we are unable to obtain regulatory approval for emricasan or IDN-7314 for any orphan population or are unable to successfully commercialize emricasan or IDN-7314 for such orphan population, it could harm our business prospects, financial condition and results of operations.

We entered into the Collaboration Agreement with Novartis, and we may form or seek additional strategic alliances or collaborations in the future. Such alliances and collaborations may inhibit future opportunities, and we may not realize the benefits of such collaborations or alliances.

We entered into the Collaboration Agreement with Novartis for the development of emricasan, and we may form or seek strategic alliances, joint ventures or collaborations or enter into licensing arrangements with other third parties that we believe will complement or augment our development and commercialization efforts with respect to future product candidates that we may develop. In connection with entering into the Collaboration Agreement, we incurred non-recurring and other charges and issued a convertible note in the amount of \$15.0 million. In addition, for the period from the execution date of the Collaboration Agreement until the earlier of five years after the first commercial sale of an emricasan product in the United States or major European market or ten years from the execution date of the Collaboration Agreement, we have agreed not to develop in any pivotal registration clinical trials or commercialize any pan-caspase inhibitors in liver disease. Further, Novartis will have a right of first negotiation prior to any offer by us to any third party for future pan-caspase inhibitors that we may develop or acquire for the treatment of liver diseases or for certain retained pan-caspase inhibitors, provided that any license or collaboration that we enter into or propose to enter into must be on terms and conditions in the aggregate no more favorable to such third party than those last offered to Novartis. These provisions and similar provisions in agreements we may enter into in the future may inhibit our ability to develop and commercialize other pan-caspase inhibitors, including IDN-7314, or enter into future alliances or collaborations to develop or commercialize other pan-caspase inhibitors, including IDN-7314.

Future efforts for additional alliances or collaborations may also require us to incur non-recurring and other charges, increase our near- and long-term expenditures, issue securities that dilute our existing stockholders or disrupt our management and business. In addition, we face significant competition in seeking appropriate strategic partners, and the negotiation process is time-consuming and complex. Furthermore, we may not be able to realize the benefit of such transactions if we are unable to successfully integrate them with our existing operations and company culture. We cannot be certain that, following a strategic transaction or license, we will achieve the revenues or specific net income that justifies such transaction.

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

Unregistered Sales of Equity Securities

None.

Use of Proceeds

None.

Issuer Purchases of Equity Securities

None.

ITEM 3. DEFAULTS UPON SENIOR SECURITIES

None.

ITEM 4. MINE SAFETY DISCLOSURES

Not applicable.

ITEM 5. OTHER INFORMATION

None.

ITEM 6. EXHIBITS

A list of exhibits is set forth on the Exhibit Index immediately preceding the signature page of this quarterly report on Form 10-Q and is incorporated herein by reference.

EXHIBIT INDEX

| Exhibit Number | Description |
|----------------|---|
| 3.1(1) | Amended and Restated Certificate of Incorporation |
| 3.2(1) | Amended and Restated Bylaws |
| 4.1(2) | Specimen Common Stock Certificate |
| 4.2(3) | First Amended and Restated Investor Rights Agreement, dated February 9, 2011 |
| 4.3(3) | Form of Warrant issued to investors in the Registrant's 2013 bridge financing |
| 4.4(2) | Form of Warrant issued to lenders under the Loan and Security Agreement, dated July 3, 2013, by and among the Registrant, Oxford Finance LLC, Silicon Valley Bank and the other lenders party thereto |
| 10.1(4) | Employment Agreement, dated August 31, 2017, by and between Keith W. Marshall, Ph.D. and the Registrant |
| 10.2(4) | Non-Qualified Inducement Stock Option Grant Notice and Stock Option Agreement, dated August 31, 2017, by and between Keith W. Marshall, Ph.D. and the Registrant |
| 23.1 | Consent of Ernst & Young LLP, Independent Registered Public Accounting Firm |
| 31.1 | Certification of Principal Executive Officer pursuant to Rules 13a-14 and 15d-14 promulgated pursuant to the Securities Exchange Act of 1934, as amended |
| 31.2 | Certification of Principal Financial Officer pursuant to Rules 13a-14 and 15d-14 promulgated pursuant to the Securities Exchange Act of 1934, as amended |
| 32.1* | Certification of Principal Executive Officer pursuant to Section 906 of the Sarbanes-Oxley Act of 2002 |
| 32.2* | Certification of Principal Financial Officer pursuant to Section 906 of the Sarbanes-Oxley Act of 2002 |
| 101.INS | XBRL Instance Document |
| 101.SCH | XBRL Taxonomy Extension Schema Document |
| 101.CAL | XBRL Taxonomy Extension Calculation Linkbase Document |
| 101.DEF | XBRL Taxonomy Extension Definition Linkbase Document |
| 101.LAB | XBRL Taxonomy Extension Label Linkbase Document |
| 101.PRE | XBRL Taxonomy Extension Presentation Linkbase Document |

(1) Incorporated by reference to the Registrant's Current Report on Form 8-K, filed with the SEC on August 1, 2013.

(2) Incorporated by reference to Amendment No. 2 to the Registrant's Registration Statement on Form S-1 (Registration No. 333-189305), filed with the SEC on July 8, 2013.

(3) Incorporated by reference to the Registrant's Registration Statement on Form S-1 (Registration No. 333-189305), filed with the SEC on June 14, 2013.

(4) Incorporated by reference to the Registrant's Current Report on Form 8-K, filed with the SEC on September 1, 2017.

* This certification is being furnished solely to accompany this quarterly report pursuant to 18 U.S.C. Section 1350, and is not being filed for purposes of Section 18 of the Securities Exchange Act of 1934, as amended, and is not to be incorporated by reference into any filing of the Registrant, whether made before or after the date hereof, regardless of any general incorporation language in such filing.

SIGNATURES

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

CONATUS PHARMACEUTICALS INC.

Date: November 2, 2017

/s/ Steven J. Mento, Ph.D.
Steven J. Mento, Ph.D.
President and Chief Executive Officer
(principal executive officer)

Date: November 2, 2017

/s/ Keith W. Marshall, Ph.D.
Keith W. Marshall, Ph.D.
Executive Vice President, Chief Operating Officer and Chief
Financial Officer
(principal financial officer)

CONSENT OF INDEPENDENT REGISTERED PUBLIC ACCOUNTING FIRM

We consent to the reference to our firm under the caption "Experts" in the Registration Statement (Form S-3 No. 333-220014) and related Prospectus of Conatus Pharmaceuticals Inc. dated August 17, 2017 for the registration of up to \$100,000,000 in aggregate principal amount of its common stock, preferred stock, debt securities, warrants and/or units and to the incorporation by reference therein of our report dated March 16, 2017, with respect to the financial statements of Conatus Pharmaceuticals Inc. included in its Annual Report (Form 10-K) for the year ended December 31, 2016, filed with the Securities and Exchange Commission.

/s/ Ernst & Young LLP

San Diego, California
November 2, 2017

CERTIFICATION OF PRINCIPAL EXECUTIVE OFFICER
PURSUANT TO SECTION 302 OF THE SARBANES-OXLEY ACT OF 2002

I, Steven J. Mento, Ph.D., certify that:

1. I have reviewed this quarterly report on Form 10-Q of Conatus Pharmaceuticals Inc.;
2. Based on my knowledge, this 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 15d-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 subsidiaries, 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 (the registrant's fourth fiscal quarter in the case of an annual report) 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 the 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: November 2, 2017

/s/ Steven J. Mento, Ph.D.

Steven J. Mento, Ph.D.
President and Chief Executive Officer
(principal executive officer)

CERTIFICATION OF PRINCIPAL FINANCIAL OFFICER
PURSUANT TO SECTION 302 OF THE SARBANES-OXLEY ACT OF 2002

I, Keith W. Marshall, Ph.D., certify that:

1. I have reviewed this quarterly report on Form 10-Q of Conatus Pharmaceuticals Inc.;
2. Based on my knowledge, this 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 15d-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 subsidiaries, 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 (the registrant's fourth fiscal quarter in the case of an annual report) 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 the 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: November 2, 2017

/s/ Keith W. Marshall, Ph.D.

Keith W. Marshall, Ph.D.
Executive Vice President, Chief Operating Officer and Chief
Financial Officer
(principal financial officer)

CERTIFICATION PURSUANT TO 18 U.S.C. SECTION 1350,
AS ADOPTED PURSUANT TO
SECTION 906 OF THE SARBANES-OXLEY ACT OF 2002

In connection with the quarterly report of Conatus Pharmaceuticals Inc. (the "Company") on Form 10-Q for the quarter ended September 30, 2017, as filed with the Securities and Exchange Commission on the date hereof (the "Report"), I, Steven J. Mento, Ph.D., President and Chief Executive Officer of 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 Report fully complies with the requirements of Section 13(a) or 15(d), as applicable, 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: November 2, 2017

/s/ Steven J. Mento, Ph.D.

Steven J. Mento, Ph.D.
President and Chief Executive Officer
(principal executive officer)

The foregoing certification is being furnished solely pursuant to 18 U.S.C. Section 1350 and is not being filed as part of the Report or as a separate disclosure document.

CERTIFICATION PURSUANT TO 18 U.S.C. SECTION 1350,
AS ADOPTED PURSUANT TO
SECTION 906 OF THE SARBANES-OXLEY ACT OF 2002

In connection with the quarterly report of Conatus Pharmaceuticals Inc. (the "Company") on Form 10-Q for the quarter ended September 30, 2017, as filed with the Securities and Exchange Commission on the date hereof (the "Report"), I, Keith W. Marshall, Ph.D., Executive Vice President, Chief Operating Officer and Chief Financial Officer of 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 Report fully complies with the requirements of Section 13(a) or 15(d), as applicable, 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: November 2, 2017

/s/ Keith W. Marshall, Ph.D.

Keith W. Marshall, Ph.D.

Executive Vice President, Chief Operating Officer and Chief
Financial Officer

(principal financial officer)

The foregoing certification is being furnished solely pursuant to 18 U.S.C. Section 1350 and is not being filed as part of the Report or as a separate disclosure document.

