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UNITED STATES
SECURITIES AND EXCHANGE COMMISSION
WASHINGTON, D.C. 20549

FORM 10-Q

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

For the quarterly period ended September 30, 2018

OR

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

For the transition period from to

Commission File Number: 001-37926

RA PHARMACEUTICALS, INC.
(Exact name of registrant as specified in its charter)

Delaware	**26-2908274**
(State or other jurisdiction of incorporation or organization)	(I.R.S. Employer Identification No.)
87 Cambridge Park Drive Cambridge, MA	**02140**
(Address of principal executive offices)	(Zip code)

617-401-4060
(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 every Interactive Data File required to be submitted 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 such files). Yes ☒ No ☐

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

Large accelerated filer ☐ Accelerated filer ☒ Non-accelerated filer ☐ Smaller reporting company ☒ Emerging growth company ☒

If an emerging growth company, indicate by check mark if the registrant has elected not to use the extended transition period for complying with any new or revised financial accounting standards 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 ☒

The number of shares outstanding of the registrant's common stock as of October 26, 2018 was 32,398,026.

NOTE REGARDING FORWARD-LOOKING STATEMENTS

This Quarterly Report on Form 10-Q contains "forward-looking statements" within the meaning of Section 27A of the Securities Act of 1933, as amended (the "Securities Act"), and Section 21E of the Securities Exchange Act of 1934, as amended (the "Exchange Act"). We have based these forward-looking statements largely on our current expectations and projections about future events and financial trends affecting the financial condition of our business. Forward-looking statements should not be read as a guarantee of future performance or results, and will not necessarily be accurate indications of the times at, or by, which such performance or results will be achieved.

Forward-looking statements include all statements that are not historical facts. In some cases, you can identify forward-looking statements by terms such as "may," "might," "will," "objective," "intend," "should," "could," "can," "would," "expect," "believe," "anticipate," "project," "target," "design," "estimate," "predict," "potential," "plan" or the negative of these terms, and similar expressions and comparable terminology intended to identify forward-looking statements. The forward-looking statements in this Quarterly Report on Form 10-Q include, without limitation, expectations regarding the sufficiency of our cash and cash equivalents; our anticipated capital requirements and uses of cash; safety, efficacy and regulatory and clinical progress of our product candidates, including zilucoplan; trial design, timeline and enrollment of our ongoing and planned clinical programs; timing of the release of clinical trial data; and our collaboration with Merck & Co., Inc., including without limitation potential milestone payments thereunder. These statements reflect our current views with respect to future events and are based on assumptions and subject to risks and uncertainties and other important factors that could cause our actual results, performance or achievements to differ materially from those expressed or implied by the forward-looking statements, including, but not limited to:

- the initiation, timing, progress and results of our research and development programs and future preclinical and clinical studies;
- the risk that topline data from our Phase 2 clinical program in paroxysmal nocturnal hemoglobinuria ("PNH") may not be indicative of final study results or results from future trials;
- our ability to advance any product candidates into, and successfully complete, clinical studies and obtain regulatory approval for them;
- our ability to identify additional product candidates using our Extreme Diversity™ platform;
- the timing or likelihood of regulatory filings and approvals;
- our ability to commercialize, market and manufacture our product candidates, if approved;
- the pricing and reimbursement of our product candidates, if approved;
- the rate and degree of market acceptance and clinical utility of any products for which we receive marketing approval;
- the implementation of our strategic plans for our business, product candidates and technology;
- the scope of protection we are able to establish and maintain for intellectual property rights covering our product candidates and technology;
- our ability to maintain and establish collaborations;
- our financial performance;
- developments relating to our competitors and our industry, including the impact of government regulation; and
- other risks and uncertainties, including the important factors listed under Item 1A, "Risk Factors" and elsewhere in our Annual Report on Form 10-K for the year ended December 31, 2017, as supplemented by our subsequent filings with the Securities and Exchange Commission ("SEC").

Given these uncertainties, you should not place undue reliance on these forward-looking statements. These forward-looking statements represent our estimates and assumptions only as of the date of this Quarterly Report on Form 10-Q and, except as required by law, we undertake no obligation to update or revise publicly any forward looking-statements, whether as a result of new information, future events or otherwise after the date of this Quarterly Report on Form 10-Q. We qualify all of our forward-looking statements by these cautionary statements.

NOTE REGARDING TRADEMARKS

All brand names or trademarks appearing in this report are the property of their respective holders. Unless the context requires otherwise, references in this report to the "Company," "we," "us," and "our" refer to Ra Pharmaceuticals, Inc.

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PART I: FINANCIAL INFORMATION

Item 1. Financial Statements

RA PHARMACEUTICALS, INC.
CONDENSED CONSOLIDATED BALANCE SHEETS
(UNAUDITED)
(In thousands, except per share data)

	September 30, 2018	December 31, 2017
Assets		
Current assets:		
Cash and cash equivalents	$ 81,059	$ 70,381
Prepaid expenses and other current assets	2,502	2,496
Total current assets	83,561	72,877
Property and equipment, net	5,394	5,606
Goodwill	183	183
Intangible assets, net	147	197
Restricted cash	1,334	1,334
Total assets	$ 90,619	$ 80,197
Liabilities and Stockholders' Equity		
Current liabilities:		
Accounts payable	$ 2,899	$ 4,348
Accrued expenses	4,749	3,937
Deferred rent	469	329
Total current liabilities	8,117	8,614
Deferred rent, net of current portion	2,003	2,359
Deferred tax liabilities	40	40
Total liabilities	10,160	11,013
Commitments and contingencies		
Stockholders' equity:		
Preferred stock, $0.001 par value; 5,000 shares authorized; no shares issued and outstanding	—	—
Common stock, $0.001 par value; 150,000 shares authorized; 32,388 and 22,626 shares issued and outstanding as of September 30, 2018 and December 31, 2017, respectively	32	23
Additional paid-in capital	252,388	192,375
Accumulated deficit	(171,961)	(123,214)
Total stockholders' equity	80,459	69,184
Total liabilities and stockholders' equity	$ 90,619	$ 80,197

See Notes to Unaudited Condensed Consolidated Financial Statements.

RA PHARMACEUTICALS, INC.
CONDENSED CONSOLIDATED STATEMENTS OF OPERATIONS
(UNAUDITED)
(in thousands, except per share data)

	Three Months Ended September 30,		Nine Months Ended September 30,	
	2018	2017	2018	2017
Operating expenses:				
Research and development	$ 13,375	$ 13,130	$ 39,092	$ 32,606
General and administrative	3,504	2,284	10,637	7,101
Total operating expenses	16,879	15,414	49,729	39,707
Loss from operations	(16,879)	(15,414)	(49,729)	(39,707)
Other income (expense), net	375	139	981	409
Net loss	$ (16,504)	$ (15,275)	$ (48,748)	$ (39,298)
Net loss per common share — basic and diluted	$ (0.51)	$ (0.68)	$ (1.60)	$ (1.74)
Weighted average number of common shares outstanding — basic and diluted	32,349	22,614	30,652	22,579

See Notes to Unaudited Condensed Consolidated Financial Statements.

RA PHARMACEUTICALS, INC.
CONDENSED CONSOLIDATED STATEMENTS OF CASH FLOWS
(UNAUDITED)
(in thousands)

	Nine Months Ended September 30,	
	2018	**2017**
Cash flows from operating activities		
Net loss	$ (48,748)	$ (39,298)
Adjustments to reconcile net loss to net cash used in operating activities:		
Depreciation and amortization	1,186	1,121
Stock-based compensation	5,553	3,847
Other, net	—	8
Changes in operating assets and liabilities:		
Prepaid expenses and other current assets	110	430
Accounts payable and accrued expenses	(757)	963
Other, net	(216)	(199)
Net cash used in operating activities	(42,872)	(33,128)
Cash flows from investing activities		
Purchase of property and equipment	(915)	(1,390)
Net cash used in investing activities	(915)	(1,390)
Cash flows from financing activities		
Proceeds from common stock offering, net of underwriter discounts	54,482	—
Payment of common stock offering costs	(371)	—
Proceeds from disgorgement of stockholder's short-swing profits	—	670
Proceeds from exercises of stock options	426	244
Other, net	(72)	(117)
Net cash provided by financing activities	54,465	797
Net increase (decrease) in cash, cash equivalents and restricted cash	10,678	(33,721)
Cash, cash equivalents and restricted cash, beginning of period	71,715	119,146
Cash, cash equivalents and restricted cash, end of period	$ 82,393	$ 85,425

See Notes to Unaudited Condensed Consolidated Financial Statements.

RA PHARMACEUTICALS, INC.
NOTES TO UNAUDITED CONDENSED CONSOLIDATED FINANCIAL STATEMENTS

1. Nature of Business and Basis of Presentation

The accompanying condensed consolidated financial statements are unaudited and have been prepared by Ra Pharmaceuticals, Inc. (the "Company") in accordance with accounting principles generally accepted in the United States ("U.S. GAAP") and pursuant to the rules and regulations of the Securities and Exchange Commission. The year-end condensed consolidated balance sheet data was derived from the Company's audited financial statements, but does not include all disclosures required by U.S. GAAP. These condensed consolidated financial statements should be read in conjunction with the Company's Annual Report on Form 10-K for the year ended December 31, 2017. The condensed consolidated financial statements, in the opinion of management, reflect all normal and recurring adjustments necessary for a fair statement of the Company's financial position and results of operations.

Description of Business

The Company is a clinical-stage biopharmaceutical company using its proprietary peptide chemistry platform to create novel therapeutics to treat life-threatening diseases that are caused by excessive or uncontrolled activation of the complement system, an essential component of the body's innate immune system. The Company's lead product candidate, zilucoplan (RA101495 SC), is being developed as a convenient self-administered subcutaneous ("SC") injection, which is an injection into the tissue under the skin, for the treatment of various complement-mediated diseases, including paroxysmal nocturnal hemoglobinuria ("PNH"), generalized myasthenia gravis ("gMG") and multiple complement-mediated renal disorders. Additionally, the Company is pursuing discovery and preclinical programs targeting selective inhibition of other uncontrolled complement pathway factors to treat a variety of neurologic, renal and inflammatory diseases. In addition to its focus on developing novel therapeutics to treat complement-mediated diseases, the Company has validated its Extreme Diversity platform by successfully identifying and delivering orally-available cyclic peptides for a non-complement cardiovascular target with a large market opportunity in a collaboration with Merck & Co., Inc. ("Merck").

The Company was incorporated in Delaware on June 27, 2008, and is located in Cambridge, Massachusetts. During 2011, the Company acquired Cosmix Verwaltungs GmbH ("Cosmix"), organized in Germany. In January 2016, the Company formed a wholly-owned subsidiary organized in the United Kingdom ("UK"), Ra Europe Limited, for the purpose of conducting clinical trials in Europe and the UK.

The Company is subject to risks common to other life science companies in the development stage, including, but not limited to, uncertainty of product development and commercialization, lack of marketing and sales history, development by its competitors of new technological innovations, dependence on key personnel, market acceptance of products, product liability, protection of proprietary technology, ability to raise additional financing, and compliance with Food and Drug Administration and other government regulations. If the Company does not successfully commercialize any of its product candidates, it will be unable to generate recurring product revenue or achieve profitability. If the Company is unable to raise capital when needed or on attractive terms, it would be forced to delay, reduce, eliminate or out-license certain of its research and development programs or future commercialization efforts.

Since inception, the Company has generated an accumulated deficit of $172.0 million as of September 30, 2018 and has devoted substantially all of its efforts to research and development, business planning, acquiring operating assets, seeking protection for its technology and product candidates, and raising capital.

Public Offerings

On October 31, 2016, the Company completed an initial public offering ("IPO"), in which the Company issued and sold 7,049,230 shares of common stock at a public offering price of $13.00 per share, resulting in net proceeds of $82.8 million after deducting $6.4 million of underwriting discounts and commissions and offering costs of $2.4 million. On November 29, 2016, the Company completed the sale of an additional 1,057,385 shares of common stock to the underwriters under the underwriters' option in the IPO to purchase additional shares at the public offering price of $13.00 per share, resulting in net proceeds of $12.8 million after deducting underwriting discounts and commissions of $1.0 million. The shares began trading on the Nasdaq Global Market on October 26, 2016.

In February 2018, the Company completed a follow-on public offering of 9,660,000 shares of common stock, including the full exercise of the underwriter's over-allotment option of 1,260,000 shares, at $6.00 per share and received aggregate net proceeds of $54.1 million, after deducting $3.5 million of underwriting discounts and commissions and approximately $0.4 million of offering expenses.

In May 2018, the Company entered into a sales agreement (the "Sales Agreement") with Stifel, Nicolaus & Company, Incorporated ("Stifel") pursuant to which the Company may sell from time to time, at its option, up to an aggregate of $50.0 million of

shares of its common stock through Stifel, as sales agent. Sales of the common stock, if any, will be made by methods deemed to be "at the market offerings." The Company has agreed to pay Stifel a commission of up to 3% of the gross proceeds from the sale of the shares of its common stock, if any. The Sales Agreement will terminate upon the earliest of: (a) the sale of $50.0 million of shares of the Company's common stock or (b) the termination of the Sales Agreement by the Company or Stifel. As of September 30, 2018, the Company has not sold any shares of common stock under this program.

Principles of Consolidation

The Company's condensed consolidated financial statements reflect its financial statements and those of its subsidiaries in which the Company holds a controlling financial interest, including Cosmix and Ra Europe Limited. Intercompany balances and transactions are eliminated in consolidation.

Use of Estimates

The preparation of condensed consolidated financial statements in accordance with U.S. GAAP requires that the Company make estimates and judgments that may affect the reported amounts of assets, liabilities, revenues, expenses and related disclosure of contingent assets and liabilities. On an on-going basis, the Company evaluates its estimates, judgments and methodologies. The Company bases its estimates on historical experience and on various other assumptions that are believed to be reasonable, the results of which form the basis for making judgments about the carrying values of assets and liabilities. Actual results may differ from these estimates under different assumptions or conditions. Changes in estimates are reflected in reported results in the period in which they become known.

Summary of Significant Accounting Policies

The Company's significant accounting policies are described in Note 2, "Summary of Significant Accounting Policies," in the Company's Annual Report on Form 10-K for the year ended December 31, 2017, except as described below.

Revenue Recognition

Effective January 1, 2018, the Company adopted Accounting Standards Codification ("ASC") Topic 606, "*Revenues from Contracts with Customers*" ("ASC 606"). This standard applies to all contracts with customers, except for contracts that are within the scope of other standards, such as leases, insurance, collaboration arrangements and financial instruments.

The Company has derived all of its revenue to date from its collaboration agreement with Merck (the "Merck Agreement"). See Note 4, *"Revenue Recognition."* The Merck Agreement is accounted for under ASC 606 since it does not represent a collaborative arrangement under ASC 808, "*Collaborative Arrangements,"* as the Company is not an active participant and is not exposed to significant risks and rewards of the arrangement.

The terms of the Merck Agreement contain multiple promised goods and services, which include licenses, research and development activities and participation on the joint steering committee. Payments under the agreement include: (i) an upfront nonrefundable license fee; (ii) payments for research and development services performed by the Company, including reimbursement for certain lab supplies and reagents; (iii) payments based upon the achievement of certain development (pre-clinical and clinical), regulatory and commercial milestones; and (iv) royalties on net product sales, if any.

Under the new revenue standard, the Company recognizes revenue when its customer obtains control of promised goods or services, in an amount that reflects the consideration that the Company expects to receive in exchange for those goods or services. The Company recognizes revenue following the five-step model prescribed under ASC 606:

- Identification of the contract with the customer;
- Identification of the performance obligations;
- Determination of the transaction price, including the constraint on variable consideration;
- Allocation of the transaction price to the performance obligations in the contract; and
- Recognition of revenue when (or as) the Company satisfies each performance obligation.

In order to account for contracts with customers, such as the Merck Agreement, the Company identifies the promised goods or services in the contract and evaluates whether such promised goods or services represent performance obligations. The Company accounts for those components as separate performance obligations when the following criteria are met:

- the customer can benefit from the good or service either on its own or together with other resources that are readily available to the customer, and
- the Company's promise to transfer the good or service to the customer is separately identifiable from other promises in the contract.

This evaluation requires subjective determinations and requires the Company to make judgments about the promised goods and services and whether such goods and services are separable from the other aspects of the contractual relationship. In determining the performance obligations, the Company evaluates certain criteria, including whether the promised good or service is capable of being distinct and whether such good or service is distinct within the context of the contract, based on consideration of the relevant facts and circumstances for each arrangement. Factors considered in this determination include the research, manufacturing and commercialization capabilities of the partner; the availability of research and manufacturing expertise in the general marketplace; and the level of integration, interrelation, and interdependence among the promises to transfer goods or services.

The transaction price is allocated among the performance obligations using the relative selling price method and the applicable revenue recognition criteria are applied to each of the separate performance obligations. At contract inception, the Company determines the standalone selling price for each performance obligation identified in the contract. If an observable price of the promised good or service sold separately is not readily available, the Company utilizes assumptions that require judgment to estimate the standalone selling price, which may include development timelines, probabilities of technical and regulatory success, reimbursement rates for personnel costs, forecasted revenues, potential limitations to the selling price of the product, expected technological life of the product and discount rates.

If the license to the intellectual property is determined to be distinct from the other performance obligations identified in the arrangement, the Company recognizes revenue when the license is transferred to the licensee and the licensee is able to use and benefit from the license. For licenses that are bundled with other promises, the Company utilizes judgment to assess the nature of the combined performance obligation to determine whether the combined performance obligation is satisfied over time or at a point in time and, if over time, the appropriate method of measuring progress for purposes of recognizing revenue from the combined performance obligation. The Company evaluates the measure of progress each reporting period and, if necessary, adjusts the measure of performance and related revenue recognition.

At the inception of each arrangement that includes precommercial milestone payments, the Company evaluates whether the milestones are considered probable of being reached and estimates the amount to be included in the transaction price using the most likely amount method. If it is probable that a significant cumulative revenue reversal would not occur, the associated milestone value is included in the transaction price. Milestone payments that are not within the Company's control, such as regulatory approvals, are not considered probable of being achieved until the uncertainty related to the milestone is resolved. The transaction price is then allocated to each performance obligation on a relative selling price basis, for which the Company recognizes revenue as or when the performance obligations under the contract are satisfied. At the end of each subsequent reporting period, the Company reevaluates the probability of achievement of such development milestones and any related constraint, and if necessary, adjust its estimate of the overall transaction price.

For arrangements that include sales-based royalties, including milestone payments based on the level of sales, and where the license is deemed to be the predominant item to which the royalties relate, the Company recognizes revenue at the later of: (i) when the related sales occur, or (ii) when the performance obligation to which some or all of the royalty has been allocated has been satisfied (or partially satisfied). To date, the Company has not recognized any royalty revenue resulting from the Merck Agreement.

Newly Adopted Accounting Pronouncements

In May 2014, the Financial Accounting Standards Board ("FASB") issued Accounting Standards Update ("ASU") 2014-09, "*Revenue from Contracts with Customers*." The standard, including subsequently issued amendments, replaces most existing revenue recognition guidance in U.S. GAAP and permits the use of either the retrospective or cumulative effect transition method. The standard requires an entity to recognize the amount of revenue to which it expects to be entitled for the transfer of promised goods or services to customers. The standard is effective for annual and interim periods beginning after December 15, 2017. The Company has one contract subject to the new standard, the Merck Agreement, and all performance obligations were completed upon the expiration of the research term in April 2016. See Note 4, *"Revenue Recognition."* The Company adopted the new standard on January 1, 2018 using the retrospective method. The adoption of ASU 2014-09 did not have a significant impact on the Company's consolidated financial statements for the periods presented.

Newly Issued Accounting Pronouncements

In February 2016, the FASB issued ASU 2016-02, "*Leases*." The standard is designed to increase transparency and comparability among organizations by recognizing lease assets and lease liabilities on the balance sheet and disclosing key information about leasing arrangements with a lease term of more than 12 months. In July 2018, the FASB issued ASU 2018-10 "Codification Improvements to Topic 842, Leases," to clarify application of certain aspects of the new leases standard and to remove inconsistencies within the guidance and ASU 2018-11 "Targeted Improvements," which provides for an alternate transition

method. Specifically, ASU 2018-11 allows the new lease standard to be applied as of the adoption date with a cumulative-effect adjustment to the opening balance of retained earnings rather than retroactive restatement of all periods presented. ASU 2016-02 is effective for interim and annual periods beginning after December 15, 2018. Early adoption is permitted. The Company is still evaluating the full impact this standard will have on its consolidated financial statements and related disclosures but expects to recognize substantially all of its leases on the balance sheet by recording a right-to-use asset and a corresponding lease liability. The Company is currently formalizing processes and controls to identify, classify and measure new leases in accordance with ASU 2016-02.

In June 2018, the FASB issued ASU 2018-07, "*Improvements to Nonemployee Share-Based Payment Accounting*." The standard aligns the measurement and classification guidance for share-based payments to nonemployees with the guidance for share-based payments to employees, with certain exceptions. Under the new guidance, the measurement of equity-classified nonemployee awards will be fixed at the grant date. The ASU is effective for fiscal years beginning after December 15, 2018, and interim periods within those fiscal years. Early adoption is permitted but not before an entity adopts the new revenue guidance. The Company does not expect ASU 2018-07 to have a significant impact on its financial statements.

In August 2018, the FASB issued ASU 2018-13, "*Disclosure Framework - Changes to the Disclosure Requirements for Fair Value Measurement*." The standard eliminates, adds and modifies certain disclosure requirements for fair value measurements as part of its disclosure framework project. Entities will no longer be required to disclose the amount of and reasons for transfers between Level 1 and Level 2 of the fair value hierarchy, but public companies will be required to disclose the range and weighted average used to develop significant unobservable inputs for Level 3 fair value measurements. The guidance is effective for all entities for fiscal years beginning after December 15, 2019 and for interim periods within those fiscal years, but entities are permitted to early adopt either the entire standard or only the provisions that eliminate or modify the requirements. The Company does not expect ASU 2018-13 to have a significant impact on its financial statements.

In September 2018, the FASB issued ASU 2018-15, "*Customer's Accounting for Implementation Costs Incurred in a Cloud Computing Arrangement That Is a Service Contract*." The standard requires a customer in a cloud computing arrangement that is a service contract to follow the internal-use software guidance in ASC 350-40 to determine which implementation costs to capitalize as assets or expense as incurred. Under the new guidance, capitalizable implementation costs related to a hosting arrangement that is a service contract will be amortized over the term of the hosting arrangement, beginning when the module or component of the hosting arrangement is ready for its intended use. The ASU is effective for fiscal years beginning after December 15, 2019, and interim periods within those fiscal years. Early adoption is permitted, including adoption in any interim period. Entities have the option to apply the guidance prospectively to all implementation costs incurred after the date of adoption or retrospectively. The new guidance requires entities to make certain disclosures in the interim and annual period of adoption. The Company has not yet selected a transition method and is evaluating the impact the adoption will have on its consolidated financial statements and related disclosures.

2. Supplemental Balance Sheet Information

Property and equipment, net

Property and equipment, net consists of the following (in thousands):

	September 30, 2018	December 31, 2017
Computer equipment and software	$ 53	$ 20
Furniture, fixtures and office equipment	388	378
Laboratory equipment	5,990	5,116
Leasehold improvements	3,755	3,753
	10,186	9,267
Accumulated depreciation	(4,792)	(3,661)
Property and equipment, net	$ 5,394	$ 5,606

Depreciation expense was $0.4 million for each of the three month periods ended September 30, 2018 and 2017 and $1.1 million and for each of the nine month periods ended September 30, 2018 and 2017.

Restricted cash

The Company is contingently liable under an unused letter of credit with a bank, related to the Company's facility lease. As a result, as of September 30, 2018 and December 31, 2017, the Company had restricted cash securing the letters of credit. The cash will be restricted until the termination or modification of the lease arrangement.

The following table reconciles the Company's restricted cash as of September 30, 2018 and December 31, 2017 to cash, cash equivalents and restricted cash presented in the condensed consolidated statement of cash flows (in thousands):

	September 30, 2018	December 31, 2017
Cash and cash equivalents	$ 81,059	$ 70,381
Restricted cash	1,334	1,334
Cash, cash equivalents and restricted cash	$ 82,393	$ 71,715

Accrued expenses

Accrued expenses consist of the following (in thousands):

	September 30, 2018	December 31, 2017
Payroll and employee-related costs	$ 2,078	$ 2,063
Research and development costs	2,199	1,464
Other	472	410
Total	$ 4,749	$ 3,937

3. Fair Value Measurements

The Company has certain assets recorded at fair value, which may be classified as Level 1, 2, or 3 within the fair value hierarchy:

- Level 1 - Fair values are determined utilizing prices (unadjusted) in active markets for identical assets or liabilities that the Company has the ability to access.
- Level 2 - Fair values are determined by utilizing quoted prices for identical or similar assets and liabilities in active markets or other market observable inputs such as interest rates, yield curves, and foreign currency spot rates.
- Level 3 - Prices or valuations that require inputs that are both significant to the fair value measurement and unobservable.

The fair value hierarchy level is determined by asset and liability class based on the lowest level of significant input. The observability of inputs may change for certain assets or liabilities. This condition could cause an asset or liability to be reclassified between levels. The Company recognizes transfers between levels within the fair value hierarchy, if any, at the end of each quarter. During the three and nine months ended September 30, 2018, there were no transfers between levels.

Valuation methodologies used for assets measured or disclosed at fair value are as follows:

- Cash equivalents - Valued at market prices determined through third-party pricing services.

Assets measured at fair value on a recurring basis are summarized below (in thousands):

	September 30, 2018			
	Level 1	Level 2	Level 3	Total
Cash equivalents — Money market funds	$ 81,047	$ —	$ —	$ 81,047
Total assets	$ 81,047	$ —	$ —	$ 81,047

	December 31, 2017			
	Level 1	Level 2	Level 3	Total
Cash equivalents — Money market funds	$ 70,449	$ —	$ —	$ 70,449
Total assets	$ 70,449	$ —	$ —	$ 70,449

4. Revenue Recognition

In April 2013, the Company entered into a multi-target collaboration and license agreement with Merck to use its proprietary drug discovery technology platform to identify orally available cyclic peptides for non-complement program targets nominated by Merck and provide specific research and development services. Under the contract, the Company granted Merck licenses under certain of its intellectual property rights to manufacture, develop and commercialize compounds and products directed to selected program targets. The agreement consists of a research phase, where the Company and Merck collaborated on identifying and pre-clinically developing orally available cyclic peptides suitable for further development by Merck, and a development and commercialization phase pursuant to which Merck has sole discretion and responsibility, including financial responsibility, for further development and commercialization of these peptides, on a program-by-program basis, from the collaboration. The research term ended in April 2016.

At the signing of the Merck Agreement, Merck paid an upfront nonrefundable, technology license fee of $4.5 million. In addition, the Merck Agreement provides for reimbursement of research and development services provided by the Company and includes low to mid-single digit percentage royalties on future sales, if any, and milestone payments that could total up to $65.0 million; including preclinical and clinical milestones of $20.0 million, $3.5 million of which have been received to date; regulatory milestones of $19.0 million; and commercial milestones of $26.0 million.

The Company assessed this arrangement in accordance with ASC 606 and concluded that the contract counterparty, Merck, is a customer. The Company has identified the following promised goods and services in connection with the Merck Agreement: (1) rights to use the Company's technology platform for each program target, and (2) the research and development services provided during the research term, including participation on the joint steering committee. The Company determined that the license to the Company's early stage intellectual property is not distinct from the research and development services and accounted for all promises as a single combined performance obligation. The primary factor considered in this determination included the expectation that the research and development services will involve significant further development of the initial intellectual property licensed to Merck.

At execution and the end of each reporting period, the transaction price allocated to the single performance obligation included the $4.5 million technology license fee received and the payments for research and development services expected to be received by the Company. At execution, none of the preclinical, clinical or regulatory milestones has been included in the transaction price, as all milestone amounts were fully constrained. As part of its evaluation of the constraint, the Company considered numerous factors, including that the receipt of the milestones is outside the control of the Company and contingent upon success of future preclinical and clinical studies and Merck's efforts. The $0.5 million and $3.0 million preclinical milestone payments received to date were added to the transaction price in the fourth quarter of 2013 and the second quarter of 2016, respectively, when they were considered probable of being reached. The Company recognized revenue under the Merck Agreement over time as it satisfied the combined performance obligation during the research term. The research term ended in April 2016 and the Company does not have any future performance obligations under this contract. At the end of each reporting period, the Company continues to assess the probability of achievement of the remaining preclinical, clinical or regulatory milestones and any related constraint and if necessary, adjusts its estimate of the overall transaction price. Any such adjustments to the transaction price will be recorded as revenue in the period of adjustment.

Any consideration related to the sales-based milestones (including royalties) will be recognized when the related sales occur as they were determined to relate predominantly to the licenses granted to Merck and therefore were excluded from the transaction price.

During the three and nine months ended September 30, 2018 and 2017, the Company did not recognize any revenue and had no contract assets and liabilities related to the Merck Agreement.

5. Stock-Based Compensation

The Company has stock-based compensation plans under which employees, directors and non-employees may be granted stock-based awards such as stock options, stock appreciation rights, restricted stock awards, unrestricted stock awards, restricted stock units, performance-based awards or dividend equivalent rights.

The following table provides stock-based compensation by the financial statement line item in which it is reflected (in thousands):

	Three Months Ended September 30,		Nine Months Ended September 30,	
	2018	2017	2018	2017
Research and development	$ 980	$ 922	$ 2,837	$ 2,288
General and administrative	815	675	2,716	1,559
Total	$ 1,795	$ 1,597	$ 5,553	$ 3,847

During the nine months ended September 30, 2018, the Company issued 1.0 million stock options with a per share weighted-average grant date fair value of $5.31 and 0.3 million of restricted stock units with a per share weighted-average grant date fair value of $7.18.

6. Net Loss Per Share

The Company computes basic and diluted earnings (loss) per share using a methodology that gives effect to the impact of outstanding participating securities (the "two-class method"). As the three and nine months ended September 30, 2018 and 2017 resulted in net losses, there is no income allocation required under the two-class method or dilution attributed to weighted average shares outstanding in the calculation of diluted loss per share.

The following common stock equivalents were excluded from the computation of diluted weighted average shares outstanding as their effect would be anti-dilutive (in thousands):

	As of September 30,	
	2018	2017
Stock options	3,996	3,358
Restricted stock units	307	—
Total	4,303	3,358

Item 2. Management's Discussion and Analysis of Financial Condition and Results of Operations

The following discussion should be read in conjunction with our condensed consolidated financial statements and accompanying footnotes appearing elsewhere in this Quarterly Report on Form 10-Q and our audited consolidated financial statements and related footnotes included in our Annual Report on Form 10-K for the year ended December 31, 2017. Some of the information contained in this discussion and analysis or set forth elsewhere in this Quarterly Report on Form 10-Q, including information with respect to our plans and strategy for our business and related financing, includes forward-looking statements that involve risks and uncertainties. Actual results may differ significantly from those projected in the forward-looking statements. Important factors that might cause future results to differ materially from those projected in the forward-looking statements include, but are not limited to, those set forth in Item 1A, "Risk Factors" and elsewhere in our Annual Report on Form 10-K for the year ended December 31, 2017, as supplemented by our subsequent filings with the SEC.

Overview

We are a clinical-stage biopharmaceutical company using our proprietary peptide chemistry platform to develop novel therapeutics for the treatment of serious diseases that are caused by excessive or uncontrolled activation of the complement system, a critical component of the immune system. We are developing our lead product candidate, zilucoplan (formerly RA101495 SC), a convenient self-administered subcutaneous ("SC") injection, which is an injection into the tissue under the skin, for various complement-mediated diseases, including the treatment of paroxysmal nocturnal hemoglobinuria ("PNH"), generalized myasthenia gravis ("gMG") and multiple complement-mediated renal disorders.

PNH is a rare, chronic, life-threatening, blood disorder where red blood cells are mistakenly attacked and destroyed by the complement system. In the second quarter of 2017, we initiated our Phase 2 clinical program for zilucoplan in PNH patients. The global, dose-finding, twelve-week open label Phase 2 program was designed to evaluate the safety, tolerability, preliminary efficacy, pharmacokinetics and pharmacodynamics of zilucoplan in patients with PNH. In June 2017 and December 2017, we released interim data and, in February 2018, completed dosing and announced topline data from this Phase 2 program. During the second and third quarter of 2018, we held End-of-Phase 2 discussions with the United States (U.S.) Food and Drug Administration (FDA), the Medicines and Healthcare products Regulatory Agency in the United Kingdom (MHRA) and Health Canada. In September 2018, based on feedback provided by FDA, as well as MHRA and Health Canada during our End-of-Phase 2 discussions, we announced our plan to initiate a global, pivotal, single-arm Phase 3 trial to evaluate the safety and efficacy of zilucoplan in approximately 40 treatment-naïve PNH patients. In addition, we expect to initiate a supportive trial in approximately 40 transfusion-independent patients switching from eculizumab to zilucoplan. We also plan to meet with the European Medicines Agency (EMA) in the fourth quarter of 2018 to discuss our global Phase 3 program and, pending the outcome of these discussions, anticipate initiating our Phase 3 clinical trials during the first half of 2019.

Myasthenia gravis ("MG") is a rare, complement-mediated, autoimmune disease that causes weakness in the skeletal muscles. Patients with MG present with muscle weakness that characteristically becomes increasingly severe with repeated use and recovers with rest. Muscle weakness can be localized to specific muscles, such as those responsible for eye movements, but often progresses to affect a broader range, including head, limb and respiratory muscles. This is often described as the generalized, or severe, form of the disease. We initiated a Phase 2 clinical trial with zilucoplan for gMG in the fourth quarter of 2017. In August 2018, we announced the early completion and enrollment of 44 patients in our Phase 2 trial in gMG surpassing our original enrollment target of 36 patients. In November 2018, we announced completion of dosing of all patients and expect to report topline data around year-end 2018.

We are also developing zilucoplan to treat other debilitating complement-mediated renal disorders and in January 2018, initiated a Phase 1b pharmacokinetic (PK) clinical trial evaluating zilucoplan in patients with renal impairment. During the second quarter of 2018, we completed dosing of all subjects and in September 2018 announced positive results from our Phase 1b PK clinical trial.

Additionally, we are pursuing discovery and preclinical programs targeting selective inhibition of other uncontrolled complement pathway factors to treat a variety of neurologic, renal and inflammatory diseases. In addition to our focus on developing novel therapeutics to treat complement-mediated diseases, we have validated our Extreme Diversity platform by successfully identifying and delivering orally-available cyclic peptides for a non-complement cardiovascular target with a large market opportunity in a collaboration with Merck & Co., Inc ("Merck").

Zilucoplan in PNH

On February 12, 2018, we announced the completion of dosing and topline data for our global Phase 2 clinical program in PNH. The data cut-off date for the topline data published on February 12, 2018 was February 7, 2018. The global, dose-finding, twelve-week open label Phase 2 program was designed to evaluate the safety, tolerability, preliminary efficacy, pharmacokinetics and pharmacodynamics of zilucoplan in patients with PNH.

We enrolled a total of 29 patients across three cohorts in the Phase 2 clinical program in PNH. The first cohort enrolled ten patients who had not previously been treated with eculizumab. We refer to these patients as eculizumab naïve patients. The second cohort enrolled 16 patients, who, prior to the trial, were treated with an eculizumab regimen and, in connection with the trial, were switched over to treatment with zilucoplan. We refer to these patients as eculizumab switch patients. The third cohort enrolled three patients. These patients are U.S. based and were inadequate responders to eculizumab and who were also switching over to zilucoplan. We refer to these patients as eculizumab inadequate responders.

The primary efficacy endpoint was the change in lactate dehydrogenase ("LDH") levels, from baseline to the mean level from week 6 to week 12 of the trial. A total of 21 patients completed the initial 12-week dosing period, ten naïve patients, eight switch patients and three inadequate responders.

Eculizumab Naïve Cohort

All ten eculizumab naïve patients successfully completed 12 weeks of dosing. Zilucoplan met the primary endpoint, demonstrating a rapid, robust, and sustained reduction in LDH levels from baseline to the mean of Weeks 6-12 ($p=0.002$) and near-complete suppression of complement activity. Fifty percent of eculizumab naïve patients (3/6) who were transfusion-dependent prior to enrollment remained transfusion-free while on study. Meaningful improvements in standard measures of quality of life, as shown by the Functional Assessment of Chronic Illness Therapy ("FACIT") fatigue score were observed, as well as a high level of patient satisfaction with SC self-administration based on patient surveys. To date, eight of the 10 patients in the naïve cohort continue in the long-term extension study, with the longest treated patients (n=2) dosed through more than 60 weeks. The clinically meaningful reduction in mean LDH observed during the 12-week Phase 2 dosing period has been sustained in the long-term extension study.

Eculizumab Switch Cohort

The eculizumab switch cohort enrolled 16 patients, comprised of five patients who were transfusion-independent at baseline (before switching to zilucoplan) and 11 who were transfusion-dependent at baseline. As noted in February 2018, the topline results from the completed switch cohort show that the LDH response observed in switch patients was bimodal based on prior transfusion requirements on eculizumab. In transfusion-independent patients from this cohort (n=5), a population segment representing the majority of patients currently treated with eculizumab (approximately 80% of patients on long-term eculizumab therapy), switching to zilucoplan resulted in overall stable mean LDH levels with only one patient (1/5) withdrawing early due to breakthrough hemolysis and reverting to eculizumab without complications. Among patients who were transfusion-dependent at baseline (n=11), breakthrough hemolysis occurred after switching in seven patients (7/11), who all reverted to eculizumab treatment without complications. Overall, eight patients in the switch cohort (four of the transfusion-dependent and four of the transfusion-independent patients) completed the 12-week treatment phase and, to date, three patients continue in the long-term extension study. Of note, our Phase 2 clinical trial of zilucoplan enrolled a disproportionately larger percentage of transfusion-dependent PNH patients (69%) compared to estimates of the percentage of transfusion-dependent PNH patients on long-term eculizumab therapy in the real world (approximately 20%).

Eculizumab Inadequate Responder Cohort

In our U.S.-based cohort of patients who were inadequate responders to eculizumab and have a history of elevated LDH levels, all three patients (two transfusion-independent, one transfusion-dependent) have completed 12 weeks of dosing and maintained stable mean LDH levels. To date, two of the three patients in the inadequate responder cohort continue in the long-term extension study.

Pooled Data (Eculizumab Switch and Inadequate Responder Cohorts)

In transfusion-independent patients switching from eculizumab to zilucoplan, pooled from both the switch cohort and the U.S.-based inadequate responder cohort (collectively, n=7), mean LDH and hemoglobin levels have remained stable in patients enrolled in the long-term extension study through the data cut-off date of February 7, 2018.

A post-hoc analysis of the eculizumab switch cohort data demonstrated that an elevated absolute reticulocyte count (> 2 times the upper limit of normal) at the time of switching from eculizumab to zilucoplan was an important predictor of breakthrough hemolysis during washout. These data are consistent with the observation that transfusion dependency on eculizumab was associated with switch failure. Recent data from the PNH National Service in the United Kingdom ("UK") has identified reticulocyte count as the single best indicator of extravascular hemolysis in PNH patients on eculizumab. These data demonstrated that reticulocyte count appears to be a better indicator of extravascular hemolysis than lactate dehydrogenase, correlating more strongly with raised bilirubin,

increased C3-loading of PNH red blood cells and an increased transfusion requirement. We therefore believe that assessing reticulocyte count will allow us to better identify those patients who are more likely to be able to successfully switch from eculizumab to zilucoplan.

As of November 5, 2018, across all cohorts, no major safety or tolerability concerns have been reported after more than 850 patient weeks of cumulative exposure across all cohorts. No meningococcal infections or thromboembolic events have been observed. Out of more than 6,000 doses administered, only nine mild (grade 1) injection site reactions have occurred in a total of five patients.

Based on the topline results of our Phase 2 studies and feedback provided by the U.S. Food and Drug Administration ("FDA"), Medicines and Healthcare products Regulatory Agency in the UK and Health Canada during End-of-Phase 2 discussions regarding our planned global Phase 3 PNH program, we plan to initiate a global, pivotal, single-arm Phase 3 trial to evaluate the safety and efficacy of zilucoplan in approximately 40 treatment-naïve PNH patients. The co-primary endpoints will be hemoglobin stabilization and reduction in lactate dehydrogenase ("LDH") levels from baseline. We anticipate the data from the planned global Phase 3 registrational trials, if positive, will serve as the basis to support submission of a New Drug Application ("NDA") for zilucoplan for the treatment of PNH. We also plan to meet with the European Medicines Agency in the fourth quarter of 2018 to discuss our global Phase 3 program and, pending the outcome of these discussions, anticipate initiating our Phase 3 clinical trials during the first half of 2019.

In addition, we expect to initiate a supportive trial in approximately 40 transfusion-independent patients switching from eculizumab to zilucoplan. The primary endpoint for this study will be maintenance of transfusion-independence after switching. In a separate CMC Type C meeting, Ra Pharma has also reached agreement with the FDA on the pharmaceutical development program required to support the Phase 3 clinical trials and approval of zilucoplan.

Zilucoplan in gMG

Zilucoplan potently inhibits C5. Inhibition of terminal complement activity at the level of C5 has been demonstrated to prevent development of disease pathology in experimental animal models of MG. Furthermore, eculizumab, a humanized monoclonal inhibitor of C5, was recently approved to treat gMG. Following promising clinical data collected to date from which we observed the favorable pharmacokinetics ("PK") and pharmacodynamics ("PD") profile for zilucoplan, we believe the convenience of once-daily, self-administration of zilucoplan may enable treatment of a broad population of gMG patients.

In December 2017, we initiated dosing in our Phase 2, multicenter, randomized, double-blind, placebo-controlled trial, designed to evaluate the safety, tolerability, and preliminary efficacy of zilucoplan in patients with gMG. At the outset of the 12-week treatment period, patients were randomized in a 1:1:1 ratio and received daily, SC doses of 0.1 mg/kg of zilucoplan, 0.3 mg/kg of zilucoplan, or matching placebo. The primary efficacy endpoint is change in Quantitative Myasthenia Gravis ("QMG") score from baseline to week 12. All patients have the opportunity to receive zilucoplan in a long-term extension study. In August 2018, we announced the completion of enrollment in our Phase 2 study in gMG, with 44 patients dosed, surpassing our original enrollment target of 36 patients and completed dosing in this trial at the end of October 2018. All 44 patients completed the 12-week study and, of these, 43 (98%) elected to enter the long-term extension study to receive active study drug. We expect to report topline data from this trial around year-end 2018.

Zilucoplan in Renal Indications

Building on the clinical data collected to date from which we observed the favorable PK and PD profile of zilucoplan, in January 2018, we initiated dosing in our Phase 1b clinical trial in patients with renal impairment, supporting development of zilucoplan in complement-mediated renal diseases. During the second quarter of 2018, we completed dosing of all subjects and in September 2018, announced positive results from this Phase 1b clinical trial.

The Phase 1b, multi-center, open-label trial was designed to evaluate the PK profile of zilucoplan in patients with severe renal impairment as a lead-in to studying zilucoplan in complement-mediated renal disorders. The trial enrolled 16 subjects, including eight patients with severe renal impairment matched with eight healthy control subjects with normal renal function. Each patient received a single, subcutaneous dose of 0.3 mg/kg of zilucoplan. The PK profile of zilucoplan was consistent across both groups, with exposures similar in renally-impaired patients and healthy volunteers. There were no adverse events reported. Overall, the data indicate that zilucoplan can be used in clinical studies of patients with renal impairment without any need for dose adjustment.

Intellectual Property

In September, we announced that the U.S. Patent and Trademark Office (USPTO) issued U.S. Patent No. 10,106,579 which covers a family of molecules, including zilucoplan. The patent provides composition of matter protection of zilucoplan and methods of use in the treatment of complement-mediated disorders. This patent is the first in a series of composition of matter and treatment filings that are designed to protect zilucoplan through at least 2035.

Financial Update

Since our inception in June 2008, we have devoted substantially all of our resources to organizing and staffing our company, business planning, raising capital, acquiring and developing our proprietary chemistry technology, identifying potential product candidates and conducting preclinical studies of our product candidates and a clinical trial of our lead product candidate, zilucoplan. To date, we have not generated any product revenue and have financed our operations primarily through the public offering and the private placement of our securities and revenue from our collaboration with Merck. As of September 30, 2018, we had received an aggregate of $235.1 million in net proceeds from the issuance of equity and debt securities and $17.5 million in payments in connection with our collaboration and license agreement with Merck ("Merck Agreement"). As of September 30, 2018, our principal source of liquidity was cash and cash equivalents, which totaled $81.1 million.

On October 31, 2016, we completed an initial public offering ("IPO"), in which we issued and sold 7,049,230 shares of our common stock at a public offering price of $13.00 per share, resulting in net proceeds to us of $82.8 million after deducting $6.4 million of underwriting discounts and commissions and offering costs of $2.4 million. On November 29, 2016, we completed the sale of an additional 1,057,385 shares of common stock to the underwriters under the underwriters' option in the IPO to purchase additional shares of common stock at the public offering price of $13.00 per share, resulting in additional net proceeds to us of $12.8 million after deducting underwriting discounts and commissions of $1.0 million.

In February 2018, we completed a follow-on public offering of 9,660,000 shares of our common stock, including the full exercise of the underwriter's over-allotment option of 1,260,000 shares, at $6.00 per share and received aggregate net proceeds of $54.1 million, after deducting $3.5 million of underwriting discounts and commissions and approximately $0.4 million of offering expenses.

In May 2018, we entered into a sales agreement (the "Sales Agreement") with Stifel, Nicolaus & Company, Incorporated ("Stifel") pursuant to which we may sell from time to time, at our option, up to an aggregate of $50.0 million of shares of our common stock through Stifel, as sales agent. Sales of the common stock, if any, will be made by methods deemed to be "at the market offerings." We have agreed to pay Stifel a commission of up to 3% of the gross proceeds from the sale of the shares of our common stock, if any. The Sales Agreement will terminate upon the earliest of: (a) the sale of $50.0 million of shares of our common stock or (b) the termination of the Sales Agreement by us or Stifel. As of September 30, 2018, we have not sold any shares of common stock under this program.

As of September 30, 2018, we had an accumulated deficit of $172.0 million. Our net losses were $48.8 million and $39.3 million for the nine months ended September 30, 2018 and 2017, respectively. We have incurred significant net operating losses in every year since our inception and expect to continue to incur increasing net operating losses and significant expenses for the foreseeable future. Our net losses may fluctuate significantly from quarter to quarter and year to year. We anticipate that our expenses will increase significantly as we:

- continue to advance our lead program, zilucoplan, through clinical development by establishing clinical proof-of-concept activity using convenient SC administration in PNH, gMG, and patients in complement-mediated renal diseases;
- continue our current research programs and development activities;
- seek to identify additional research programs and additional product candidates;
- initiate preclinical testing and clinical trials for any product candidates we identify and develop, maintain, expand and protect our intellectual property portfolio;
- hire additional research, clinical and scientific personnel; and
- incur additional costs associated with operating as a public company, including expanding our operational, finance and management teams.

We do not expect to generate revenue from product sales unless and until we successfully complete development and obtain regulatory approval for a product candidate, which we expect will take a number of years and is subject to significant uncertainty. We believe that our available funds will not be sufficient to enable us to complete our Phase 3 clinical study in PNH. It is also possible that we will not achieve the progress that we expect with respect to zilucoplan because the actual costs and timing of clinical development activities are difficult to predict and are subject to substantial risks and delays. We will be required to obtain further funding through public or private equity offerings, debt financings, collaborations and licensing arrangements or other sources. Adequate additional financing may not be available to us on acceptable terms, or at all. Our failure to raise capital as and when needed would have a negative impact on our financial condition and our ability to pursue our business strategy.

Financial Overview

Revenue

We have derived all of our revenue to date from the Merck Agreement, which we entered in April 2013. Under the Merck Agreement, we collaborated with Merck and used our proprietary drug discovery technology platform to identify orally available cyclic peptides for non-complement targets nominated by Merck and provided specific research and development services. At the signing, Merck paid us an upfront, non-refundable, license fee payment of $4.5 million. In addition, during the research term, which ended in April 2016, Merck reimbursed us for research and development services provided by us in accordance with a pre-specified number of our full-time equivalent employees ("FTEs") working under the Merck Agreement. At the conclusion of the research term, Merck elected to continue the development of a non-complement cardiovascular program target with a large market opportunity, for which we had received $3.5 million in preclinical milestone payments as of September 30, 2018. We are also entitled to receive future aggregate milestone payments of up to $61.5 million and low-to-mid single digit percentage royalties on any future sales for this program target. For additional information about the Merck Agreement, see Item 8, "Financial Statements and Supplementary Data" in our Annual Report on Form 10-K for the year ended December 31, 2017.

To date, we have not generated any revenue from product sales and do not expect to do so in the near future. We expect that our revenue will be less than our expenses for the foreseeable future and that we will experience increasing losses as we continue our development of, and seek regulatory approvals for, our product candidates and begin to commercialize any approved products. Our ability to generate revenue for each product candidate for which we receive regulatory approval will depend on numerous factors, including competition, commercial manufacturing capability and market acceptance of our products.

Research and Development Expenses

Research and development expenses consist primarily of costs incurred for our research activities, including development of our proprietary chemistry technology platform, and our preclinical and clinical candidates, which include:

- employee-related expenses, including salaries, benefits, and stock-based compensation expense;
- expenses incurred under agreements with contract research organizations ("CROs"), contract manufacturing organizations ("CMOs"), and independent contractors that conduct research and development, preclinical and clinical activities on our behalf;
- costs of purchasing lab supplies and non-capital equipment used in our preclinical activities and in manufacturing preclinical study and clinical trial materials;
- consulting, licensing and professional fees related to research and development activities; and
- facility costs, depreciation, and other expenses, which include direct and allocated expenses for rent and maintenance of facilities, insurance and other supplies.

We expense research and development costs as incurred. We recognize costs for certain development activities, such as preclinical studies and clinical trials, based on an evaluation of the progress to completion of specific tasks using information provided to us by our vendors such as patient enrollment or clinical site activations for services received and efforts expended.

Research and development activities are central to our business model. We expect research and development costs to increase significantly for the foreseeable future as our current development programs progress and new programs are added.

The following table sets forth our research and development expenses related to our product pipeline:

	Three Months Ended September 30,		Nine Months Ended September 30,	
	2018	2017	2018	2017
	(in thousands)			
Zilucoplan	$ 5,450	$ 6,836	$ 17,277	$ 15,289
Other pipeline programs	2,045	956	4,167	2,460
Allocated costs	7,495	7,792	21,444	17,749
Unallocated costs	5,880	5,338	17,648	14,857
Total	$ 13,375	$ 13,130	$ 39,092	$ 32,606

The expenses allocated to our product pipeline in the table above relate to CRO and CMO costs associated with our pre-clinical studies and clinical trials. We do not allocate compensation, benefits and other employee-related expenses, costs related to facilities, depreciation, share-based compensation, research and development support services, laboratory supplies and certain other costs directly to programs.

Because of the numerous risks and uncertainties associated with product development, we cannot determine with certainty the duration and completion costs of the current or future preclinical studies and clinical trials or if, when, or to what extent we will generate revenues from the commercialization and sale of our product candidates. We may never succeed in achieving regulatory approval for our product candidates. The duration, costs, and timing of preclinical studies and clinical trials and development of our product candidates will depend on a variety of factors, including:

- successful completion of preclinical studies and Investigational New Drug-enabling studies;
- successful enrollment in, and completion of, clinical trials;
- receipt of marketing approvals from applicable regulatory authorities;
- establishing commercial manufacturing capabilities or making arrangements with third-party manufacturers;
- obtaining and maintaining patent and trade secret protection and non-patent exclusivity;
- launching commercial sales of the product, if and when approved, whether alone or in collaboration with others;
- acceptance of the product, if and when approved, by patients, the medical community and third-party payors;
- effectively competing with other therapies and treatment options;
- a continued acceptable safety profile following approval;
- enforcing and defending intellectual property and proprietary rights and claims; and
- achieving desirable medicinal properties for the intended indications.

A change in the outcome of any of these factors could mean a significant change in the costs and timing associated with the development of our current and future preclinical and clinical product candidates. For example, if the FDA, or another regulatory authority were to require us to conduct clinical trials beyond those that we currently anticipate will be required for the completion of clinical development, or if we experience significant delays in execution of or enrollment in any of our preclinical studies or clinical trials, we could be required to expend significant additional financial resources and time on the completion of preclinical and clinical development. We expect our research and development expenses to increase for the foreseeable future as we continue the development of product candidates.

General and Administrative Expenses

General and administrative expenses consist primarily of employee related expenses, including salaries, benefits, and stock-based compensation, for personnel in executive, finance, facility operations and administrative functions. Other significant costs include facility costs not otherwise included in research and development expenses, legal fees relating to patent and corporate matters, and fees for accounting, tax and consulting services.

We anticipate that our general and administrative expenses will increase in the future to support continued research and development activities, and potential commercialization of our product candidates. These increases will likely include increased costs related to the hiring of additional personnel and fees to outside consultants, lawyers and accountants, among other expenses.

Other Income (Expense), Net

Other income (expense), net primarily consists of interest income earned on our cash and cash equivalents.

Critical Accounting Policies and Significant Judgments and Estimates

Our discussion and analysis of our liquidity, capital resources and results of operations is based upon our condensed consolidated financial statements prepared in accordance with generally accepted accounting principles in the U.S. The preparation of these financial statements requires us to make certain estimates and assumptions that may affect the reported amounts of assets and liabilities, the reported amounts of revenues and expenses during the reported periods and related disclosures. These estimates and assumptions 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 our historical experience, trends in the industry and various other factors that are believed to be reasonable under the circumstances. Actual results may differ from our estimates under different assumptions or conditions.

We believe that our application of the following accounting policies, each of which require significant judgments and estimates on the part of management, is the most critical to aid in fully understanding and evaluating our reported financial results: (1) revenue recognition, (2) research and development expenses, and (3) stock-based compensation. Our critical accounting policies are described in our Annual Report on Form 10-K for the year ended December 31, 2017, except as described below.

Revenue Recognition

Effective January 1, 2018, we adopted Accounting Standards Codification Topic 606, "Revenues from Contracts with Customers" ("ASC 606"). This standard applies to all contracts with customers, except for contracts that are within the scope of other standards, such as leases, insurance, collaboration arrangements and financial instruments.

We have derived all of our revenue to date from the Merck Agreement. The Merck Agreement is accounted for under ASC 606 since it does not represent a collaborative arrangement as we are not an active participant and are not exposed to significant risks and rewards of the arrangement.

The terms of the Merck Agreement contain multiple promised goods and services, which include licenses, research and development activities and participation on the joint steering committee. Payments under the agreement include: (i) an upfront nonrefundable license fee; (ii) payments for research and development services performed by the Company, including reimbursement for certain lab supplies and reagents; (iii) payments based upon the achievement of certain development (pre-clinical and clinical), regulatory and commercial milestones; and (iv) royalties on net product sales, if any.

Under the new revenue standard, we recognize revenue when our customer obtains control of promised goods or services, in an amount that reflects the consideration that we expect to receive in exchange for those goods or services. We recognize revenue following the five-step model prescribed under ASC 606:

- Identification of the contract with the customer;
- Identification of the performance obligations;
- Determination of the transaction price, including the constraint on variable consideration;
- Allocation of the transaction price to the performance obligations in the contract; and
- Recognition of revenue when (or as) the Company satisfies each performance obligation.

In order to account for contracts with customers, such as the Merck Agreement, we identify the promised goods or services in the contract and evaluate whether such promised goods or services represent performance obligations. We account for those components as separate performance obligations when the following criteria are met:

- the customer can benefit from the good or service either on its own or together with other resources that are readily available to the customer, and
- our promise to transfer the good or service to the customer is separately identifiable from other promises in the contract.

This evaluation requires subjective determinations and requires us to make judgments about the promised goods and services and whether such goods and services are separable from the other aspects of the contractual relationship. In determining the performance obligations, we evaluate certain criteria, including whether the promised good or service is capable of being distinct and whether such good or service is distinct within the context of the contract, based on consideration of the relevant facts and circumstances for each arrangement. Factors considered in this determination include the research, manufacturing and commercialization capabilities of the partner, the availability of research and manufacturing expertise in the general marketplace, and the level of integration, interrelation, and interdependence among the promises to transfer goods or services.

The transaction price is allocated among the performance obligations using the relative selling price method, and the applicable revenue recognition criteria are applied to each of the separate performance obligations. At contract inception, we determine the standalone selling price for each performance obligation identified in the contract. If an observable price of the

promised good or service sold separately is not readily available, we utilize assumptions that require judgment to estimate the standalone selling price, which may include development timelines, probabilities of technical and regulatory success, reimbursement rates for personnel costs, forecasted revenues, potential limitations to the selling price of the product, expected technological life of the product, and discount rates.

If the license to the intellectual property is determined to be distinct from the other performance obligations identified in the arrangement, we recognize revenue when the license is transferred to the licensee and the licensee is able to use and benefit from the license. For licenses that are bundled with other promises, we utilize judgment to assess the nature of the combined performance obligation to determine whether the combined performance obligation is satisfied over time or at a point in time and, if over time, the appropriate method of measuring progress for purposes of recognizing revenue from the combined performance obligation. We evaluate the measure of progress each reporting period and, if necessary, adjust the measure of performance and related revenue recognition. When we recognize revenue allocated to the license at a point in time, we may experience significant fluctuations in our revenue from quarter to quarter and year to year depending on the timing of transactions.

At the inception of each arrangement that includes precommercial milestone payments, we evaluate whether the milestones are considered probable of being reached and estimates the amount to be included in the transaction price using the most likely amount method. If it is probable that a significant cumulative revenue reversal would not occur, the associated milestone value is included in the transaction price. Milestone payments that are not within our control, such as regulatory approvals, are not considered probable of being achieved until the uncertainty related to the milestone is resolved. The transaction price is then allocated to each performance obligation on a relative selling price basis, for which we recognize revenue as or when the performance obligations under the contract are satisfied. At the end of each subsequent reporting period, we reevaluate the probability of achievement of such development milestones and any related constraint, and if necessary, adjust its estimate of the overall transaction price. Any such adjustments are recorded on a cumulative catch-up basis, which may significantly affect our revenue in the period of adjustment.

For arrangements that include sales-based royalties, including milestone payments based on the level of sales, and where the license is deemed to be the predominant item to which the royalties relate, we recognize revenue at the later of: (i) when the related sales occur, or (ii) when the performance obligation to which some or all of the royalty has been allocated has been satisfied (or partially satisfied). To date, we have not recognized any royalty revenue resulting from the Merck Agreement.

Result of Operations

Three Months Ended September 30, 2018 and 2017

The following table summarizes our results of operations:

	Three Months Ended September 30,			
	2018	2017	$ Change	% Change
	(in thousands, except percentages)			
Operating expenses:				
Research and development	$ 13,375	$ 13,130	$ 245	1.9%
General and administrative	3,504	2,284	1,220	53.4%
Total operating expenses	16,879	15,414	1,465	9.5%
Loss from operations	(16,879)	(15,414)	(1,465)	9.5%
Other income (expense), net	375	139	236	169.8%
Net loss	$ (16,504)	$ (15,275)	$ (1,229)	8.0%

Research and Development Expenses

Research and development expenses increased by approximately $0.3 million to $13.4 million for the three months ended September 30, 2018, from $13.1 million for the three months ended September 30, 2017. This increase was attributable to: a $0.3 million increase in compensation, benefits and other employee-related expenses due to 2018 salary increases and higher average headcount to support our increased research and development activities; a $0.2 million increase in consulting and professional fees; a $0.1 million increase in non-cash stock-based compensation, primarily relating to our annual grant awarded in February 2018; partially offset by a $0.3 million decrease in CRO and CMO expenses for our non-clinical studies and clinical trials, primarily relating to our zilucoplan program.

General and Administrative Expenses

General and administrative expenses increased by $1.2 million to $3.5 million for the three months ended September 30, 2018, from $2.3 million for the three months ended September 30, 2017. This increase was attributable to: a $0.4 million increase in consulting and professional fees; a $0.3 million increase in compensation, benefits and other employee-related expenses due to 2018 salary increases and higher average headcount to support our increased activities; a $0.1 million increase in non-cash stock-based compensation, primarily relating to our annual grants awarded in February 2018; a $0.1 million increase in patent costs; a $0.1 million increase in insurance, legal and audit costs; and a $0.2 million net increase in other expenses.

Other Income (Expense), Net

Other income (expense), net increased by approximately $0.2 million to $0.4 million in other income, net during the three months ended September 30, 2018, from approximately $0.2 million in other income, net for the three months ended September 30, 2017. This increase was primarily attributable to a $0.2 million increase in interest income.

Nine Months Ended September 30, 2018 and 2017

The following table summarizes our results of operations:

	Nine Months Ended September 30,			
	2018	2017	$ Change	% Change
	(in thousands, except percentages)			
Operating expenses:				
Research and development	$ 39,092	$ 32,606	$ 6,486	19.9%
General and administrative	10,637	7,101	3,536	49.8%
Total operating expenses	49,729	39,707	10,022	25.2%
Loss from operations	(49,729)	(39,707)	(10,022)	25.2%
Other income (expense), net	981	409	572	139.9%
Net loss	$ (48,748)	$ (39,298)	$ (9,450)	24.0%

Research and Development Expenses

Research and development expenses increased by $6.5 million to $39.1 million for the nine months ended September 30, 2018, from $32.6 million for the nine months ended September 30, 2017. This increase was attributable to: a $3.7 million increase in CRO and CMO expenses for our non-clinical studies and clinical trials, primarily relating to our zilucoplan program; a $1.7 million increase in compensation, benefits and other employee-related expenses due to 2018 salary increases and higher average headcount to support our increased research and development activities; a $0.5 million increase in non-cash stock-based compensation, primarily relating to our annual grants awarded in February 2018 and 2017; a $0.3 million increase in consulting and professional fees; and a $0.7 million net increase in other expenses; partially offset by a $0.4 million decrease in laboratory supply and reagent expenses.

General and Administrative Expenses

General and administrative expenses increased by $3.5 million to $10.6 million for the nine months ended September 30, 2018, from $7.1 million for the three months ended September 30, 2017. This increase was attributable to: a $1.2 million increase in non-cash stock-based compensation, primarily relating to our annual grants awarded in February 2018 and 2017; a $1.1 million increase in compensation, benefits and other employee-related expenses due to 2018 salary increases and higher average headcount to support our increased activities; a $0.9 million increase in consulting and professional fees; and a $0.3 million net increase in other expenses.

Other Income (Expense), Net

Other income (expense), net increased by $0.6 million to $1.0 million in other income, net during the nine months ended September 30, 2018, from $0.4 million in other income, net for the nine months ended September 30, 2017. This increase primarily attributable to a $0.5 million increase in interest income.

Liquidity and Capital Resources

Overview

We have funded our operations from inception through September 30, 2018 primarily through the public offerings and the private placement of our securities and revenue from our collaboration with Merck. As of September 30, 2018, we had received an aggregate of $235.1 million in net proceeds from the issuance of equity and debt securities and $17.5 million in payments in connection with our collaboration and license agreement with Merck. As of September 30, 2018, we had cash and cash equivalents of $81.1 million.

On October 31, 2016, we completed our IPO, in which we issued and sold 7,049,230 shares of common stock at a public offering price of $13.00 per share, resulting in net proceeds to us of $82.8 million after deducting $6.4 million of underwriting discounts and commissions and offering costs of $2.4 million. On November 29, 2016, we completed the sale of an additional 1,057,385 shares of common stock to the underwriters under the underwriters' option in the IPO to purchase additional shares of common stock at the public offering price of $13.00 per share, resulting in net proceeds to us of $12.8 million after deducting underwriting discounts and commissions of $1.0 million. The shares began trading on the Nasdaq Global Market on October 26, 2016.

In February 2018, we completed a follow-on public offering of 9,660,000 shares of our common stock, including the full exercise of the underwriter's over-allotment option of 1,260,000 shares, at $6.00 per share and received aggregate net proceeds of $54.1 million, after deducting $3.5 million of underwriting discounts and commissions and approximately $0.4 million of offering expenses.

In May 2018, we entered into the Sales Agreement with Stifel pursuant to which we may sell from time to time, at our option, up to an aggregate of $50.0 million of shares of our common stock through Stifel, as sales agent. Sales of the common stock, if any, will be made by methods deemed to be "at the market offerings." We have agreed to pay Stifel a commission of up to 3% of the gross proceeds from the sale of the shares of our common stock, if any. The Sales Agreement will terminate upon the earliest of: (a) the sale of $50.0 million of shares of our common stock, or (b) the termination of the Sales Agreement by us or Stifel. As of September 30, 2018, we have not sold any shares of common stock under this program.

Cash Flows

The following table provides information regarding our cash flows:

	Nine Months Ended September 30,	
	2018	2017
	(in thousands)	
Net cash provided by (used in):		
Operating activities	$ (42,872)	$ (33,128)
Investing activities	(915)	(1,390)
Financing activities	54,465	797
Net increase (decrease) in cash	$ 10,678	$ (33,721)

Net Cash Used in Operating Activities

Cash used in operating activities represents the cash receipts and disbursements related to all of our activities other than investing and financing activities. Operating cash flow is derived by adjusting our net loss for (1) non-cash operating items such as depreciation and amortization and stock-based compensation as well as (2) changes in operating assets and liabilities, which reflect timing differences between the receipt and payment of cash associated with transactions and when they are recognized in our results of operations.

Net cash used in operating activities was $42.9 million for the nine months ended September 30, 2018 compared to $33.1 million for the nine months ended September 30, 2017. The increase in net cash used in operations was attributable primarily to: a $9.5 million increase in our net loss as a result of higher operating expenses, primarily in connection with our pre-clinical studies and clinical trials related to our zilucoplan program and other research and development pipeline programs, and a net decrease in operating liabilities; partially offset by a net decrease in operating assets and higher non-cash expenses, including stock-based compensation, depreciation and amortization.

Net Cash Used in Investing Activities

Net cash used in investing activities was $0.9 million for the nine months ended September 30, 2018 compared to $1.4 million for the nine months ended September 30, 2017. The decrease in cash used in investing activities was due primarily to a reduction in purchases of property and equipment.

Net Cash Provided by Financing Activities

Net cash provided by financing activities was $54.5 million for the nine months ended September 30, 2018 compared to $0.8 million for the nine months ended September 30, 2017. The increase in cash provided by financing activities was due primarily to the $54.5 million proceeds from the February 2018 follow-on offering and an increase of approximately $0.2 million in proceeds from the exercises of stock options; partially offset by the payment of issuance costs of $0.4 million and by proceeds of $0.7 million from the disgorgement of a stockholder's short-swing profits received in the second quarter of 2017.

Funding Requirements

We expect our expenses to increase in connection with our ongoing activities, particularly as we initiate our Phase 3 clinical trials of zilucoplan in PNH, continue clinical trials of zilucoplan in additional indications, such as gMG, advance the development of pipeline programs, initiate new research and preclinical development efforts and seek marketing approval for any product candidates that we successfully develop. In addition, if we obtain marketing approval for any of our product candidates, we expect to incur significant commercialization expenses related to establishing sales, marketing, distribution and other commercial infrastructure to commercialize such products. Furthermore, we anticipate increased costs associated with being and operating as a public company. Accordingly, we will need to obtain substantial additional funding in connection with our continuing operations. If we are unable to raise capital when needed or on attractive terms, we would be forced to delay, reduce or eliminate our research and development programs or future commercialization efforts.

We believe that our available funds as of September 30, 2018 will enable us to fund our operating expenses and capital expenditure requirements through the end of 2019. We do not expect to generate revenue from product sales unless and until we successfully complete development and obtain regulatory approval for a product candidate, which we expect will take a number of years and is subject to significant uncertainty. Additionally, we believe that our available funds will be sufficient to enable us to prepare and plan for the initiation of our Phase 3 clinical trials of zilucoplan for the treatment of PNH, obtain top line data from our ongoing Phase 2 clinical trial in gMG and advance our other preclinical pipeline programs. We expect that these funds will not, however, be sufficient to enable us to complete our Phase 3 clinical study in PNH. It is also possible that we will not achieve the progress that we expect with respect to zilucoplan because the actual costs and timing of clinical development activities are difficult to predict and are subject to substantial risks and delays. We have based our projections of operating capital requirements on assumptions that may prove to be incorrect and we may use all of our available capital resources sooner than we expect. Because of the numerous risks and uncertainties associated with the development and commercialization of zilucoplan and the research, development and commercialization of other potential product candidates, we are unable to estimate the exact amount of our operating capital requirements. Our future capital requirements will depend on many factors, including:

- the scope, progress, timing, costs and results of clinical trials of zilucoplan;
- research and preclinical development efforts for any future product candidates that we may develop;
- our ability to enter into and the terms and timing of any collaborations, licensing agreements or other arrangements;
- the number of future product candidates that we pursue and their development requirements;

- the outcome, timing and costs of seeking regulatory approvals;
- the costs of commercialization activities for any of our product candidates that receive marketing approval to the extent such costs are not the responsibility of any future collaborators, including the costs and timing of establishing product sales, marketing, distribution and manufacturing capabilities;
- subject to receipt of marketing approval, revenue, if any, received from commercial sales of our current and future product candidates;
- our headcount growth and associated costs as we expand our research and development and establish a commercial infrastructure;
- the costs of preparing, filing and prosecuting patent applications, maintaining and protecting our intellectual property rights and defending against intellectual property related claims; and
- the costs of operating as a public company. Identifying potential product candidates and conducting preclinical studies and clinical trials is a time-consuming, expensive and uncertain process that takes many years to complete, and we may never generate the necessary data or results required to obtain marketing approval and achieve product sales. In addition, our product candidates, if approved, may not achieve commercial success. Accordingly, we will need to continue to rely on additional financing to achieve our business objectives. Adequate additional financing may not be available to us on acceptable terms, or at all.

Until such time, if ever, as we can generate substantial product revenues, we expect to finance our cash needs through a combination of equity offerings, debt financings, collaborations, strategic alliances and licensing arrangements. To the extent that we raise additional capital through the sale of equity or convertible debt securities, the ownership interest of our existing stockholders will be diluted, and the terms of these securities may include liquidation or other preferences that adversely affect the rights of a common stockholder. Debt financing, if available, may involve agreements that include covenants limiting or restricting our ability to take specific actions, such as incurring additional debt, making capital expenditures or declaring dividends.

If we raise funds through additional collaborations, strategic alliances or licensing arrangements with third parties, we may have to relinquish valuable rights to our technologies, future revenue streams, research programs or product candidates or to grant licenses on terms that may not be favorable to us. If we are unable to raise additional funds through equity or debt financings when needed, we may be required to delay, limit, reduce or terminate our product development or future commercialization efforts or grant rights to develop and market product candidates that we would otherwise prefer to develop and market ourselves.

Contractual Commitments and Obligations

The disclosure of our contractual obligations and commitments was reported in our Annual Report on Form 10-K for the year ended December 31, 2017. There have been no material changes from the contractual commitments and obligations previously disclosed in our Annual Report on Form 10-K.

Off-Balance Sheet Arrangements

As of September 30, 2018, we did not have any significant off-balance sheet arrangements, as defined in Item 303(a)(4)(ii) of SEC Regulation S-K promulgated under the Exchange Act.

Recent Accounting Pronouncements

For a discussion of recently adopted or issued accounting pronouncements please refer to Note 1, “Nature of Business and Basis of Presentation” in this Quarterly Report on Form 10-Q.

Jumpstart our Business Startups Act of 2012 Act

The Jumpstart our Business Startups Act of 2012 (the “JOBS Act”) permits an “emerging growth company” such as us to take advantage of an extended transition period to comply with new or revised accounting standards applicable to public companies. We have chosen to “opt out” of this provision and will comply with new or revised accounting standards as required when they are adopted. This decision to opt out of the extended transition period under the JOBS Act is irrevocable.

Item 3. Quantitative and Qualitative Disclosures About Market Risk

Interest Rate Risk

We are exposed to market risk related to changes in interest rates. As of September 30, 2018, we had cash and cash equivalents of $81.1 million, consisting primarily of money market funds. Our primary exposure to market risk is interest rate

sensitivity, which is affected by changes in the general level of U.S. interest rates, particularly because our cash equivalents are in held in short-term money market funds. Due to short-term duration of our investment portfolio and the low risk profile of our investments, an immediate 100 basis point change in interest rates would not have a material effect on the fair market value of our portfolio.

Foreign Currency Risk

We are also exposed to market risk related to changes in foreign currency exchange rates. From time to time, we engage contract research organizations, or CROs, and investigational sites globally. We are therefore subject to fluctuations in foreign currency rates in connection with these engagements. We do not currently hedge our foreign currency exchange rate risk. As of September 30, 2018, we had minimal or no assets or liabilities denominated in foreign currencies.

Effects of Inflation

We do not believe that inflation and changing prices during the three months ended September 30, 2018 had a significant impact on our results of operations or financial condition.

Item 4. Controls and Procedures

(a) Evaluation of Disclosure Controls and Procedures

Our management, with the participation of our Chief Executive Officer and Chief 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 Exchange Act) as of the end of the period covered by this Quarterly Report on Form 10-Q. Management recognizes that any disclosure controls and procedures, no matter how well designed and operated, can provide only reasonable assurance of achieving their objectives. Our disclosure controls and procedures have been designed to provide reasonable assurance of achieving their objectives. Based on such evaluation, our Chief Executive Officer and Chief Financial Officer concluded that our disclosure controls and procedures were effective at the reasonable assurance level as of September 30, 2018.

(b) Changes in Internal Controls

There were no changes in the Company's internal control over financial reporting identified in connection with the evaluation required by paragraph (d) of Exchange Act Rules 13a-15 or 15d-15 that occurred during the quarter ended September 30, 2018 that materially affected, or were reasonably likely to materially affect, the Company's internal control over financial reporting.

PART II — OTHER INFORMATION

Item 1A. Risk Factors

In addition to the other information set forth in this Quarterly Report on Form 10-Q, careful consideration should be given to the risk factors discussed in Item 1A, "Risk Factors" in our Annual Report on Form 10-K for the year ended December 31, 2017, which could materially affect our business, financial condition, and/or future results. The risks described in our Annual Report on Form 10-K are not the only risks we face. Additional risks and uncertainties not currently known to us or that we currently deem to be immaterial also may materially adversely affect our business, financial condition, and/or operating results. There have been no material changes to the risk factors set forth in our Annual Report on Form 10-K for the year ended December 31, 2017.

Item 2. Unregistered Sales of Equity Securities and Use of Proceeds

Recent Sales of Unregistered Securities

None.

Use of Proceeds

In October 2016, we issued and sold 7,049,230 shares of our common stock and, in November 2016, pursuant to the underwriters' option to purchase additional shares, we issued and sold 1,057,385 shares of common stock to the underwriters of our initial public offering, or IPO, at a public offering price of $13.00 per share, for aggregate gross proceeds of $105.4 million. All of the shares issued and sold in the IPO were registered under the Securities Act pursuant to a Registration Statement on Form S-1 (File No. 333- 213917), which was declared effective by the SEC on October 25, 2016. Credit Suisse Securities (USA) LLC, Jeffries LLC and BMO Capital Markets Corp. acted as joint book-running managers of the offering and as representatives of the underwriters.

The net proceeds to us, after deducting underwriting discounts and commissions of $7.4 million and offering expenses of approximately $2.4 million, were approximately $95.6 million.

As of September 30, 2018, we had used $71.8 million of the net proceeds from our IPO.

No offering expenses were paid directly or indirectly to any of our directors or officers, or their associates, or persons owning 10.0% or more of any class of our equity securities or to any other affiliates.

There has been no material change in the planned use of proceeds from our IPO as described in our final prospectus filed with the SEC pursuant to Rule 424(b) of the Securities Act on October 26, 2016. We are holding the balance of the net proceeds from the initial public offering in investments in primarily money market funds.

Item 6. Exhibits

3.1	Third Amended and Restated Certificate of Incorporation of the Registrant, as currently in effect (incorporated by reference to Exhibit 3.1 to the Registrant's Quarterly Report on Form 10-Q (File No. 333-213917) filed November 29, 2016).
3.2	Amended and Restated By-laws of the Registrant, as currently in effect (incorporated by reference to Exhibit 3.2 to the Registrant's Quarterly Report on Form 10-Q (File No. 333-213917) filed November 29, 2016).
31.1*	Certification of Chief Executive Officer pursuant to Rules 13a-14(a) or 15d-14(a) of the Securities Exchange Act of 1934, as adopted pursuant to Section 302 of the Sarbanes-Oxley Act of 2002.
31.2*	Certification of Chief Financial Officer pursuant to Rules 13a-14(a) or 15d-14(a) of the Securities Exchange Act of 1934, as adopted pursuant to Section 302 of the Sarbanes-Oxley Act of 2002.
32.1**	Certifications pursuant to 18 U.S.C. Section 1350, as adopted pursuant to Section 906 of The Sarbanes-Oxley Act of 2002, by Douglas A. Treco, Ph.D., President and Chief Executive Officer of the Company, and David C. Lubner, Executive Vice President and Chief Financial Officer of the Company.
101.INS	Extensible Business Reporting Language (XBRL) Instance Document.
101.SCH	XBRL Schema Document.
101.CAL	XBRL Calculation Linkbase Document.
101.LAB	XBRL Labels Linkbase Document.
101.PRE	XBRL Presentation Linkbase Document.
101.DEF	XBRL Definition Linkbase Document.

* Filed herewith.
** Furnished herewith.

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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.

Dated: November 7, 2018

RA PHARMACEUTICALS, INC.

By: /s/ Douglas A. Treco
Douglas A. Treco, Ph.D.
President and Chief Executive Officer
(Principal Executive Officer)

By: /s/ David C. Lubner
David C. Lubner
Executive Vice President and Chief Financial Officer
(Principal Financial Officer)

Exhibit 31.1

CERTIFICATION

I, Douglas A. Treco, certify that:

1. I have reviewed this Quarterly Report on Form 10-Q of Ra 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 7, 2018

/s/ Douglas A. Treco
Douglas A. Treco, Ph.D.
President and Chief Executive Officer

Exhibit 31.2

CERTIFICATION

I, David C. Lubner, certify that:

1. I have reviewed this Quarterly Report on Form 10-Q of Ra 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.

Dated: November 7, 2018

/s/ David C. Lubner
David C. Lubner
Executive Vice President and Chief Financial Officer

Exhibit 32.1

**CERTIFICATIONS OF CEO AND CFO PURSUANT TO 18 U.S.C. SECTION 1350,
AS ADOPTED PURSUANT TO
SECTION 906 OF THE SARBANES-OXLEY ACT OF 2002**

In connection with this Quarterly Report on Form 10-Q of Ra Pharmaceuticals, Inc. (the "Company") for the period ended September 30, 2018, as filed with the Securities and Exchange Commission on the date hereof (the "Report"), each of the undersigned, Douglas A. Treco, Ph.D., President and Chief Executive Officer of the Company, and David C. Lubner, Executive Vice President and Chief Financial Officer of the Company, hereby certifies, pursuant to 18 U.S.C. (section) 1350, as adopted pursuant to (section) 906 of the Sarbanes-Oxley Act of 2002, that to the best of his or her knowledge:

(1) The Report fully complies with the requirements of Section 13(a) or 15(d) of the Securities Exchange Act of 1934; and

(2) The information contained in the Report fairly presents, in all material respects, the financial condition and results of operations of the Company.

Dated: November 7, 2018

/s/ Douglas A. Treco
Douglas A. Treco, Ph.D.
President and Chief Executive Officer

/s/ David C. Lubner
David C. Lubner
Executive Vice President and Chief Financial Officer