

**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 **June 30, 2019**

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

**87 Cambridge Park Drive**  
**Cambridge, MA**  
(Address of principal executive offices)

**26-2908274**  
(I.R.S. Employer  
Identification No.)

**02140**  
(Zip code)

**617-401-4060**  
(Registrant's telephone number, including area code)

**N/A**  
(Former name, former address and former fiscal year, if changed since last report)

Securities registered pursuant to Section 12(b) of the Act:

<u>Title of each class</u>	<u>Trading Symbol(s)</u>	<u>Name of each exchange on which registered</u>
Common Stock, \$0.001 par value per share	RARX	Nasdaq Global Market

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 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 "large accelerated filer," "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

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The number of shares outstanding of the registrant's common stock as of August 1, 2019 was 47,014,618.

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## 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, including a Phase 3 clinical program evaluating zilucoplan for the treatment of generalized myasthenia gravis and a Phase 2 trial of zilucoplan for the treatment of immune-mediated necrotizing myopathy, and future pre-clinical and clinical studies;
- the risk that topline data from our Phase 2 clinical programs in generalized myasthenia gravis may not be indicative of 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, 2018, 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)**

	<u>June 30, 2019</u>	<u>December 31, 2018</u>
<b>Assets</b>		
Current assets:		
Cash and cash equivalents	\$ 173,603	\$ 209,822
Prepaid expenses and other current assets	6,051	2,585
Total current assets	<u>179,654</u>	<u>212,407</u>
Property and equipment, net	4,775	5,165
Operating lease right-of-use assets, net	2,734	—
Restricted cash	1,334	1,334
Other assets	741	314
Total assets	<u>\$ 189,238</u>	<u>\$ 219,220</u>
<b>Liabilities and Stockholders' Equity</b>		
Current liabilities:		
Accounts payable	\$ 4,498	\$ 3,245
Accrued expenses	6,173	6,477
Operating lease liabilities	1,073	—
Deferred rent	—	479
Total current liabilities	<u>11,744</u>	<u>10,201</u>
Operating lease liabilities, net of current portion	3,784	—
Deferred rent, net of current portion	—	1,880
Deferred tax liabilities	21	21
Total liabilities	<u>15,549</u>	<u>12,102</u>
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; 42,373 and 42,072 shares issued and outstanding as of June 30, 2019 and December 31, 2018, respectively	42	42
Additional paid-in capital	402,740	395,233
Accumulated deficit	<u>(229,093)</u>	<u>(188,157)</u>
Total stockholders' equity	<u>173,689</u>	<u>207,118</u>
Total liabilities and stockholders' equity	<u>\$ 189,238</u>	<u>\$ 219,220</u>

See Notes to Unaudited Condensed Consolidated Financial Statements.

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

	<u>Three Months Ended June 30,</u>		<u>Six Months Ended June 30,</u>	
	<u>2019</u>	<u>2018</u>	<u>2019</u>	<u>2018</u>
Operating expenses:				
Research and development	\$ 17,914	\$ 12,305	\$ 33,196	\$ 25,717
General and administrative	4,933	3,821	9,740	7,133
Total operating expenses	<u>22,847</u>	<u>16,126</u>	<u>42,936</u>	<u>32,850</u>
Loss from operations	(22,847)	(16,126)	(42,936)	(32,850)
Other income, net	950	380	2,000	606
Net loss	<u>\$ (21,897)</u>	<u>\$ (15,746)</u>	<u>\$ (40,936)</u>	<u>\$ (32,244)</u>
Net loss per common share — basic and diluted	\$ (0.52)	\$ (0.49)	\$ (0.97)	\$ (1.08)
Weighted average number of common shares outstanding — basic and diluted	42,294	32,307	42,235	29,789

See Notes to Unaudited Condensed Consolidated Financial Statements.

**RA PHARMACEUTICALS, INC.**  
**CONDENSED CONSOLIDATED STATEMENTS OF STOCKHOLDERS' EQUITY**  
**(UNAUDITED)**  
**(in thousands)**

	Common Stock		Additional Paid-In	Accumulated	Total
	Shares	Amount	Capital	Deficit	Stockholders' Equity
			Amount	Amount	Amount
<b>January 1, 2019</b>	<b>42,072</b>	<b>\$ 42</b>	<b>\$ 395,233</b>	<b>\$ (188,157)</b>	<b>\$ 207,118</b>
Exercise of common stock options	118	—	769	—	769
Stock-based compensation	—	—	2,890	—	2,890
Shares issued upon vesting of restricted stock and under the employee purchase plan, net of tax withholdings	64	—	(484)	—	(484)
Net loss	—	—	—	(19,039)	(19,039)
<b>March 31, 2019</b>	<b>42,254</b>	<b>42</b>	<b>398,408</b>	<b>(207,196)</b>	<b>191,254</b>
Exercise of common stock options	96	—	748	—	748
Stock-based compensation	—	—	3,445	—	3,445
Shares issued upon vesting of restricted stock and under the employee purchase plan, net of tax withholdings	23	—	139	—	139
Net loss	—	—	—	(21,897)	(21,897)
<b>June 30, 2019</b>	<b>42,373</b>	<b>\$ 42</b>	<b>\$ 402,740</b>	<b>\$ (229,093)</b>	<b>\$ 173,689</b>

	Common Stock		Additional Paid-In	Accumulated	Total
	Shares	Amount	Capital	Deficit	Stockholders' Equity
			Amount	Amount	Amount
<b>January 1, 2018</b>	<b>22,626</b>	<b>\$ 23</b>	<b>\$ 192,375</b>	<b>\$ (123,214)</b>	<b>\$ 69,184</b>
Issuance of common stock from public offerings, net of underwriter discounts and issuance costs	9,660	9	54,034	—	54,043
Stock-based compensation	—	—	1,946	—	1,946
Net loss	—	—	—	(16,498)	(16,498)
<b>March 31, 2018</b>	<b>32,286</b>	<b>32</b>	<b>248,355</b>	<b>(139,712)</b>	<b>108,675</b>
Exercise of common stock options	50	—	179	—	179
Stock-based compensation	—	—	1,812	—	1,812
Net loss	—	—	—	(15,746)	(15,746)
<b>June 30, 2018</b>	<b>32,336</b>	<b>\$ 32</b>	<b>\$ 250,346</b>	<b>\$ (155,458)</b>	<b>\$ 94,920</b>

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

	Six Months Ended June 30,	
	2019	2018
<b>Cash flows from operating activities</b>		
Net loss	\$ (40,936)	\$ (32,244)
Adjustments to reconcile net loss to net cash used in operating activities:		
Depreciation and amortization	1,063	772
Stock-based compensation	6,335	3,758
Other, net	49	—
Changes in operating assets and liabilities:		
Prepaid expenses and other current assets	(3,466)	(534)
Accounts payable and accrued expenses	1,056	(693)
Operating lease liabilities	(490)	—
Other, net	—	(102)
Net cash used in operating activities	(36,389)	(29,043)
<b>Cash flows from investing activities</b>		
Purchase of property and equipment	(416)	(558)
Net cash used in investing activities	(416)	(558)
<b>Cash flows from financing activities</b>		
Proceeds from common stock offering, net of underwriter discounts	—	54,482
Payment of common stock offering costs	(586)	(371)
Payments related to restricted stock units vesting	(583)	—
Proceeds from exercises of stock options and ESPP shares	1,755	179
Other, net	—	(13)
Net cash provided by financing activities	586	54,277
Net increase (decrease) in cash, cash equivalents and restricted cash	(36,219)	24,676
Cash, cash equivalents and restricted cash, beginning of period	211,156	71,715
<b>Cash, cash equivalents and restricted cash, end of period</b>	<b>\$ 174,937</b>	<b>\$ 96,391</b>
<b>Reconciliation of cash, cash equivalents and restricted cash:</b>		
Cash, cash equivalents and restricted cash, end of period	\$ 174,937	\$ 96,391
Less restricted cash	(1,334)	(1,334)
Cash and cash equivalents, end of period	<b>\$ 173,603</b>	<b>\$ 95,057</b>

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, 2018. 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, 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 generalized myasthenia gravis (“gMG”), immune-mediated necrotizing myopathy (“IMNM”), and other complement-mediated disorders. Additionally, the Company is pursuing discovery and pre-clinical programs targeting selective inhibition of other uncontrolled complement pathway factors to treat a variety of neurologic, renal, and inflammatory diseases. In addition to the Company’s 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 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 \$229.1 million as of June 30, 2019 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. As of June 30, 2019, the Company had cash and cash equivalents of \$173.6 million, which, in addition to the net proceeds of \$140.2 million from the follow-on public offering closed in July 2019, is expected to fund operating expenses and capital expenditure requirements through at least the end of 2021.

***Public Offerings***

In July 2019, the Company completed a follow-on public offering of 4,600,000 shares of common stock, including the full exercise of the underwriters’ option to purchase an additional 600,000 shares, at \$32.50 per share and received aggregate net proceeds of \$140.2 million, after deducting \$9.0 million of underwriting discounts and commissions and approximately \$0.3 million of offering expenses.

***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, Ra Europe Limited, and Ra Pharmaceuticals Security Corporation. Intercompany balances and transactions are eliminated in consolidation.

***Reclassifications***

Certain reclassifications have been made to the prior year condensed consolidated balance sheet to conform to the current year presentation. These reclassifications have no impact on the Company’s net loss or cash flows.

*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, 2018. There have been no material changes to the significant accounting policies during the period ended June 30, 2019, except as described below.

*Leases*

Effective January 1, 2019, the Company adopted Accounting Standards Codification ("ASC"), Topic 842, *Leases* ("ASC 842"), using the required modified retrospective approach and utilizing the effective date as its date of initial application. The prior period is presented in accordance with the previous guidance in ASC 840, *Leases*.

At the inception of an arrangement, the Company determines whether the arrangement is or contains a lease based on the unique facts and circumstances present. Leases with a term greater than one year are recognized on the balance sheet as right-of-use assets, lease liabilities and, if applicable, long-term lease liabilities. The Company has elected not to recognize on the balance sheet leases with terms of one year or less. Operating lease liabilities and their corresponding right-of-use assets are recorded based on the present value of lease payments over the expected remaining lease term. However, certain adjustments to the right-of-use asset may be required for items such as incentives received. The interest rate implicit in lease contracts is typically not readily determinable. As a result, the Company utilizes its incremental borrowing rates, which are the rates incurred to borrow on a collateralized basis over a similar term an amount equal to the lease payments in a similar economic environment.

In accordance with the guidance in ASC 842, components of a lease should be split into three categories: lease components (e.g. land, building, etc.), non-lease components (e.g. common area maintenance, consumables, etc.), and non-components (e.g. property taxes, insurance, etc.). Then the fixed and in-substance fixed contract consideration (including any related to non-components) must be allocated based on the respective relative fair values to the lease components and non-lease components.

Although separation of lease and non-lease components is required, certain practical expedients are available. Entities may elect the practical expedient to not separate lease and non-lease components. Rather, they would account for each lease component and the related non-lease component together as a single component. For new and amended leases beginning in 2019 and after, the Company has elected to account for the lease and non-lease components for leases for classes of all underlying assets and allocate all of the contract consideration to the lease component only.

*Newly Adopted Accounting Pronouncements*

In February 2016, the FASB issued ASU 2016-02, "*Leases*", ("ASU 2016-02"), which superseded the lease accounting requirements in ASC 840, *Leases* and created a new ASC 842, *Leases*. ASC 842 requires a lessee to recognize assets and liabilities on the balance sheet for most leases and changes many key definitions, including the definition of a lease. The new standard includes a short-term lease exception for leases with a term of 12 months or less, as part of which a lessee can make an accounting policy election not to recognize lease assets and lease liabilities. Lessees will continue to differentiate between finance leases (previously referred to as capital leases) and operating leases using classification criteria that are substantially similar to the previous guidance.

ASU 2016-02 became effective on January 1, 2019, requiring the use of a modified retrospective transition approach applied at the beginning of the earliest comparative period presented in the financial statements. In July 2018, the FASB issued ASU 2018-11, *Leases, Targeted Improvements*, ("ASU 2018-11"), which contains certain amendments to ASU 2016-02 intended to provide relief in implementing the new standard. ASU 2018-11 provides registrants with an option to not restate comparative periods presented in the financial statements. The Company adopted this new standard on January 1, 2019 using a cumulative-effect adjustment on the effective date of the standard, for which comparative periods are presented in accordance with the previous guidance in ASC 840, *Leases*.

In adopting the new standard, the Company elected to utilize the available package of practical expedients permitted under the transition guidance within the new standard, which does not require the reassessment of the following: i) whether existing or

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expired arrangements are or contain a lease, ii) the lease classification of existing or expired leases, and iii) whether previous initial direct costs would qualify for capitalization under the new lease standard. Additionally, the Company made an accounting policy election to keep leases with a term of 12 months or less off of its balance sheet. The initial adoption of this standard resulted in the recognition of operating lease liabilities and right-of-use assets of \$5.3 million and \$3.0 million, respectively, on the Company's balance sheet. The \$2.3 million difference between the account balances relates to the write-off of the previously existing ASC 840 balances, which was primarily driven by the tenant improvements allowance. The adoption of the standard did not have a material effect on the statements of operations or statement of cash flows.

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 is fixed at the grant date. The Company adopted this new standard on January 1, 2019. The adoption of ASU 2018-07 did not have a significant impact on its financial statements.

## 2. Supplemental Balance Sheet Information

### *Property and equipment, net*

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

	June 30, 2019	December 31, 2018
Computer equipment and software	\$ 60	\$ 53
Furniture, fixtures and office equipment	390	390
Laboratory equipment	6,399	6,149
Construction in progress	91	—
Leasehold improvements	3,744	3,755
	10,684	10,347
Accumulated depreciation	(5,909)	(5,182)
Property and equipment, net	\$ 4,775	\$ 5,165

Depreciation expense was \$0.4 million and \$0.3 million for the three months ended June 30, 2019 and 2018, respectively, and \$0.8 million and \$0.7 million for the six months ended June 30, 2019 and 2018, respectively.

### *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 June 30, 2019 and December 31, 2018, the Company had restricted cash securing the letters of credit. The cash will be restricted until the termination or modification of the lease arrangement.

### *Accrued expenses*

Accrued expenses consist of the following (in thousands):

	June 30, 2019	December 31, 2018
Payroll and employee-related costs	\$ 2,236	\$ 3,309
Research and development costs	3,509	2,491
Other	428	677
Total	\$ 6,173	\$ 6,477

## 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.

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The fair value hierarchy level is determined by asset and liability class based on the lowest level of significant input. During the three and six months ended June 30, 2019, there were no transfers between levels.

The fair value of the cash equivalents was determined through quoted prices provided by third-party pricing services.

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

	June 30, 2019			Total
	Level 1	Level 2	Level 3	
Cash equivalents — Money market funds	\$ 173,498	\$ —	\$ —	\$ 173,498
Total assets	<u>\$ 173,498</u>	<u>\$ —</u>	<u>\$ —</u>	<u>\$ 173,498</u>

  

	December 31, 2018			Total
	Level 1	Level 2	Level 3	
Cash equivalents — Money market funds	\$ 210,404	\$ —	\$ —	\$ 210,404
Total assets	<u>\$ 210,404</u>	<u>\$ —</u>	<u>\$ —</u>	<u>\$ 210,404</u>

**4. Leases**

We lease certain office space, laboratory space, and equipment. At the inception of an arrangement, the Company determines whether the arrangement is or contains a lease based on the unique facts and circumstances present. The Company does not recognize right-of-use assets or lease liabilities for leases determined to have a term of 12 months or less. For new and amended leases beginning in 2019 and after, the Company has elected to account for the lease and non-lease components for leases for classes of all underlying assets and allocate all of the contract consideration to the lease component only.

In September 2015, the Company entered into an operating lease for laboratory and office space at its headquarters in Cambridge, Massachusetts. The lease expires in April 2023 and contains various clauses for renewal at the Company's option and certain rent escalation clauses. The Company is also obligated to pay operating costs, including property taxes, insurance, maintenance and other operating expenses. In connection with the lease, the Company was provided tenant improvements allowance totaling approximately \$2.7 million by the landlord as reimbursement for capital improvements to the facility.

As of June 30, 2019, the Company's balance sheet included operating lease liabilities and right-of-use assets of \$4.9 million and \$2.7 million, respectively. The difference between the account balances relates to the write-off of the previously existing ASC 840 balances, which was primarily driven by the tenant improvements allowance. Lease related financial information is summarized below (in thousands):

Operating leases	Three Months Ended June 30, 2019		Six Months Ended June 30, 2019	
Operating lease cost	\$	229	\$	459
Short-term lease cost		35		45
Variable lease cost		229		459
Total operating lease costs	<u>\$</u>	<u>493</u>	<u>\$</u>	<u>963</u>

  

Other information	June 30, 2019	
Operating cash flows used for operating leases	\$	695
Weighted average remaining lease term in years		3.9
Weighted average discount rate		8.0%

  

Year Ended December 31,	Maturity of Lease Liabilities	
2019 (remaining six months)	\$	708
2020		1,442
2021		1,483
2022		1,524
2023		513
Total lease payments		5,670
Less: imputed interest		(813)
Total operating lease liabilities	<u>\$</u>	<u>4,857</u>

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Future minimum commitments due under operating lease agreements as of December 31, 2018 were as follows (in thousands):

Year Ended December 31,	Minimum Lease Payments
2019	\$ 1,403
2020	1,442
2021	1,483
2022	1,524
2023	513
Total operating lease liabilities	<u>\$ 6,365</u>

## 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 June 30,		Six Months Ended June 30,	
	2019	2018	2019	2018
Research and development	\$ 1,912	\$ 826	\$ 3,559	\$ 1,857
General and administrative	1,533	986	2,776	1,901
Total	<u>\$ 3,445</u>	<u>\$ 1,812</u>	<u>\$ 6,335</u>	<u>\$ 3,758</u>

During the six months ended June 30, 2019, the Company issued 1.7 million stock options with a per share weighted-average grant date fair value of \$14.34. During the six months ended June 30, 2019, 102,329 restricted stock units vested.

During the three and six months ended June 30, 2019, 4,506 and 27,544 shares were net settled with an aggregate value of \$0.1 million and \$0.6 million in satisfaction of tax withholdings. No such net settlement occurred during the six months ended June 30, 2018.

## 6. Net Loss Per Share

The Company computes basic and diluted net loss per share using a methodology that gives effect to the impact of outstanding participating securities (the “two-class method”). As the three and six months ended June 30, 2019 and 2018 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 June 30,	
	2019	2018
Stock options	5,494	3,965
Restricted stock units	198	318
Total	<u>5,692</u>	<u>4,283</u>

## 7. Subsequent Events

On July 15, 2019, the Company entered into a license agreement (the “Agreement”) for a proprietary drug delivery technology. In connection with entering into the Agreement, the Company paid a non-refundable and non-creditable upfront payment of \$2.0 million, and the Company may be required to pay up to an aggregate of \$14.5 million in development milestones and other license payments, up to \$55.0 million in sales milestone payments, and tiered single-digit royalties on net sales of products utilizing the licensed technology. The term of the Agreement continues until the expiration of the applicable royalty term on a country-by-country and product-by-product basis.

## 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, 2018. 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, 2018, 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. Inappropriate activation of the complement system can quickly turn it from a beneficial defense system to an aggressor that plays a major role in immune and inflammatory diseases. The complement system, which consists of approximately 30 interacting proteins, offers a target-rich opportunity for us to leverage our proprietary peptide chemistry platform, which was pioneered by Nobel Laureate Dr. Jack Szostak and allows us to inhibit certain uncontrolled complement pathway factors involved in complement-mediated diseases. Known as our Extreme Diversity platform, this proprietary macrocyclic peptide chemistry technology allows us to produce synthetic macrocyclic peptides that combine the diversity and specificity of antibodies with the pharmacological properties of small molecules. We believe this technology will allow us to pursue challenging targets for which only monoclonal antibodies have been developed.

We are developing our lead product candidate, zilucoplan, a potent, synthetic macrocyclic peptide C5 inhibitor, formulated for convenient self-administered subcutaneous injection, which is an injection into the tissue under the skin, for the treatment of various complement-mediated diseases, including generalized myasthenia gravis, or gMG, immune-mediated necrotizing myopathy, or IMNM, and other tissue-based, complement-mediated disorders with high unmet medical need.

Myasthenia gravis, or 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 of 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 we reported positive top-line data in December 2018. In May 2019, we presented results from the open-label, long-term extension study, in which statistically significant and clinically meaningful improvements in primary and secondary endpoints were sustained in patients treated with zilucoplan at 24 weeks. In April 2019, we announced the successful completion of End-of-Phase 2 interactions with the U.S. Food and Drug Administration, or FDA, for its Phase 3 clinical trial of zilucoplan in gMG. Based on FDA feedback and advice provided by the European Medicines Agency, or EMA, in the second quarter of 2019, we plan to initiate a single, pivotal, 12-week, Phase 3, randomized, double-blind, placebo-controlled trial evaluating the efficacy of zilucoplan in patients with gMG in the second half of 2019.

IMNM is an autoimmune myopathy characterized by skeletal muscle necrosis, severe proximal limb weakness, and elevated creatine kinase, or CK, levels. IMNM is categorized into two subtypes defined by the presence of distinct autoantibodies against 3-hydroxy-3-methylglutaryl-coenzyme A reductase, or signal recognition particle. In IMNM, these autoantibodies drive complement-mediated necrosis of muscle fibers, resulting in severe, progressive, and debilitating proximal muscle weakness. IMNM affects more than 15,000 patients in the United States, European Union, and Japan. There are currently no approved therapies for the treatment of IMNM. In June 2019, we announced the FDA's clearance of our Investigational New Drug, or IND, application for zilucoplan for the treatment of IMNM. We expect to initiate a Phase 2 clinical trial evaluating zilucoplan for the treatment of IMNM in the second half of 2019.

We have a life-cycle management plan with an extended release, or XR, program for zilucoplan and an oral, small molecule C5 inhibitor, as well as inhibitors of other complement factors for certain renal, autoimmune, and central nervous system, or CNS, diseases. Our XR program includes two formulations: the poly(D,L-lactic-co-glycolic acid), or PLGA, XR formulation of zilucoplan and the FluidCrystal<sup>®</sup>, or FC, XR formulation of zilucoplan.

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In April 2019, we presented pre-clinical data for the PLGA XR formulation of zilucoplan, in which rapid and sustained pharmacodynamic inhibition of complement C5 was achieved with once-weekly subcutaneous dosing in non-human primates. We believe these data support the possibility of once weekly or less frequent dosing for zilucoplan.

In July 2019, we entered into an exclusive worldwide license agreement for the use of Camurus AB's proprietary FC technology to develop, manufacture, and commercialize a long-acting formulation of zilucoplan. In July 2019, we reported pre-clinical data for the FC XR formulation of zilucoplan, in which non-human primates receiving a single dose of the FC XR formulation of zilucoplan rapidly achieved and maintained target levels of complement inhibition for at least seven days without the need for intravenous loading. We anticipate the XR program entering human clinical studies in the first half of 2020.

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., or Merck. In December 2018, Merck paid us a development milestone as part of this collaboration.

### **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 pre-clinical 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 public offerings and the private placement of our securities and revenue from our collaboration with Merck. As of June 30, 2019, we had received an aggregate of \$375.3 million in net proceeds from the issuance of equity and debt securities and \$20.0 million in payments in connection with our collaboration and license agreement with Merck, or the Merck Agreement.

In July 2019, we completed a follow-on public offering of 4,600,000 shares of our common stock, including the full exercise of the underwriters' over-allotment option to purchase an additional 600,000 shares, at \$32.50 per share and received aggregate net proceeds of \$140.2 million, after deducting \$9.0 million of underwriting discounts and commissions and approximately \$0.3 million of offering expenses.

As of June 30, 2019, we had an accumulated deficit of \$229.1 million. Our net losses were \$40.9 million and \$32.2 million for the six months ended June 30, 2019 and 2018, 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, a self-administered, subcutaneous C5 inhibitor, through clinical development using convenient SC administration in gMG, IMNM, and other tissue-based, complement-mediated disorders with high unmet medical need;
- continue our current research programs and development activities;
- seek to identify additional research programs and additional product candidates;
- initiate pre-clinical testing and clinical trials for any product candidates we identify and develop, maintain, expand and protect our intellectual property portfolio;
- hire additional research, clinical, scientific, and commercial personnel;
- incur additional costs associated with operating as a public company, including expanding our operational, financial and management teams; and
- incur additional costs associated with 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.

As of June 30, 2019, we had cash and cash equivalents of \$173.6 million, which, in addition to the net proceeds of \$140.2 million from the follow-on offering closed in July 2019, is expected to fund operating expenses and capital expenditure requirements through at least the end of 2021. 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 cash and cash equivalents as of June 30, 2019, including our net proceeds

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from the July 2019 follow-on offering, will be sufficient to enable us to fund pre-commercialization activities for zilucoplan and the XR formulations of zilucoplan, and the clinical development of our pipeline programs. 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 a collaboration and license agreement with Merck, which we entered into in April 2013. Under the Merck Agreement, we used our proprietary drug discovery technology platform to identify orally available cyclic peptides for non-complement targets nominated by Merck. As of June 30, 2019, we recorded \$20.0 million in revenue in connection with our Merck Agreement. No payments were received from Merck during the three and six months ended June 30, 2019. We are also entitled to receive future aggregate milestone payments of up to \$59.0 million and low-to-mid single digit percentage royalties on any future sales under the Merck Agreement. 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, 2018.

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 pre-clinical 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, pre-clinical and clinical activities on our behalf;
- costs of purchasing lab supplies and non-capital equipment used in our pre-clinical activities and in manufacturing pre-clinical 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 pre-clinical 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.

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

	Three Months Ended June 30,		Six Months Ended June 30,	
	2019	2018	2019	2018
	(in thousands)			
Zilucoplan	\$ 7,517	\$ 5,224	\$ 14,152	\$ 11,827
Other pipeline programs	1,978	1,299	2,545	2,122
Allocated costs	9,495	6,523	16,697	13,949
Unallocated costs	8,419	5,782	16,499	11,768
Total	<u>\$ 17,914</u>	<u>\$ 12,305</u>	<u>\$ 33,196</u>	<u>\$ 25,717</u>

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 pre-clinical studies, clinical trials, pharmaceutical development, and chemical, manufacturing and controls 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 pre-clinical studies and clinical trials and development of our product candidates will depend on a variety of factors, including:

- successful completion of pre-clinical 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 pre-clinical and clinical product candidates. For example, if the Food and Drug Administration (“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 pre-clinical studies or clinical trials, we could be required to expend significant additional financial resources and time on the completion of pre-clinical 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.

[Table of Contents](#)**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.

There have been no material changes to our critical accounting policies from those described in “Management’s Discussion and Analysis of Financial Condition and Results of Operations” in our Annual Report on Form 10-K for the year ended December 31, 2018. We believe that our application of the following accounting policies, each of which require significant judgments and estimates on the part of management, and each of which is described in “Management’s Discussion and Analysis of Financial Condition and Results of Operations” in our Annual Report on Form 10-K for the year ended December 31, 2018, 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.

**Results of Operations****Three Months Ended June 30, 2019 and 2018**

The following table summarizes our results of operations:

	Three Months Ended		\$ Change	% Change
	2019	2018		
	(in thousands, except percentages)			
Operating expenses:				
Research and development	\$ 17,914	\$ 12,305	\$ 5,609	45.6%
General and administrative	4,933	3,821	1,112	29.1%
Total operating expenses	22,847	16,126	6,721	41.7%
Loss from operations	(22,847)	(16,126)	(6,721)	41.7%
Other income, net	950	380	570	150.0%
Net loss	\$ (21,897)	\$ (15,746)	\$ (6,151)	39.1%

**Research and Development Expenses**

Research and development expenses increased by \$5.6 million to \$17.9 million for the three months ended June 30, 2019, from \$12.3 million for the three months ended June 30, 2018. This increase was attributable to: a \$3.0 million increase in CRO and CMO expenses for our zilucoplan development program; a \$1.9 million increase in compensation, benefits, non-cash stock-based compensation and other employee-related expenses due to 2019 salary increases and higher average headcount to support our increased research and development activities; a \$0.3 million increase in consulting and professional fees; and a \$0.4 million net increase in other expenses.

**General and Administrative Expenses**

General and administrative expenses increased by \$1.1 million to \$4.9 million for the three months ended June 30, 2019, from \$3.8 million for the three months ended June 30, 2018. This increase was attributable to: a \$0.7 million increase in compensation, benefits, non-cash stock-based compensation and other employee-related expenses due to 2019 salary increases and higher average headcount to support our increased activities; a \$0.2 million increase in consulting and professional fees related to pre-commercialization activities; a \$0.3 million net increase in other expenses primarily relating to patent costs; partially offset by a \$0.1 million decrease in legal, audit and insurance costs.

**Other Income, Net**

Other income, net increased by \$0.6 million to \$1.0 million in other income, net during the three months ended June 30, 2019, from \$0.4 million in other income, net for the three months ended June 30, 2018. This increase was due primarily to a \$0.6 million increase in interest income.

**Six Months Ended June 30, 2019 and 2018**

	Six Months Ended June 30,		S Change	% Change
	2019	2018		
	(in thousands, except percentages)			
Operating expenses:				
Research and development	\$ 33,196	\$ 25,717	\$ 7,479	29.1%
General and administrative	9,740	7,133	2,607	36.5%
Total operating expenses	42,936	32,850	10,086	30.7%
Loss from operations	(42,936)	(32,850)	(10,086)	30.7%
Other income, net	2,000	606	1,394	230.0%
Net loss	\$ (40,936)	\$ (32,244)	\$ (8,692)	27.0%

**Research and Development Expenses**

Research and development expenses increased by \$7.5 million to \$33.2 million for the six months ended June 30, 2019, from \$25.7 million for the six months ended June 30, 2018. This increase was attributable to: a \$2.8 million increase in CRO and CMO expenses for our zilucoplan development program; a \$3.2 million increase in compensation, benefits, non-cash stock-based compensation and other employee-related expenses due to 2019 salary increases and higher average headcount to support our increased research and development activities; a \$0.7 million increase in consulting and professional fees; a \$0.2 million increase in laboratory supplies and reagent expenses; and a \$0.6 million net increase in other expenses.

**General and Administrative Expenses**

General and administrative expenses increased by \$2.6 million to \$9.7 million for the six months ended June 30, 2019, from \$7.1 million for the six months ended June 30, 2018. This increase was attributable to: a \$1.4 million increase in compensation, benefits, non-cash stock-based compensation and other employee-related expenses due to 2019 salary increases and higher average headcount to support our increased activities; a \$0.5 million increase in consulting and professional fees related to pre-commercialization activities; a \$0.3 million increase in legal, audit and insurance costs; a \$0.2 million increase to patent costs; and a \$0.2 million net increase in other expenses.

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**Other Income (Expense), Net**

Other income (expense), net increased by \$1.4 million to \$2.0 million in other income, net during the six months ended June 30, 2019, from \$0.6 million in other income, net for the six months ended June 30, 2018. This increase was due primarily related to an increase in interest income.

**Liquidity and Capital Resources**

**Overview**

We have funded our operations from inception through June 30, 2019 primarily through the public offerings and the private placement of our securities and revenue from our collaboration with Merck. As of June 30, 2019, we had received an aggregate of \$375.3 million in net proceeds from the issuance of equity and debt securities and \$20.0 million in payments in connection with our collaboration and license agreement with Merck. As of June 30, 2019, we had cash and cash equivalents of \$173.6 million.

In July 2019, we completed a follow-on public offering of 4,600,000 shares of common stock, including the full exercise of the underwriters' option to purchase an additional 600,000 shares, at \$32.50 per share and received aggregate net proceeds of \$140.2 million, after deducting \$9.0 million of underwriting discounts and commissions and approximately \$0.3 million of offering expenses.

**Cash Flows**

The following table provides information regarding our cash flows:

	Six Months Ended	
	June 30,	
	2019	2018
	(in thousands)	
Net cash provided by (used in):		
Operating activities	\$ (36,389)	\$ (29,043)
Investing activities	(416)	(558)
Financing activities	586	54,277
Net increase (decrease) in cash	<u>\$ (36,219)</u>	<u>\$ 24,676</u>

### ***Net Cash Used in Operating Activities***

Cash flows used in operating activities represent 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 \$36.4 million for the six months ended June 30, 2019 compared to \$29.0 million for the six months ended June 30, 2018. The increase in net cash used in operations was attributable to: a \$8.7 million increase in our net loss as a result of higher operating expenses, primarily in connection with our zilucoplan development program and other research and development pipeline programs; a net decrease in operating liabilities; partially offset by a net decrease in operating assets and higher non-cash expenses, including stock-based compensation and depreciation.

### ***Net Cash Used in Investing Activities***

Net cash used in investing activities was \$0.4 million for the six months ended June 30, 2019 compared to approximately \$0.6 million for the six months ended June 30, 2018. The decrease in cash used in investing activities was due primarily to a decrease in purchases of property and equipment.

### ***Net Cash Provided by Financing Activities***

Net cash provided by financing activities was approximately \$0.6 million for the six months ended June 30, 2019 compared to \$54.3 million for the six months ended June 30, 2018. The decrease in cash provided by financing activities was due primarily to the \$54.5 million in proceeds raised in the comparative period from the February 2018 follow-on offering and \$0.6 million in payments related to restricted stock units vesting; partially offset by an increase of \$1.6 million in proceeds from exercises of stock option and purchases of shares under our Employee Stock Purchase Plan.

### ***Funding Requirements***

We expect our expenses to increase in connection with our ongoing development activities, particularly as we advance the Phase 3 clinical program for zilucoplan, continue clinical trials of zilucoplan in additional indications, advance the development of our pipeline programs, initiate new research and 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.

As of June 30, 2019, we had cash and cash equivalents of \$173.6 million, which, in addition to the net proceeds of \$140.2 million from our follow-on public offering closed in July 2019, is expected to fund operating expenses and capital expenditure requirements through at least the end of 2021. 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, and research and pre-clinical development efforts for, our current and future product candidates;
- 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;

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- our headcount growth and associated costs as we expand our research and development and establish a commercial infrastructure; and
- the costs of preparing, filing and prosecuting patent applications, maintaining and protecting our intellectual property rights and defending against intellectual property related claims.

Identifying potential product candidates and conducting pre-clinical 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.

### **Off-Balance Sheet Arrangements**

As of June 30, 2019, 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” to our condensed consolidated financial statements included elsewhere in this Quarterly Report on Form 10-Q.

### **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 June 30, 2019, we had cash and cash equivalents of \$173.6 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 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 June 30, 2019, 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 June 30, 2019 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 June 30, 2019.

**(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 June 30, 2019 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, 2018, which could materially affect our business, financial condition, and/or future results. The risks described below and 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, 2018.

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

*Recent Sales of Unregistered Securities*

None.

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**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 \(File No. 001-37926\) 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 \(File No. 001-37926\) filed November 29, 2016\).](#)
- 10.1 [Open Market Sales Agreement<sup>SM</sup>, dated as of May 9, 2019, by and between the Registrant and Jefferies LLC \(incorporated by reference to Exhibit 10.1 to the Registrant's Current Report on Form 8-K \(File No. 001-37926\) filed May 9, 2019\).](#)
- 10.2\*# [Non-Employee Director Compensation Policy, effective April 1, 2019.](#)
- 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.

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\* Filed herewith.

\*\* Furnished herewith.

# Indicates a management contract or any compensatory plan, contract or arrangement.

**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: August 7, 2019

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)

## RA PHARMACEUTICALS, INC.

## NON-EMPLOYEE DIRECTOR COMPENSATION POLICY

The purpose of this Non-Employee Director Compensation Policy of Ra Pharmaceuticals, Inc. (the “Company”), is to provide a total compensation package that enables the Company to attract and retain, on a long-term basis, high-caliber directors who are not employees or officers of the Company or its subsidiaries. In furtherance of the purpose stated above, effective April 1, 2019, all non-employee directors shall be paid compensation for services provided to the Company as set forth below:

**Cash Retainers**

The below annual retainers for general availability and participation in meetings and conference calls of our Board of Directors and its committees, as applicable, are to be paid quarterly, pro rated based on the number of actual days served by the director during such calendar quarter.

	<b>Annual Retainer</b>
<b>Board of Directors:</b>	
Non-Executive Chairman	\$ 65,000
Other Non-Employee Directors	\$ 40,000
<b>Audit Committee:</b>	
Committee Chairman	\$ 15,000
Other Committee Members	\$ 7,500
<b>Compensation Committee:</b>	
Committee Chairman	\$ 10,000
Other Committee Members	\$ 5,000
<b>Nominating and Corporate Governance Committee:</b>	
Committee Chairman	\$ 8,000
Other Committee Members	\$ 4,000

**Equity Retainers**

**Initial Award:** An initial, one-time equity award (the “Initial Award”) of 25,000 shares to each new non-employee director upon his or her election to the Board of Directors, which shall vest annually over three years, provided, however, that all vesting shall cease if the director resigns from the Board of Directors or otherwise ceases to serve as a director of the Company, unless the Board of Directors determines that the circumstances warrant continuation of vesting. This Initial Award applies only to non-employee directors who are first elected to the Board of Directors effective as of or subsequent to the Company’s initial public offering. If the Initial Award is in the form of a stock option, such stock option shall have a per share exercise price equal to the Fair Market Value (as defined in the Company’s 2016 Stock Option and Incentive Plan) of the Company’s common stock on the date of grant.

**Annual Award:** On each date of the Company’s Annual Meeting of Stockholders following the completion of the Company’s initial public offering (the “Annual Meeting”), each continuing non-employee member of the Board of Directors who has served as a director for

the previous three months will receive an annual equity award (the “Annual Award”) of 15,000 shares, which shall vest in full upon the earlier to occur of the first anniversary of the date of grant or the date of the next Annual Meeting; provided, however, that all vesting shall cease if the director resigns from the Board of Directors or otherwise ceases to serve as a director, unless the Board of Directors determines that the circumstances warrant continuation of vesting. If the Annual Award is in the form of a stock option, such stock option shall have a per share exercise price equal to the Fair Market Value (as defined in the Company’s 2016 Stock Option and Incentive Plan) of the Company’s common stock on the date of grant.

**Expenses**

The Company will reimburse all reasonable out-of-pocket expenses incurred by non-employee directors in attending meetings of the Board or any Committee.

Effective April 1, 2019

## 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: August 7, 2019

/s/ Douglas A. Treco

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Douglas A. Treco, Ph.D.

President and Chief Executive Officer

## 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: August 7, 2019

/s/ David C. Lubner

David C. Lubner

Executive Vice President and Chief Financial Officer

**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 June 30, 2019, 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 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: August 7, 2019

/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