
**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, 2017

OR

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

For the transition period from _____ to _____

Commission File Number: 001-37926



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)

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

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

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

Large accelerated filer
Non-accelerated filer
(Do not check if a smaller reporting company)

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 30, 2017 was 22,626,684.

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 are based on information available at the time those statements are made and/or management’s good faith belief as of that time with respect to future events, and are subject to risks and uncertainties that could cause actual performance or results to differ materially from those expressed in or suggested by the forward-looking statements.

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 intended to identify forward-looking statements, and similar expressions and comparable terminology intended to identify forward-looking statements. These statements reflect our current views with respect to future events and are based on assumptions and subject to risks and uncertainties, including those set forth in Item 1A, “Risk Factors” and elsewhere in our Annual Report on Form 10-K for the year ended December 31, 2016, as supplemented by our subsequent filings with the Securities and Exchange Commission (the “SEC”). Forward-looking statements include, but are not limited to, statements about:

- the initiation, timing, progress and results of our research and development programs and future preclinical and clinical studies;
- the risk that initial data from our ongoing Phase 2 clinical trial in paroxysmal nocturnal hemoglobinuria (“PNH”) may not be indicative of final study results or data from other patients that we obtain from this clinical trial;
- 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;
- the commercialization, marketing and manufacturing of 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 expectations related to the use of proceeds from our initial public offering, and estimates of our expenses, future revenues, capital requirements and our needs for additional financing;
- 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 those listed under the caption “Risk Factors” in our Annual Report on Form 10-K for the year ended December 31, 2016.

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, 2017	December 31, 2016
Assets		
Current assets:		
Cash and cash equivalents	\$ 84,091	\$ 117,812
Prepaid expenses and other current assets	1,123	1,690
Total current assets	85,214	119,502
Property and equipment, net	5,967	5,537
Intangible assets, net	213	262
Goodwill	183	183
Restricted cash	1,334	1,334
Total assets	\$ 92,911	\$ 126,818
Liabilities and Stockholders' Equity		
Current liabilities:		
Accounts payable	\$ 3,377	\$ 3,252
Accrued expenses	3,994	3,182
Deferred rent	432	303
Total current liabilities	7,803	6,737
Deferred rent, net of current portion	2,472	2,800
Deferred tax liabilities	59	59
Total liabilities	10,334	9,596
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; 22,622 and 22,546 shares issued and outstanding as of September 30, 2017 and December 31, 2016, respectively	23	23
Additional paid-in capital	190,627	185,963
Accumulated deficit	(108,073)	(68,764)
Total stockholders' equity	82,577	117,222
Total liabilities and stockholders' equity	\$ 92,911	\$ 126,818

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,	
	2017	2016	2017	2016
Revenue	\$ —	\$ —	\$ —	\$ 4,928
Operating expenses:				
Research and development	13,130	7,079	32,606	18,541
General and administrative	2,284	1,042	7,101	3,418
Total operating expenses	15,414	8,121	39,707	21,959
Loss from operations	(15,414)	(8,121)	(39,707)	(17,031)
Other income (expense), net	139	7	409	(945)
Net loss	\$ (15,275)	\$ (8,114)	\$ (39,298)	\$ (17,976)
Net loss per common share — basic and diluted	\$ (0.68)	\$ (14.22)	\$ (1.74)	\$ (32.73)
Weighted average number of common shares outstanding — basic and diluted	22,614	571	22,579	549

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,	
	2017	2016
Cash flows from operating activities		
Net loss	\$ (39,298)	\$ (17,976)
Adjustments to reconcile net loss to net cash used in operating activities:		
Depreciation and amortization	1,121	1,001
Stock-based compensation	3,847	521
Change in fair value of preferred stock tranche rights	—	960
Other, net	8	15
Changes in operating assets and liabilities:		
Prepaid expenses and other current assets	430	131
Non-current assets	—	(925)
Accounts payable and accrued expenses	963	2,907
Deferred rent	(199)	2,409
Deferred revenue	—	(1,862)
Net cash used in operating activities	<u>(33,128)</u>	<u>(12,819)</u>
Cash flows from investing activities		
Purchase of property and equipment	(1,390)	(4,494)
Other, net	—	136
Net cash used in investing activities	<u>(1,390)</u>	<u>(4,358)</u>
Cash flows from financing activities		
Proceeds from issuance of preferred stock, net of underwriter discounts	—	29,250
Payment of preferred stock issuance costs	—	(22)
Proceeds from disgorgement of stockholder's short-swing profits	670	—
Proceeds from exercises of stock options	244	109
Other, net	(117)	(350)
Net cash provided by financing activities	<u>797</u>	<u>28,987</u>
Net increase (decrease) in cash and cash equivalents	(33,721)	11,810
Cash and cash equivalents, beginning of period	117,812	19,386
Cash and cash equivalents, end of period	<u>\$ 84,091</u>	<u>\$ 31,196</u>

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, 2016. 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, RA101495, 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 paroxysmal nocturnal hemoglobinuria (“PNH”), a rare, chronic, life-threatening, blood disorder where red blood cells are mistakenly attacked and destroyed by the complement system. The Company is also developing RA101495, administered SC, to treat other debilitating complement-mediated diseases such as generalized myasthenia gravis (“gMG”), atypical hemolytic uremic syndrome (“aHUS”), and lupus nephritis (“LN”). Additionally, the Company is pursuing discovery and preclinical programs targeting selective inhibition of other uncontrolled complement pathway factors to treat a variety of ophthalmologic, 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.

Since inception, the Company has generated an accumulated deficit of \$108.1 million as of September 30, 2017 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.

Initial Public Offering

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 (“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.

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.

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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.

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, 2016.

Newly Adopted Accounting Pronouncements

In March 2016, the Financial Accounting Standards Board ("FASB") issued Accounting Standards Update ("ASU") 2016-09, "*Improvements to Employee Share-Based Payment Accounting*." The standard reduces complexity in several aspects of the accounting for employee share-based compensation, including the income tax consequences, classification of awards as either equity or liabilities, and classification on the statement of cash flows. The ASU is effective for fiscal years beginning after December 15, 2016, and interim periods within those fiscal years. The adoption of ASU 2016-09 during the quarter ended March 31, 2017 did not have a significant impact on the Company's financial statements and related disclosures. Upon adoption, the Company elected to account for forfeitures when they occur and recorded a cumulative effect adjustment of \$11,200 to accumulated deficit.

In November 2015, the FASB issued ASU 2015-17, "*Balance Sheet Classification of Deferred Taxes*," that requires companies to classify all deferred tax assets and liabilities, along with any valuation allowance, as noncurrent on the balance sheet instead of separating deferred taxes into current and noncurrent amounts. The guidance does not change the existing requirement that only permits offsetting within a jurisdiction. The ASU is effective for fiscal years beginning after December 15, 2016, and interim periods within those fiscal years. The adoption of ASU 2015-17 during the quarter ended March 31, 2017 did not have a significant impact on the Company's financial statements and related disclosures.

Newly Issued Accounting Pronouncements

In May 2014, the FASB issued ASU 2014-09, "*Revenue from Contracts with Customers*." The standard, including subsequently issued amendments, will replace most existing revenue recognition guidance in U.S. GAAP when it becomes effective and permits the use of either the retrospective or cumulative effect transition method. The standard will require 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 will be 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 identified performance obligations were completed upon the expiration of the research term of the Merck Agreement in April 2016. See Note 5, "Revenue Recognition." The Company has not yet selected a transition method and is still evaluating the impact the adoption will have on its consolidated financial statements for the years ended December 31, 2016, 2015, 2014 and 2013 and related disclosures.

In February 2016, the FASB issued ASU 2016-02, "*Leases*." The standard established the principles that lessees and lessors will apply to report useful information to users of financial statements about the amount, timing and uncertainty of cash flows arising from a lease. The ASU is effective for fiscal years beginning after December 15, 2018, and interim periods within those fiscal years. 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.

In August 2016, the FASB issued ASU 2016-15, "*Classification of Certain Cash Receipts and Cash Payments*." The standard addresses the classification of certain transactions within the statement of cash flows, including cash payments for debt prepayment or debt extinguishment costs, contingent consideration payments made after a business combination, and distributions received from equity method investments. The ASU is effective for fiscal years beginning after December 15, 2017, and interim periods within those fiscal years. Early adoption is permitted. The adoption of this standard is not expected to have a material impact on the consolidated financial statements and related disclosures.

In November 2016, the FASB issued ASU 2016-18, "*Restricted Cash*." The standard addresses the classification and presentation of restricted cash and restricted cash equivalents within the statement of cash flows. The ASU is effective for fiscal years beginning after December 15, 2017, and interim periods within those fiscal years. Early adoption is permitted. The Company is still evaluating the impact this standard will have on its consolidated financial statements and related disclosures.

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In January 2017, the FASB issued ASU 2017-04, “*Simplifying the Test for Goodwill Impairment.*” The standard simplifies the accounting for goodwill impairment by removing Step 2 of the goodwill impairment test, which requires a hypothetical purchase price allocation. The ASU is effective for annual or interim goodwill impairment tests in fiscal years beginning after December 15, 2019, and should be applied on a prospective basis. Early adoption is permitted for interim or annual goodwill impairment tests performed on testing dates after January 1, 2017. The adoption of this standard is not expected to have a material impact on the consolidated financial statements and related disclosures.

In May 2017, the FASB issued ASU 2017-09, “*Scope of Modification Accounting.*” The standard clarifies when changes to the terms or conditions of a share-based payment award must be accounted for as modifications. The ASU is effective for fiscal years beginning after December 15, 2017, and interim periods within those fiscal years. Early adoption is permitted. The adoption of this standard is not expected to have a material impact on the consolidated financial statements and related disclosures.

2. Supplemental Balance Sheet Information

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

	September 30, 2017	December 31, 2016
Computer equipment and software	\$ 20	\$ —
Furniture, fixtures, and other	378	365
Laboratory equipment	5,090	3,642
Construction in progress	8	—
Leasehold improvements	3,744	3,732
	9,240	7,739
Accumulated depreciation	(3,273)	(2,202)
Property and equipment, net	<u>\$ 5,967</u>	<u>\$ 5,537</u>

Depreciation expense was \$0.4 million and \$0.3 million for the three months ended September 30, 2017 and 2016 and \$1.1 million and \$1.0 million for the nine months ended September 30, 2017 and 2016, respectively.

Accrued expenses consist of the following (in thousands):

	September 30, 2017	December 31, 2016
Payroll and employee-related costs	\$ 1,540	\$ 1,451
Research and development costs	2,284	1,326
Other	170	405
Total	<u>\$ 3,994</u>	<u>\$ 3,182</u>

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, 2017, 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.

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

	September 30, 2017			Total
	Level 1	Level 2	Level 3	
Cash equivalents — Money market funds	\$ 84,961	\$ —	\$ —	\$ 84,961
Total assets	\$ 84,961	\$ —	\$ —	\$ 84,961

	December 31, 2016			Total
	Level 1	Level 2	Level 3	
Cash equivalents — Money market funds	\$ 117,708	\$ —	\$ —	\$ 117,708
Total assets	\$ 117,708	\$ —	\$ —	\$ 117,708

4. Redeemable Convertible Preferred Stock

Series A Preferred Stock

In February 2010, the Company issued 12,887,999 shares of Series A Preferred Stock pursuant to the Series A Preferred Stock agreement at a price of \$0.80176 per share. Additionally, investors were granted the right to purchase up to an additional 21,552,566 shares of the Company's Series A Preferred Stock at a price of \$0.80176 per share, in two subsequent closings upon the Company meeting certain milestone criteria. In February 2012 and March 2014, the Board of Directors waived certain milestone events provided for in the Series A Preferred Stock agreement and the Company issued 10,776,283 and 10,776,283 shares, respectively, of Series A Preferred Stock at a price of \$0.80176 per share. In October 2016, upon the closing of the Company's IPO, all outstanding shares of Series A Preferred Stock converted into 4,920,074 shares of the Company's common stock.

Series B Preferred Stock

In July 2015, the Company issued 31,564,630 shares of Series B-1 Preferred Stock pursuant to the Series B Preferred Stock agreement at a price of \$0.92667 per share. Additionally, investors were granted the right to purchase up to an additional 29,362,452 shares of the Company's Series B-2 Preferred Stock at a price of \$0.99617, in any number of subsequent closings upon the request of each investor or in a mandatory closing upon the Company meeting certain milestone criteria. In June 2016, the Board of Directors and required certain investors waived certain milestone events provided for in the Series B Preferred Stock agreement and the Company issued 29,362,452 shares of Series B-2 Preferred Stock at a price of \$0.99617 per share. In October 2016, upon the closing of the Company's IPO, all outstanding shares of Series B-1 and B-2 Preferred Stock converted into 8,703,859 shares of the Company's common stock.

Series A and Series B-2 Preferred Stock Tranche Rights

The Company determined the rights of the investors to purchase additional shares of Series A and Series B-2 Preferred Stock met the definition of a freestanding financial instrument and were recognized as a liability at fair value upon the initial issuance of Series A and Series B-1 Preferred Stock in February 2010 and July 2015, respectively. The Company adjusted the carrying value of the Series A and Series B-2 Preferred Stock Tranche Rights liability to its estimated fair value at each subsequent reporting date and immediately prior to the subsequent issuances through charges to other income (expense), net in the condensed consolidated statement of operations. The Series A Preferred Stock Tranche Rights liability was extinguished in March 2014 and the Series B-2 Preferred Stock Tranche Rights liability was extinguished in June 2016. During the three months ended March 31, 2016 and June 30, 2016, the Company adjusted the Series B-2 Preferred Stock Tranche Rights liability to its fair value and recorded a charge of \$0.8 million and \$0.2 million, respectively.

5. Revenue Recognition

In April 2013, the Company entered into a multi-target collaboration and license agreement with Merck ("Merck Agreement") 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 agreement, 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.

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At the signing of the Merck Agreement, Merck made an upfront non-refundable, 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 milestone payments that could total up to \$65.0 million.

The Company has identified two deliverables in connection with the Merck Agreement: (1) rights to access the Company's technology platform for each program target, and (2) the research and development services provided during the research term. The Company has determined that none of the deliverables have standalone value. Since the separability criteria have not been met for any of the deliverables, the deliverables were accounted for as a single combined unit of accounting. The Company has recognized revenue in connection with the upfront non-refundable license fee ratably over the research term. Payments for research and development services and reimbursement for certain lab supplies and reagents have been recognized as services are performed.

The Company has determined that the \$3.5 million in milestone payments received was substantive in nature as they were commensurate with the enhancement of value resulting from the Company's performance under the Merck Agreement, related solely to past performance and were reasonable relative to all of the deliverables and payment terms within the arrangement. Accordingly, the Company has accounted for these milestone payments under the milestone method. The Company is entitled to receive future aggregate milestone payments of up to \$61.5 million for the non-complement cardiovascular target selected, consisting of remaining preclinical and clinical milestones of \$16.5 million, regulatory milestones of \$19.0 million, and commercial milestones of \$26.0 million, and low-to-mid single digit percentage royalties on future sales, if any. Following the end of the research term, any future milestone payments will be recognized as revenue upon achievement, assuming all other revenue recognition criteria are met, as no further performance obligations exist for the Company under the Merck Agreement.

During the three and nine months ended September 30, 2016, the Company recognized revenue of zero and \$4.2 million related to upfront, non-refundable payments, respectively. For the three and nine months ended September 30, 2016, the Company recognized zero and \$0.7 million related to research and development services and reimbursable expenses, respectively. No revenue was recognized during the nine months ended September 30, 2017.

6. 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,	
	2017	2016	2017	2016
Research and development	\$ 922	\$ 149	\$ 2,288	\$ 309
General and administrative	675	102	1,559	212
Total	<u>\$ 1,597</u>	<u>\$ 251</u>	<u>\$ 3,847</u>	<u>\$ 521</u>

During the nine months ended September 30, 2017, the Company issued 1.5 million stock options with a per share weighted average grant date fair value of \$11.76.

7. 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, 2017 and 2016 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.

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The following Common Stock equivalents, presented on an as converted basis, were excluded from the computation of diluted weighted average shares outstanding as their effect would be anti-dilutive (in thousands):

	As of September 30,	
	2017	2016
Convertible preferred stock	—	13,624
Common stock warrants	—	223
Stock options	3,358	2,095
Total	3,358	15,942

On April 1, 2015, the Company entered into a Convertible Note Purchase Agreement with the holders of Series A Preferred Stock and issued Convertible Notes and warrants to purchase 222,775 shares of the Company's Common Stock. In October 2016, upon the closing of the IPO, all of the outstanding warrants net exercised, in accordance with their terms, into 221,573 shares of Common Stock.

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, 2016. 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. 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, 2016, 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. 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, our 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 chemistry technology will allow us to pursue challenging targets for which only monoclonal antibodies have been developed.

We are developing our lead product candidate, RA101495, a convenient self-administered subcutaneous ("SC") injection, which is an injection into the tissue under the skin, for the treatment of paroxysmal nocturnal hemoglobinuria, or PNH. PNH is a rare, chronic, life-threatening, blood disorder where red blood cells are mistakenly attacked and destroyed by the complement system. We initiated our Phase 2 clinical program for RA101495 in PNH patients in the second quarter of 2017. The global, dose-finding, twelve-week open label Phase 2 program is designed to evaluate the safety, tolerability, preliminary efficacy, pharmacokinetics and pharmacodynamics of RA101495 in patients with PNH. The study evaluates RA101495 in three cohorts. Cohort A includes eculizumab-naïve patients, Cohort B includes patients switching from eculizumab to RA101495 and the third cohort includes patients who are currently treated with eculizumab, but have evidence of an inadequate response.

In June 2017, we released initial data from the Phase 2 program on two eculizumab-naïve patients treated with RA101495 with seven weeks of follow-up. Data showed no safety or tolerability concerns, no injection site reactions, near-complete inhibition of hemolytic activity, rapid declines in lactate dehydrogenase ("LDH") and 100% compliance with the once daily, subcutaneous self-administration dosing regimen. Based on these initial data, we commenced enrollment in Cohort B, comprised of patients switching from eculizumab to RA101495. As of November 9, 2017, 28 patients have been enrolled in the Phase 2 program. A total of 27 patients have been dosed with RA101495, including 10 patients in Cohort A, 16 patients in Cohort B, and 1 patient in the third cohort. We remain on track to report additional data from our Phase 2 program around year-end 2017.

We are also developing RA101495, administered SC, to treat other debilitating complement-mediated diseases such as generalized myasthenia gravis ("gMG"), atypical hemolytic uremic syndrome ("aHUS"), and lupus nephritis ("LN"). We expect to initiate a Phase 2 clinical trial with RA101495 for gMG and a Phase 1b clinical trial supporting development in aHUS and LN in the fourth quarter of 2017. Additionally, we are pursuing discovery and preclinical programs targeting selective inhibition of other uncontrolled complement pathway factors to treat a variety of ophthalmologic, 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").

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, RA101495. 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, 2017, we had received an aggregate of \$181.0 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, 2017, our principal source of liquidity was cash and cash equivalents, which totaled \$84.1 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

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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.

As of September 30, 2017, we had an accumulated deficit of \$108.1 million. Our net losses were \$39.3 million and \$18.0 million for the nine months ended September 30, 2017 and 2016, 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, RA101495, through clinical development by establishing clinical proof-of-concept activity using convenient SC administration in PNH patients, particularly as we continue our Phase 2 clinical trial in PNH;
- 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 believe that, based on our current operating plan, our available funds will enable us to fund our operating expenses and capital expenditure requirements through the fourth quarter of 2018. 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 obtain top-line data from our ongoing Phase 2 clinical trials of RA101495 for the treatment of PNH, initiate a Phase 2 in gMG and a Phase 1b supporting development in aHUS and LN, as well as 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 RA101495 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 our collaboration and license agreement with Merck ("the Merck Agreement") which we entered into 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, 2017. 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, 2016.

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

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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,	
	2017	2016	2017	2016
	(in thousands)			
RA101495	\$ 6,836	\$ 3,267	\$ 15,289	\$ 7,841
Other pipeline programs	956	51	2,460	343
Allocated costs	7,792	3,318	17,749	8,184
Unallocated costs	5,338	3,761	14,857	10,357
Total	<u>\$ 13,130</u>	<u>\$ 7,079</u>	<u>\$ 32,606</u>	<u>\$ 18,541</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.

Historically, we had not provided program costs because we have not tracked or recorded our research and development expenses on a program-by-program basis. Beginning in the first quarter of 2017, we began to allocate costs related to our third-party vendors directly to programs. Prior-period amounts in the table above were reclassified to conform to the current period’s presentation.

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;

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- 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 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 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, potential commercialization of our product candidates and increased costs of operating as a public company. 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. Additionally, we anticipate increased costs associated with being a public company, including expenses related to services associated with maintaining compliance with exchange listing and Securities and Exchange Commission (“the SEC”) requirements, director and officer insurance costs and investor and public relations costs.

Other Income (Expense), Net

Other income (expense), net primarily consists of interest income earned on our cash and cash equivalents and the increase in fair value of preferred stock tranche rights.

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.

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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) accrued 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, 2016.

Result of Operations

Three Months Ended September 30, 2017 and 2016

The following table summarizes our results of operations:

	Three Months Ended September 30,		\$ Change	% Change
	2017	2016		
	(in thousands, except percentages)			
Operating expenses:				
Research and development	13,130	7,079	6,051	85.5%
General and administrative	2,284	1,042	1,242	119.2%
Total operating expenses	15,414	8,121	7,293	89.8%
Loss from operations	(15,414)	(8,121)	(7,293)	89.8%
Other income (expense), net	139	7	132	1,885.7%
Net loss	\$ (15,275)	\$ (8,114)	\$ (7,161)	88.3%

Research and Development Expenses

Research and development expenses increased by approximately \$6.0 million to \$13.1 million for the three months ended September 30, 2017, from \$7.1 million for the three months ended September 30, 2016. This increase was attributable to a \$4.5 million increase in CRO and CMO expenses for our non-clinical studies and clinical trials, due primarily to our RA101495 program; a \$0.8 million increase in non-cash stock-based compensation, due primarily to our annual grants made in February 2017 and higher average stock price; a \$0.7 million increase in compensation, benefits, and other employee-related expenses due to higher average headcount to support our increased research and development activities and 2017 salary increases; and a \$0.2 million net increase in other expenses; partially offset by a \$0.2 million decrease in consulting and professional fees.

General and Administrative Expenses

General and administrative expenses increased by approximately \$1.3 million to \$2.3 million for the three months ended September 30, 2017, from \$1.0 million for the three months ended September 30, 2016. This increase was attributable to a \$0.6 million increase in non-cash stock-based compensation due primarily to our annual grants made in February 2017 and higher average stock price; a \$0.3 million increase in compensation, benefits, and other employee-related expenses due to higher average headcount to support our increased activities and 2017 salary increases; a \$0.2 million increase in insurance, legal and audit costs, primarily due to operating as a public company; and a \$0.2 million net increase in other expenses.

Other Income (Expense), Net

Other income (expense), net increased by \$0.1 million to \$0.1 million in other income, net for the three months ended September 30, 2017, from less than \$0.1 million in other expense, net for the three months ended September 30, 2016. This increase was due primarily to a \$0.2 million increase in interest income, partially offset by other expenses of less than \$0.1 million.

Nine months ended September 30, 2017 and 2016

The following table summarizes our results of operations:

	Nine Months Ended September 30,		\$ Change	% Change
	2017	2016		
	(in thousands, except percentages)			
Revenue	\$ —	\$ 4,928	\$ (4,928)	(100.0)%
Operating expenses:				
Research and development	32,606	18,541	14,065	75.9%
General and administrative	7,101	3,418	3,683	107.8%
Total operating expenses	39,707	21,959	17,748	80.8%
Loss from operations	(39,707)	(17,031)	(22,676)	133.1%
Other income (expense), net	409	(945)	1,354	(143.3)%
Net loss	\$ (39,298)	\$ (17,976)	\$ (21,322)	118.6%

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Revenue

Revenue decreased by \$4.9 million to zero for the nine months ended September 30, 2017, from \$4.9 million for the nine months ended September 30, 2016, which included a \$3.0 million pre-clinical milestone payment and \$1.9 million in upfront non-refundable license fees earned and reimbursement of research and development services provided by us in accordance with a pre-specified number of our FTE employees working under the Merck Agreement. The research term of the Merck Agreement expired in April 2016.

Research and Development Expenses

Research and development expenses increased by \$14.1 million to \$32.6 million for the nine months ended September 30, 2017, from \$18.5 million for the nine months ended September 30, 2016. This increase was attributable to a \$9.6 million increase in CRO and CMO expenses for our pre-clinical studies and clinical trials, related primarily to our RA101495 program; a \$2.4 million increase in compensation, benefits, and other employee-related expenses due to higher average headcount to support our increased research and development activities and 2017 salary increases; a \$2.0 million increase in non-cash stock-based compensation due primarily to our annual grants made in February 2017 and higher average stock price; and a \$0.5 million increase in laboratory supply and reagent expenses; a \$0.2 million net increase in other expenses; partially offset by a \$0.6 million decrease in consulting and legal fees.

General and Administrative Expenses

General and administrative expenses increased by \$3.7 million to \$7.1 million for the nine months ended September 30, 2017, from \$3.4 million for the nine months ended September 30, 2016. This increase was attributable to a \$1.3 million increase in non-cash stock-based compensation due primarily to our annual grants made in February 2017 and higher average stock price; a \$0.8 million increase in compensation, benefits, and other employee-related expenses due primarily to higher average headcount to support our increased activities and 2017 salary increases; a \$0.8 million increase in insurance, legal and audit costs, primarily due to operating as a public company; a \$0.5 million increase in patent costs; and a \$0.5 million net increase in other expenses; partially offset by a \$0.2 million decrease in consulting and professional fees due primarily to lower costs associated with market research.

Other Income (Expense), Net

Other income (expense), net increased by approximately \$1.3 million to \$0.4 million in other income, net for the nine months ended September 30, 2017, from approximately \$0.9 million in other expense, net for the nine months ended September 30, 2016. This increase was due primarily to the Series B-2 Preferred Stock Tranche Rights fair value adjustment of \$1.0 million recorded during the nine months ended September 30, 2016 and a \$0.4 million increase in interest income, partially offset by a \$0.1 million in net other expense.

Liquidity and Capital Resources

Overview

We have funded our operations from inception through September 30, 2017 primarily through the public offering and the private placement of our securities and revenue from our collaboration with Merck. As of September 30, 2017, we had received an aggregate of \$181.0 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, 2017, we had cash and cash equivalents of \$84.1 million.

Cash Flows

The following table provides information regarding our cash flows:

	Nine Months Ended September 30,	
	2017	2016
	(in thousands)	
Net cash provided by (used in):		
Operating activities	\$ (33,128)	\$ (12,819)
Investing activities	(1,390)	(4,358)
Financing activities	797	28,987
Net increase (decrease) in cash	\$ (33,721)	\$ 11,810

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, stock-based compensation and changes in fair value of preferred stock tranche rights 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 \$33.1 million for the nine months ended September 30, 2017 compared to \$12.8 million for the nine months ended September 30, 2016. The increase in net cash used in operations was attributable primarily to a \$21.3 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 RA101495 program and other research and development pipeline programs; and a net decrease in operating assets and net increase in operating liabilities; partially offset by higher non-cash expenses, including depreciation, amortization and stock-based compensation.

Net Cash Used in Investing Activities

Net cash used in investing activities was \$1.4 million for the nine months ended September 30, 2017 compared to \$4.4 million for the nine months ended September 30, 2016. 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 \$0.8 million for the nine months ended September 30, 2017 compared to \$29.0 million for the nine months ended September 30, 2016. The decrease in cash provided by financing activities was due primarily to the Series B-2 Preferred Stock financing completed in June 2016, which resulted in net proceeds of \$29.2 million; partially offset by proceeds of \$0.7 million from the disgorgement of a stockholder's short-swing profits and higher proceeds from exercises of stock options.

Funding Requirements

We expect our expenses to increase in connection with our ongoing activities, particularly as we continue our Phase 2 clinical trials of RA101495 in PNH, initiate clinical trials of RA101495 in additional indications, including gMG, aHUS, and LN, 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.

On October 31, 2016, we completed an 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. We believe that, based on our current operating plan, our existing cash and cash equivalents as of September 30, 2017, will enable us to fund our operating expenses and capital expenditure requirements through the fourth quarter of 2018. 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 RA101495 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 RA101495;
- 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, 2016. 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, 2017, 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, 2017, we had cash and cash equivalents of \$84.1 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, 2017, 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, 2017 had a significant impact on our results of operations or financial condition.

Item 4. Controls and Procedures

(a) Evaluation of Disclosure Controls and Procedures

We have established disclosure controls and procedures designed to ensure that information required to be disclosed in the reports that we file or submits under the Securities Exchange Act of 1934, as amended (Exchange Act) is recorded, processed, summarized and reported within the time periods specified in the SEC’s rules and forms and is accumulated and communicated to management, including the principal executive officer (our Chief Executive Officer) and principal financial officer (our Chief Financial Officer), to allow timely decisions regarding required disclosure.

Our management, under the supervision and 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

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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, 2017.

(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 the Exchange Act Rules 13a-15 or 15d-15 that occurred during the quarter ended September 30, 2017 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, 2016, 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, 2016 except as described below.

We face substantial competition, which may result in others discovering, developing or commercializing products before or more successfully than we do, and reducing or eliminating our commercial opportunity.

The development and commercialization of new products is highly competitive. We expect that we, and any future collaborators, will face significant competition from major pharmaceutical companies, specialty pharmaceutical companies and biotechnology companies worldwide with respect to any of our product candidates that we, or any future collaborators, may seek to develop or commercialize in the future, including from drugs that act through the complement system and drugs that use different approaches. The principal competitor for our program in PNH is eculizumab, a C5 inhibitor, which is marketed as Soliris by Alexion Pharmaceuticals and is the only drug approved for the treatment of PNH. Alexion Pharmaceuticals is also developing a next-generation C5 inhibitor named ALXN 1210 that is designed to use a less frequent intravenous dosing schedule. We are also aware that there are a number of other companies that are actively developing product candidates for the treatment of PNH, including a product candidate directed at complement component 3, or C3, inhibition that is currently in preclinical development by Amyndas Pharmaceuticals, a product candidate directed at C3 inhibition such as APL-2 that is currently in clinical development by Apellis Pharmaceuticals, product candidates directed at C5 inhibition such as ALN-CC5, an RNAi therapeutic targeting the production of C5 being developed by Alnylam that is in early clinical trials, Coversin, a small protein inhibitor of C5 being developed by Akari Pharmaceuticals that is in early clinical trials, LFG316, a monoclonal antibody inhibitor of C5 being developed by Novartis Pharma, a biosimilar product candidate ABP595 being developed by Amgen that is currently in clinical trials, RO7112689, a monoclonal antibody inhibitor of C5 being developed by F. Hoffmann-La Roche, and other product candidates directed at other mechanisms of complement inhibition such as TNT009, an antibody against C1s, being developed by True North Therapeutics (acquired by Bioverativ) in early clinical trials, and ACH-4471, an orally available small molecule inhibitor of complement Factor D, that is currently in development by Achillion Pharmaceuticals.

MG is currently treated with cholinesterase inhibitors and non-specific immunosuppressive agents, including azathioprine, cyclophosphamide, cyclosporine, intravenous immunoglobulin, or IVIG, mycophenolate, prednisone, and tacrolimus. Alexion Pharmaceuticals recently announced approval of eculizumab for the treatment of refractory MG in Europe and gMG in the United States. Both rituximab, marketed by F. Hoffmann-La Roche, and belimumab, marketed by GlaxoSmithKline, which target B cell activity, are in clinical development for gMG. Anti-CD40, being developed as CFZ533 by Novartis Pharma, bortezomib, and the FcRN agonist ARGX-113 developed by Argen-X, are being tested in clinical trials in gMG. A therapeutic vaccine targeting B and T-cell receptors (CV-MG-01) is in early clinical testing for gMG.

In addition, we are aware that there are a number of companies that are actively developing product candidates that are in clinical development for the treatment of geographic atrophy, or GA, a more severe form of AMD to which dry AMD progresses, including lampalizumab, a Factor D complement inhibitor for the treatment of GA being developed by F. Hoffmann-La Roche that is in Phase 3 clinical trials, LFG316, an anti-C5 monoclonal antibody being developed by Novartis Pharma that has recently completed a 12 month Phase 2 study and failed to demonstrate efficacy (effectiveness) against the development of geographic atrophy, the end stage of dry age-related macular degeneration, Zimura, a C5 inhibitor being developed by Ophthotech that is entering Phase 2/3 clinical trials, APL-2, a C3 inhibitor developed by Apellis Pharmaceuticals in phase 2 clinical trial, and other product candidates that

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do not target the complement system that are in Phase 2 or Phase 3 clinical trials, including compounds being developed by Acucela, Allergan, GlaxoSmithKline and Novartis Pharma.

Our competitors may succeed in developing, acquiring or licensing technologies and products that are more effective, have fewer side effects or more tolerable side effects or are less costly than any product candidates that we are currently developing or that we may develop, which could render our product candidates obsolete and noncompetitive.

Our commercial opportunity could be reduced or eliminated if our competitors develop and commercialize products that are safer, more effective, have fewer or less severe side effects, are more convenient or are less expensive than any products that we, or any future collaborators, may develop. Our competitors also may obtain FDA or other marketing approval for their products before we, or any future collaborators, are able to obtain approval for ours, which could result in our competitors establishing a strong market position before we, or any future collaborators, are able to enter the market.

Many of our existing and potential future competitors have significantly greater financial resources and expertise in research and development, manufacturing, preclinical testing, conducting clinical trials, obtaining marketing approvals and marketing approved products than we do, and may be able to reduce the price at which they sell their products. Mergers and acquisitions in the pharmaceutical and biotechnology industries may result in even more resources being concentrated among a smaller number of our competitors. Smaller or early stage companies may also prove to be significant competitors, particularly through collaborative arrangements with large and established companies. These competitors also compete with us in recruiting and retaining qualified scientific and management personnel and establishing clinical trial sites and patient registration for clinical trials, as well as in acquiring technologies complementary to, or necessary for, the development of our product candidates.

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 in our IPO and, in November 2016, we issued and sold 1,057,385 shares of common stock to the underwriters under the underwriters' option to purchase additional shares of common stock, 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 26, 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 \$2.4 million, were approximately \$95.6 million. 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.

As of September 30, 2017, we used \$11.5 million of the net proceeds from our IPO.

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Item 6. Exhibits

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.

* The certifications furnished in Exhibit 32.1 hereto are deemed to accompany this Quarterly Report on Form 10-Q and will not be deemed "filed" for purposes of Section 18 of the Securities Exchange Act of 1934, as amended, except to the extent that the Registrant specifically incorporates it by reference. Such certifications will not be deemed to be incorporated by reference into any filings under the Securities Act of 1933, as amended, or the Securities Exchange Act of 1934, as amended, except to the extent that the Registrant specifically incorporates it by reference.

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 9, 2017

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)

**CERTIFICATION OF PRINCIPAL EXECUTIVE OFFICER PURSUANT TO
RULE 13a-14(a) / RULE 15d-14(a) OF THE SECURITIES EXCHANGE ACT OF 1934, AS AMENDED**

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)) 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. (Paragraph omitted pursuant to SEC Release Nos. 33-8238/34-47986 and 33-8392/34-49313);
 - 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 9, 2017

/s/ Douglas A. Treco

Douglas A. Treco, Ph.D.

President and Chief Executive Officer

**CERTIFICATION OF PRINCIPAL FINANCIAL OFFICER PURSUANT TO
RULE 13a-14(a) / RULE 15d-14(a) OF THE SECURITIES EXCHANGE ACT OF 1934, AS AMENDED**

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)) 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. (Paragraph omitted pursuant to SEC Release Nos. 33-8238/34-47986 and 33-8392/34-49313);
 - 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 9, 2017

/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 September 30, 2017, 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 9, 2017

/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
