
**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, 2016

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, or a smaller reporting company. See definitions of "accelerated filer," "large accelerated filer" and "smaller reporting company" in Rule 12b-2 of the Exchange Act. (Check one):

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

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 November 29, 2016: 22,546,165 shares.

INFORMATION REGARDING FORWARD-LOOKING STATEMENTS

This Quarterly Report on Form 10-Q contains “forward-looking statements” that involve substantial risks and uncertainties. All statements other than statements of historical facts contained in this Quarterly Report on Form 10-Q, including statements regarding our future results of operations and financial position, strategy and plans and our expectations for future operations, are forward-looking statements within the meaning of Section 27A of the Securities Act of 1933, as amended, and Section 21E of the Securities Exchange Act of 1934, as amended. The words “anticipate,” “believe,” “contemplates,” “continue,” “could,” “design,” “estimate,” “expect,” “intend,” “likely,” “may,” “ongoing,” “plan,” “potential,” “predict,” “project,” “will,” “would,” “seek,” “should,” “target,” or the negative version of these words and similar expressions are intended to identify forward-looking statements. We have based these forward-looking statements on our current expectations and projections about future events and trends that we believe may affect our financial condition, results of operations, strategy, short- and long-term business operations and objectives and financial needs. These forward-looking statements are subject to a number of risks, uncertainties, assumptions and other important factors, including those described in the section titled “Risk Factors” and “Management’s Discussion and Analysis of Financial Condition and Results of Operations” included in this Quarterly Report on Form 10-Q and our final prospectus dated October 25, 2016, filed pursuant to Rule 424(b)(4) under the Securities Act of 1933 on October 26, 2016. In light of these risks, uncertainties, assumptions and other factors, the forward-looking events and circumstances discussed in this Quarterly Report on Form 10-Q or our final prospectus dated October 25, 2016, filed pursuant to Rule 424(b)(4) under the Securities Act of 1933 on October 26, 2016 may not occur, and actual results could differ materially and adversely from those anticipated or implied in the forward-looking statements. Given these uncertainties, you should not place undue reliance on these forward-looking statements.

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;
- 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.”

TABLE OF CONTENTS

	<u>Page</u>
PART I — FINANCIAL INFORMATION	
Item 1. Financial Statements (Unaudited)	
Condensed Consolidated Balance Sheets as of September 30, 2016 and December 31, 2015	4
Condensed Consolidated Statements of Operations for the Three and Nine Months Ended September 30, 2016 and 2015	5
Condensed Consolidated Statements of Cash Flows for the Nine Months ended September 30, 2016 and 2015	6
Notes to Condensed Consolidated Financial Statements	7
Item 2. Management’s Discussion and Analysis of Financial Condition and Results of Operations	24
Item 3. Quantitative and Qualitative Disclosures About Market Risk	33
Item 4. Controls and Procedures	34
PART II — OTHER INFORMATION	35
Item 1. Risk Factors	35
Item 2. Unregistered Sales of Equity Securities and Use of Proceeds	78
Item 6. Exhibits	78
SIGNATURES	80

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

	September 30, 2016	December 31, 2015
Assets		
Current assets:		
Cash and cash equivalents	\$ 31,196	\$ 19,386
Accounts receivable	—	6
Prepaid expenses and other current assets	669	794
Total current assets	31,865	20,186
Property and equipment, net	5,673	2,143
Intangible assets, net	278	327
Goodwill	183	183
Restricted cash	1,334	1,463
Other non-current assets	1,315	40
Total assets	<u>\$ 40,648</u>	<u>\$ 24,342</u>
Liabilities, redeemable convertible preferred stock and stockholders' deficit		
Current liabilities:		
Accounts payable	\$ 2,775	\$ 1,319
Accrued expenses	2,735	1,274
Deferred rent	219	104
Deferred revenue	—	1,621
Preferred stock tranche rights	—	2,620
Total current liabilities	5,729	6,938
Non-current liabilities:		
Deferred rent, net of current portion	2,904	610
Deferred tax liabilities	77	77
Deferred revenue, net of current portion	—	241
Total liabilities	8,710	7,866
Commitments and contingencies (Note 8)		
Redeemable convertible preferred stock:		
Series A redeemable convertible preferred stock, \$0.001 par value, 34,440,565 shares authorized, issued, and outstanding at September 30, 2016 and December 31, 2015; aggregate liquidation preference of \$27,613 at September 30, 2016 and December 31, 2015	27,311	27,311
Series B-1 redeemable convertible preferred stock, \$0.001 par value, 31,564,630 shares authorized, issued, and outstanding at September 30, 2016 and December 31, 2015; aggregate liquidation preference of \$29,250 at September 30, 2016 and December 31, 2015	26,364	26,364
Series B-2 redeemable convertible preferred stock, \$0.001 par value, 29,362,452 shares authorized at September 30, 2016 and December 31, 2015; 29,362,452 and 0 shares issued and outstanding at September 30, 2016 and December 31, 2015; aggregate liquidation preference of \$29,250 and \$0 at September 30, 2016 and December 31, 2015	32,808	—
Stockholders deficit:		
Common stock, \$0.001 par value, 125,000,000 shares authorized at September 30, 2016 and December 31, 2015; 594,044 and 536,690 shares issued and outstanding at September 30, 2016 and December 31, 2015	1	1
Additional paid-in capital	3,330	2,700
Accumulated deficit	(57,876)	(39,900)
Total stockholders' deficit	(54,545)	(37,199)
Total liabilities, redeemable convertible preferred stock and stockholders' deficit	<u>\$ 40,648</u>	<u>\$ 24,342</u>

See accompanying notes to these unaudited condensed consolidated financial statements.

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

	<u>Three Months Ended September 30,</u>		<u>Nine Months Ended September 30,</u>	
	<u>2016</u>	<u>2015</u>	<u>2016</u>	<u>2015</u>
Revenue	\$ —	\$ 1,096	\$ 4,928	\$ 3,273
Operating expenses:				
Research & development	7,079	4,027	18,541	10,593
General & administrative	1,042	539	3,418	1,549
Total operating expenses	<u>8,121</u>	<u>4,566</u>	<u>21,959</u>	<u>12,142</u>
Loss from operations	(8,121)	(3,470)	(17,031)	(8,869)
Other income (expense):				
Interest (expense) with related parties	—	(14)	—	(159)
Loss on debt extinguishment with related parties	—	(602)	—	(602)
Other income (expense)	7	154	(945)	250
Total other income (expense), net	<u>7</u>	<u>(462)</u>	<u>(945)</u>	<u>(511)</u>
Net loss	<u>\$ (8,114)</u>	<u>\$ (3,932)</u>	<u>\$ (17,976)</u>	<u>\$ (9,380)</u>
Reconciliation of net loss to net loss attributable to common shareholders:				
Net loss	(8,114)	(3,932)	(17,976)	(9,380)
Gain on extinguishment of redeemable convertible preferred shares	—	1,673	—	1,673
Net loss attributable to common shareholders	<u>\$ (8,114)</u>	<u>\$ (2,259)</u>	<u>\$ (17,976)</u>	<u>\$ (7,707)</u>
Net loss per share attributable to common stockholders - basic and diluted	<u>\$ (14.22)</u>	<u>\$ (4.57)</u>	<u>\$ (32.73)</u>	<u>\$ (15.84)</u>
Weighted-average common shares used in net loss per share attributable to common stockholders - basic and diluted	<u>570,558</u>	<u>494,302</u>	<u>549,138</u>	<u>486,479</u>

See accompanying notes to these unaudited condensed consolidated financial statements

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

	Nine Months Ended September 30,	
	2016	2015
Cash flows from operating activities:		
Net loss	\$ (17,976)	\$ (9,380)
Adjustments to reconcile net loss to net cash used in operating activities:		
Depreciation and amortization	1,001	618
Accretion of discount and debt issuance costs of convertible notes with shareholders	—	50
Non-cash interest expense with shareholders	—	109
Loss on debt extinguishment with shareholders	—	602
Stock-based compensation	521	121
Change in fair value of preferred stock tranche rights	960	169
Change in fair value of derivative liability	—	(98)
Other non-cash operating activities	15	1
Changes in operating assets and liabilities:		
Prepaid expenses and other current assets	125	(300)
Accounts receivable	6	(1,443)
Other non-current assets	(925)	—
Accounts payable and accrued expenses	2,907	940
Deferred rent and other non current liabilities	2,409	(108)
Deferred revenue	(1,862)	(96)
Net cash used in operating activities	<u>(12,819)</u>	<u>(8,815)</u>
Cash flows from investing activities:		
Purchase of property and equipment	(4,494)	(152)
Proceeds from sale of property and equipment	7	1
Decrease (increase) in restricted cash	129	(1,334)
Net cash used in investing activities	<u>(4,358)</u>	<u>(1,485)</u>
Cash flows from financing activities:		
Proceeds from issuance of preferred stock	29,250	23,803
Payment of preferred stock issuance costs	(22)	(193)
Proceeds from issuance of convertible notes with shareholders	—	5,000
Payment of debt issuance costs	—	(6)
Payments of proposed public offering costs	(350)	—
Proceeds from exercise of common stock warrants	—	1
Proceeds from exercise of stock options	109	76
Net cash provided by financing activities	<u>28,987</u>	<u>28,681</u>
Net increase in cash and cash equivalents	11,810	18,381
Cash and cash equivalents, beginning of period	19,386	4,039
Cash and cash equivalents, end of period	<u>\$ 31,196</u>	<u>\$ 22,420</u>
Supplemental disclosure of noncash investing and financing activity:		
Reclassification of Series B-2 tranche right liability to the balance of Series B-2 redeemable convertible preferred stock	\$ 3,580	\$ —
Gain on extinguishment of Series A redeemable convertible preferred stock	\$ —	\$ 1,673
Conversion of convertible notes with shareholders into Series B-1 redeemable convertible preferred stock	\$ —	\$ 5,109
Changes in accounts payable and accrued expenses related to fixed asset additions	<u>\$ 10</u>	<u>\$ 26</u>

See accompanying notes to these unaudited condensed consolidated financial statements.

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

1. Nature of Business

Ra Pharmaceuticals, Inc. (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, or SC, injection, which is an injection into the tissue under the skin for the treatment of paroxysmal nocturnal hemoglobinuria, or 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 myasthenia gravis, or MG, and lupus nephritis, or LN. The Company is also 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 a collaboration with Merck & Co., Inc., or Merck, for a non-complement cardiovascular target.

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. The condensed consolidated financial statements include the assets and liabilities and operating results of the Company and its wholly-owned subsidiary, Cosmix. 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 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.

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 \$82.9 million after deducting \$6.4 million of underwriting discounts and commissions and offering costs of \$2.3 million. On November 29, 2016, we completed the sale of an additional 1,057,385 shares of common stock to the underwriters under the underwriters’ option in the IPO to purchase additional shares of common stock at the public offering price of \$13.00 per share, resulting in net proceeds to us of \$12.8 million after deducting underwriting discounts and commissions. The shares began trading on the NASDAQ Global Market on October 26, 2016. Upon the closing of the IPO, all of the outstanding shares of convertible preferred stock automatically converted into 13,623,933 shares of common stock at the applicable conversion ratio then in effect and the outstanding warrants to purchase 222,775 shares of common stock net exercised, in accordance with their terms, into 221,573 shares of common stock.

2. Summary of Significant Accounting Policies

Unaudited Interim Financial Information

The condensed consolidated financial statements of the Company included herein have been prepared, without audit, pursuant to the rules and regulations of the Securities and Exchange Commission (the “SEC”). Certain information and footnote disclosures normally included in financial statements prepared in accordance with U.S. GAAP have been condensed or omitted from this report, as is permitted by such rules and regulations. Accordingly, these financial statements should be read in conjunction with the financial statements as of and for the year ended December 31, 2015, and notes thereto, included in the Company’s final prospectus for the IPO filed with the Securities and Exchange Commission pursuant to Rule 424(b)(4) on October 26, 2016 (the “Prospectus”).

[Table of Contents](#)

Basis of Presentation and Use of Estimates

The accompanying condensed consolidated financial statements are unaudited. The Company's unaudited interim condensed consolidated financial statements have been prepared in accordance with accounting principles generally accepted in the United States ("U.S. GAAP"). Any reference in these notes to applicable guidance is meant to refer to the authoritative United States generally accepted accounting principles as found in the Accounting Standards Codification ("ASC") and Accounting Standards Updates ("ASU") of the Financial Accounting Standards Board ("FASB").

The unaudited interim condensed consolidated financial statements have been prepared on the same basis as the audited financial statements. The unaudited condensed consolidated financial statements include the accounts of Ra Pharmaceuticals, Inc. and its wholly owned subsidiary. All intercompany transactions and balances of the subsidiary have been eliminated in consolidation. In the opinion of the Company's management, the accompanying unaudited interim condensed consolidated financial statements contain all adjustments which are necessary to present fairly the Company's financial position as of September 30, 2016, the results of its operations for the three and nine months ended September 30, 2016 and 2015 and cash flows for the nine months ended September 30, 2016 and 2015. Such adjustments are of a normal and recurring nature. The results for the three and nine months ended September 30, 2016 are not necessarily indicative of the results for the year ending December 31, 2016, or for any future period.

The preparation of financial statements in conformity with U.S. GAAP requires management to make estimates and assumptions that affect the reported amounts in the financial statements and accompanying notes. Management considers many factors in selecting appropriate financial accounting policies and in developing the estimates and assumptions that are used in the preparation of the financial statements. Management must apply significant judgment in this process. In addition, other factors may affect estimates, which include, but are not limited to, expected business and operational changes, sensitivity and volatility associated with the assumptions used in developing estimates and whether historical trends are expected to be representative of future trends. Management's estimation process often may yield a range of potentially reasonable estimates and management must select an amount that falls within that range of reasonable estimates. On an ongoing basis, the Company's management evaluates its estimates related to, but not limited to, estimates related to convertible notes ("Convertible Notes"), bifurcated features embedded in the Convertible Notes, preferred stock tranche rights, stock-based compensation expense, clinical and pre-clinical trial accruals, and reported amounts of revenues and expenses during the period. The Company bases its estimates on historical experience and other market-specific or other relevant assumptions that it believes to be reasonable under the circumstances. Actual results may differ from those estimates or assumptions.

Reverse Stock Split

In connection with preparing for its IPO, the Company effected a one-for-seven reverse stock split of the Company's common stock. The reverse stock split became effective on October 14, 2016. The par value and authorized shares of common stock and convertible preferred stock were not adjusted as a result of the reverse stock split. All share and per share amounts within the financial statements and notes thereto have been retroactively adjusted for all periods presented to give effect to this reverse stock split, including reclassifying an amount equal to the reduction in par value of common stock to additional paid-in capital.

[Table of Contents](#)

Summary of Significant Accounting Policies

The Company's significant accounting policies are described in Note 2, "Summary of Significant Accounting Policies," in the Prospectus. There have been no material changes to the significant accounting policies during the period ended September 30, 2016.

Recent Accounting Pronouncements

In May 2014, the Financial Accounting Standards Board ("FASB") issued Accounting Standards Update ("ASU") No. 2014-09, *Revenue from Contracts with Customers* ("ASU 2014-09"), which supersedes existing revenue recognition guidance. The standard's core principle is that a company will recognize revenue when it transfers promised goods or services to customers in an amount that reflects the consideration to which the company expects to be entitled in exchange for those goods or services. The standard defines a five-step process to achieve this principle and will require companies to use more judgment and make more estimates than under the current guidance. The Company expects that these judgments and estimates will include identifying performance obligations in the customer contract, estimating the amount of variable consideration to include in the transaction price and allocating the transaction price to each separate performance obligation. ASU 2014-09 also requires additional disclosure about the nature, amount, timing and uncertainty of revenue and cash flows arising from customer contracts. In April 2016, the FASB issued ASU 2016-10, *Revenue From Contracts With Customers: Identifying Performance Obligations and Licensing* ("ASU 2016-10"), which addresses certain implementation issues and clarifies certain core revenue recognition principles of ASU 2014-09. In July 2015, the FASB voted to delay the effective date of this standard such that ASU 2014-09, as amended by ASU 2016-10, will be effective for the Company for annual periods beginning after December 15, 2017 and for interim periods within those fiscal years. Early adoption of the standard is permitted for annual periods beginning after December 15, 2016. The Company is evaluating the impact that the adoption of these ASUs will have on its financial statements.

In August 2014, the FASB issued ASU 2014-15, *Presentation of Financial Statements—Going Concern (Subtopic 205-40): Disclosure of Uncertainties about an Entity's ability to Continue as a Going Concern* ("ASU 2014-15"), which provides guidance in GAAP about management's responsibility to evaluate whether there is substantial doubt about an entity's ability to continue as a going concern and to provide related footnote disclosures. The amendments in ASU 2014-15 are effective for the annual reporting period ending after December 15, 2016 and for annual and interim periods thereafter. Early adoption is permitted. The Company is currently evaluating the potential impact of ASU 2014-15 on its financial statements.

In April 2015, the FASB issued ASU 2015-03, *Interest — Imputation of Interest (Subtopic 835-30): Simplifying the Presentation of Debt Issuance Costs* ("ASU 2015-03"), which changes the presentation of debt issuance costs in financial statements. Under this authoritative guidance, an entity presents such costs in the balance sheet as a direct deduction from the related debt liability rather than as an asset. Amortization of the costs is reported as interest expense. The new guidance was effective for the Company beginning January 1, 2016. Early adoption was permitted. ASU 2015-03 did not have a significant impact on the Company's financial statements.

In November 2015, the FASB issued ASU 2015-17, *Income Taxes (Topic 740): Balance Sheet Classification of Deferred Taxes* ("ASU 2015-17"), which changes the classification of deferred taxes in financial statements. This update requires that deferred tax liabilities and assets be classified as noncurrent on the balance sheet. The current requirement that deferred tax liabilities and assets of each jurisdiction of an entity be offset and presented as a single amount is not affected by the amendments in this update. The amendments in ASU 2015-17 are effective for annual periods beginning after December 15, 2016, and interim periods within those annual periods. Early adoption is permitted. The Company does not expect ASU 2015-17 to have an impact on its financial statements.

[Table of Contents](#)

In February 2016, the FASB issued ASU 2016-02, *Leases (Topic 842)* (“ASU 2016-02”), which changes the presentation of assets and liabilities relating to leases. The core principle of Topic 842 is that a lessee should recognize the assets and liabilities that arise from leases. All leases create an asset and a liability for the lessee in accordance with FASB Concepts Statement No. 6, *Elements of Financial Statements*, and, therefore, recognition of those lease assets and lease liabilities represents an improvement over previous GAAP, which did not require lease assets and lease liabilities to be recognized for most leases. The provisions of this guidance are effective for annual periods beginning after December 15, 2018, and for interim periods therein. The Company has not yet assessed the impact of this new standard on its financial statements.

In March 2016, the FASB issued ASU 2016-09, *Stock Compensation (Topic 718)* (“ASU 2016-09”), which is intended to simplify several aspects of the accounting for share-based payment award transactions. The guidance will be effective for the fiscal year beginning after December 15, 2016, including interim periods within that year. The Company is currently evaluating the potential impact ASU 2016-09 may have on its financial position.

3. Fair Value Measurements

Below is a summary of assets and liabilities measured at fair value as of September 30, 2016 and December 31, 2015 (in thousands):

	As of September 30, 2016			Total
	Quoted Prices in Active Markets (Level 1)	Significant Observable Inputs (Level 2)	Significant Unobservable Inputs (Level 3)	
Financial Assets				
Cash equivalents - Money market funds	\$ 30,958	\$ —	\$ —	\$ 30,958
Total	\$ 30,958	\$ —	\$ —	\$ 30,958
	As of December 31, 2015			Total
	Quoted Prices in Active Markets (Level 1)	Significant Observable Inputs (Level 2)	Significant Unobservable Inputs (Level 3)	
Financial Assets				
Cash equivalents - Money market funds	\$ 19,049	\$ —	\$ —	\$ 19,049
Total	\$ 19,049	\$ —	\$ —	\$ 19,049
Financial Liabilities				
Preferred stock tranche rights	\$ —	\$ —	\$ 2,620	\$ 2,620
Total	\$ —	\$ —	\$ 2,620	\$ 2,620

The Company’s cash equivalents consist principally of money market funds. Cash equivalents are stated at fair value and consist of Level 1 financial instruments in the fair value hierarchy.

Preferred Stock Tranche Rights are stated at fair value and are considered Level 3 inputs because their fair value measurement is based, in part, on significant inputs not observed in the market. The Company determined the fair value of Preferred Stock Tranche Rights as described in Note 9.

[Table of Contents](#)

4. Property and Equipment, net

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

	<u>As of</u> <u>September 30,</u> <u>2016</u>	<u>As of</u> <u>December 31,</u> <u>2015</u>
Computer equipment and software	\$ 43	\$ 71
Furniture, fixtures, and other	365	62
Laboratory equipment	3,503	2,941
Construction in progress	—	535
Leasehold improvements	<u>3,732</u>	<u>793</u>
	7,643	4,402
Accumulated depreciation	<u>(1,970)</u>	<u>(2,259)</u>
Property and equipment, net	<u>\$ 5,673</u>	<u>\$ 2,143</u>

Depreciation expense was \$0.3 million and \$0.2 million for the three months ended September 30, 2016 and 2015, respectively, and \$1.0 million and \$0.6 million for the nine months ended September 30, 2016 and 2015, respectively.

5. Accrued Expenses

Accrued expenses consist of the following (in thousands):

	<u>As of</u> <u>September 30,</u> <u>2016</u>	<u>As of</u> <u>December 31,</u> <u>2015</u>
Payroll and employee-related costs	\$ 805	\$ 60
Research and development costs	1,217	545
Consulting costs	135	122
Professional fees	493	62
Other	<u>85</u>	<u>485</u>
Total	<u>\$ 2,735</u>	<u>\$ 1,274</u>

6. Convertible Notes

On April 1, 2015, the Company entered into a Convertible Note Purchase Agreement (the “Note Agreement”) with the holders of its existing Series A Preferred Stock. Upon entering the Note Agreement, the Company issued Convertible Notes and warrants to purchase 221,521 shares of the Company’s common shares. In connection with the issuance, the Company received gross proceeds of \$5.0 million and incurred issuance costs of \$6,000. The Convertible Notes bore interest at a rate of 8% per annum and were payable in full at the earliest of March 2018 (“Maturity Date”), an event of default as defined in the Note Agreement, or the sale of the Company. As discussed in more detail below, the notes and accrued interest thereon converted into shares of Series B-1 Preferred Stock on July 10, 2015.

[Table of Contents](#)

The Convertible Notes included certain features resulting in automatic conversion or possible early redemption. In the event the Company sold or issued shares (“Equity Securities”) resulting in cash proceeds to the Company of no less than \$5 million (“Qualified Financing”), the Convertible Notes would automatically convert into the Equity Securities at a conversion price equal to the price per share paid by the investors purchasing the Equity Securities. In the event of the Company undergoing a change in control prior to the Maturity Date, the Convertible Notes would automatically convert into the right to receive 3 times the outstanding principal, plus accrued interest. Lastly, the Convertible Notes included a put feature, at the option of the holders, whereby upon an event of default, repayment of the Convertible Notes could be accelerated in the amount of outstanding principal, plus accrued interest. Each of these three features were embedded derivatives that required bifurcation and accounting as liabilities to be measured at fair value at each reporting date.

The embedded features requiring separate accounting were combined and valued upon issuance using a single income valuation approach. The Company estimated the fair value of the combined embedded derivative identified above using a “with and without” income valuation approach. Under this approach, the Company estimated the present value of the fixed interest rate debt based on the fair value of similar debt instruments excluding the embedded features. This amount was then compared to the fair value of the debt instrument including the embedded features using a probability weighted approach by assigning each embedded derivative feature a probability of occurrence, with consideration provided for the settlement amount including conversion terms, prepayment penalties, the expected life of the liability and the applicable discount rate.

The Company recorded approximately \$0.1 million as the fair value of the combined embedded derivative liability on April 1, 2015, resulting in a debt discount. The debt discount was being amortized as interest expense over the life of the Convertible Notes under the effective interest method prior to conversion. Changes in the estimated fair value of the embedded features were recorded as a component of other income (expense) in the condensed consolidated statement of operations.

As of July 10, 2015 and April 1, 2015, the Company ascribed a probability to the mandatory conversion feature upon a Qualified Financing of approximately 100% and 98%, respectively. As of July 10, 2015 and April 1, 2015, the Company ascribed a probability to the redemption feature upon a change in control of approximately 0% and 1%, respectively. For all other features included in the combined embedded derivative, the Company estimated a 0% and 1% probability of occurrence as of July 10, 2015 and April 1, 2015, respectively. The Company classified the liability within Level 3 of the fair value hierarchy as the probability factors are unobservable inputs and significant to the valuation model.

On July 10, 2015, the Company received gross proceeds of approximately \$24.1 million from the issuance of Series B-1 Preferred Stock to new and existing investors at a price per share of \$0.92667. This transaction was a Qualified Financing and resulted in the automatic conversion of the notes into 5,512,743 shares of Series B-1 Preferred Stock at a conversion price of \$0.92667 per share. Upon conversion, the Company recorded the difference between the fair value of the Series B-1 Preferred Stock issued and the carrying value of the Convertible Notes plus accrued interest as a \$0.6 million loss on debt extinguishment in its condensed consolidated statement of operations.

The warrants issued in connection with the Convertible Notes may be exercised at any time for shares of common stock at an exercise price of \$0.01 per share. The warrants expire in March 2022. The Company estimated the fair value of these warrants as of the issuance date using a Black-Scholes option pricing model with the following assumptions:

	<u>As of</u> <u>April 1, 2015</u>
Risk-free interest rate	1.65%
Expected dividend yield	0%
Expected term (in years)	7.0
Expected volatility	86.0%

The proceeds from the Convertible Notes and warrants issuance were allocated between the notes and the warrants on a relative fair value basis. The Company allocated \$0.5 million of the proceeds from the Convertible Notes to the common stock warrants and recognized this amount as additional paid-in capital with a corresponding debt discount upon issuance. As of September 30, 2016, the common stock warrants issued in connection with the Convertible Notes had not been exercised and remained outstanding. In October 2016, upon the closing of the IPO, all of the outstanding warrants net exercised, in accordance with their terms, into shares of common stock. See Note 9 for further details.

7. Commitments and Contingencies

Significant contracts and agreements

In April 2013, the Company entered into a collaboration and license agreement with Merck under which the Company granted Merck an exclusive license to develop, manufacture, and commercialize the Company's cyclomimetics compounds. See Note 11 for further details.

Lease commitments

In September 2010, the Company entered into a lease for approximately 6,500 square feet of research and development laboratories and general office space in One Kendall Square, Cambridge, Massachusetts. In September 2012, the Company amended the original lease to provide for additional space of approximately 4,000 square feet in the same facility. The Company's lease expired on April 30, 2016.

In September 2015, the Company entered into a new lease for approximately 27,000 square feet of research and development laboratories and general office space in 87 Cambridge Park Drive, Cambridge, Massachusetts. The lease commenced in September 2015 and expires in April 2023. The Company moved its operations to the space in April 2016.

In connection with the new lease, the Company recorded a lease incentive obligation for reimbursable costs under a tenant improvement allowance provided by the landlord. As of September 30, 2016 and December 31, 2015, the Company has recorded a lease incentive obligation of \$2.7 million and \$0.4 million in deferred rent in the condensed consolidated balance sheet.

The Company has accounted for all facility leases as operating leases. Rent expense was \$0.2 million and \$0.1 million for the three months ended September 30, 2016 and 2015, respectively, and \$0.8 million and \$0.2 million for the nine months ended September 30, 2016 and 2015, respectively.

Future minimum commitments due under these non-cancelable operating lease agreements as of September 30, 2016 are as follows (in thousands):

[Table of Contents](#)

<u>Years ending December 31,</u>	<u>Operating Leases</u>
2016 (remaining three months)	\$ 249
2017	1,222
2018	1,358
2019	1,396
2020	1,435
Thereafter	3,502
Total minimum lease payments	<u>\$ 9,162</u>

Restricted cash

As of September 30, 2016 and December 31, 2015, the Company had restricted cash of \$1.3 million and \$1.5 million, respectively. During the nine months ended September 30, 2016, restricted cash decreased pursuant to the return of \$0.1 million letter of credit for the Company's facility lease at One Kendall Square that expired during the period. During the year ended December 31, 2015, restricted cash increased pursuant to a letter of credit of \$1.4 million for the Company's facility lease at 87 Cambridge Park Drive in Cambridge, Massachusetts. Letters of credit are secured by cash equivalents held in a money market fund.

Litigation

The Company is not a party to any litigation and does not have contingency reserves established for any litigation liabilities.

8. 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, for gross proceeds of \$9.7 million, and the conversion of the principal and accrued interest totaling \$0.6 million related to convertible notes and incurred issuance costs of \$0.2 million, (the "First Tranche"). Additionally, in accordance with the terms of the Series A Preferred Stock agreement, 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, in two subsequent closings (the "Second Tranche" and "Third Tranche," respectively), upon the Company meeting certain milestone criteria by approval of the Board of Directors and approval of investors holding a majority of the Series A Preferred Stock (the "Series A Preferred Stock Tranche Rights").

In February 2012, the Board of Directors waived certain Second Tranche milestone events provided for in the Series A Preferred Stock agreement and the Company issued 10,776,283 shares of Series A Preferred Stock at a price of \$0.80176 for gross proceeds of \$8.6 million and incurred issuance costs of \$6,000.

In March 2014, the Board of Directors waived certain Third Tranche milestone events provided for in the Series A Preferred Stock agreement and the Company issued 10,776,283 shares of Series A Preferred Stock at a price of \$0.80176 for gross proceeds of \$8.6 million and incurred issuance costs of \$9,000.

[Table of Contents](#)

As of September 30, 2016 and December 31, 2015, the total authorized, outstanding and issued Series A Preferred Stock of the Company was 34,440,565 shares. 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 A Preferred Stock Tranche Rights

The Company determined the right of the investors to purchase shares of Series A Preferred Stock in two future tranches met the definition of a freestanding financial instrument and was recognized as a liability at fair value upon the initial issuance of the First Tranche of Series A Preferred Stock in February 2010. The Company adjusted the carrying value of the Series A Preferred Stock Tranche Rights liability to its estimated fair value at each subsequent reporting date and immediately prior to the issuance of the Second and Third Tranche of Series A Preferred Stock through charges to other income (expense) in the condensed consolidated statement of operations. Upon the closing of each tranche, the marked-to-market fair value of the liability was included in the carrying value of Series A Preferred Stock issued.

Immediately prior to the issuance of the Third Tranche of Series A Preferred Stock in March 2014, the Company adjusted the carrying value of the liability to its estimated fair value of \$3.4 million and recorded the adjustment of \$1.6 million within other income on the condensed consolidated statements of operations. The fair value of the Third Tranche obligation was determined using the fair value of the Series A Preferred Stock under the guideline public company option-pricing method on the date of the issuance.

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 for gross proceeds of \$24.1 million and the conversion of the principal and accrued interest totaling \$5.1 million related to the April 2015 Convertible Notes (Note 7) and incurred issuance costs of \$0.2 million (the "Series B-1 Closing").

Additionally, in accordance with the terms of the Series B Preferred Stock agreement, investors were granted the right to purchase up to an additional 29,362,452 shares of the Company's Series B-2 Preferred Stock ("Series B-2 Preferred Stock") at a price of \$0.99617 (the "Series B-2 Preferred Stock Tranche Rights"), in any number of subsequent closings upon the request of each investor (the "Subsequent Closings") or in a mandatory closing upon the Company meeting certain milestone criteria by approval of the Board of Directors and certain investors (the "Mandatory Closing"). Upon consummation of a Mandatory Closing, if an investor fails to purchase the full amount of Series B-2 Preferred Stock allocated to the investor in the Series B Preferred Stock agreement at the Mandatory Closing, then all shares of Series A Preferred Stock and Series B Preferred Stock held by such investor will automatically be converted into shares of common stock of the Company initially on a one for one basis. In June 2016, the Board of Directors and required certain investors waived the milestone criteria and triggered the Mandatory Closing of the Series B-2 Preferred Stock.

In June 2016, the Company issued 29,362,452 shares of Series B-2 Preferred Stock pursuant to the Series B Preferred Stock Agreement at a price of \$0.99617 per share, for gross proceeds of \$29.2 million and incurred issuance costs of \$22,000 ("the Series B-2 Closing").

[Table of Contents](#)

The Company incurred \$0.2 million of costs related to the issuance of the Series B-1 Preferred Stock. \$17,000 of the Series B-1 Preferred Stock issuance costs were allocated to Preferred Stock Tranche Rights relating to the Series B-2 Preferred Stock upon issuance. The amount allocated to the Series B-1 Preferred Stock was offset against the proceeds upon closing of the issuance of the first tranche of Series B Preferred Stock. The amount allocated to the future tranche rights was recorded as an offset to the tranche right liability at issuance. The Company incurred \$22,000 of costs related to the issuance of the Series B-2 Preferred Stock.

As of September 30, 2016 and December 31, 2015, the total authorized, outstanding and issued Series B-1 Preferred Stock of the Company was 31,564,630 shares. As of September 30, 2016, the total authorized, outstanding and issued Series B-2 Preferred Stock of the Company was 29,362,452 shares. As of December 31, 2015, the total authorized Series B-2 Preferred Stock of the Company was 29,362,452 shares, and no shares were outstanding and issued. 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 B-2 Preferred Stock Tranche Rights

The Company determined the right of the investors to purchase shares of Series B-2 Preferred Stock in a Subsequent Closing or in a Mandatory Closing met the definition of a freestanding financial instrument and was recognized as a liability at fair value upon the initial issuance of the Series B-1 Preferred Stock in July 2015.

As of June 14, 2016, December 31, 2015 and July 10, 2015, the Series B-2 Preferred Stock Tranche Rights liability had a fair value of \$3.6 million, \$2.6 million and \$2.7 million, respectively. The fair value of the Series B-2 Preferred Stock Tranche Rights liability was determined using the probability-weighted present value of the benefit of the investment with the following assumptions:

	<u>As of</u> <u>June 14, 2016</u>	<u>As of</u> <u>December 31, 2015</u>	<u>As of</u> <u>July 10, 2015</u>
Probability of Series B-2 Preferred Stock closing	100%	80%	85%
Expected years until Series B-2 Preferred Stock closing	—	0.5	0.7
Discount rate	0%	19%	18%
Risk-free interest rate	0.0%	2.7%	2.9%
Expected dividend yield	0%	0%	0%

The Company adjusted the carrying value of the Series B-2 Preferred Stock Tranche Rights liability to its estimated fair value at each reporting date subsequent to issuance and immediately prior to the issuance of the Series B-2 Preferred Stock on June 14, 2016 through charges to other income (expense) in the condensed consolidated statement of operations. During the three and nine months ended September 30, 2016, the Company recognized total other expense of \$0 and \$1.0 million, respectively, related to changes in the fair value of the Series B-2 Preferred Stock Tranche Rights.

The change in the carrying value of the Series A Preferred Stock Tranche Rights liability and the Series B-2 Preferred Stock Tranche Rights liability (collectively, the "Preferred Stock Tranche Rights") on the condensed consolidated balance sheets during the nine months ended September 30, 2016 and the year ended December 31, 2015 was as follows:

[Table of Contents](#)

	<u>Nine Months Ended September 30, 2016</u>	<u>Year Ended December 31, 2015</u>
Beginning Balance	2,620	—
Issuance of Series B-2 Preferred Stock Tranche Rights	—	2,694
Change in Fair Value	960	(74)
Issuance of Series B-2 Preferred Stock	(3,580)	—
Ending Balance	<u>—</u>	<u>2,620</u>

The Series B-2 Preferred Stock Tranche Rights liability was extinguished in June 2016 upon the issuance of the Series B-2 Preferred Stock. Immediately prior to the issuance of the Series B-2 Preferred Stock in June 2016, the Company adjusted the carrying value of the liability to its estimated fair value of \$3.6 million.

The rights, preferences, and privileges of the Series A, Series B-1 and Series B-2 (collectively the “Preferred Stock”) are included in the Prospectus. There were no changes to the rights, preferences, and privileges of the Preferred Stock during the nine months ended September 30, 2016.

9. Common Stock

The Company was authorized to issue up to 125,000,000 shares of common stock with a \$0.001 par value per share as of September 30, 2016 and December 31, 2015. As of September 30, 2016 and December 31, 2015, the Company had 594,044 and 536,690 shares, respectively, of common stock issued and outstanding.

The rights, preferences, and privileges of the Company’s common stock are included in the Prospectus. There were no changes to the rights, preferences, and privileges of the common stock during the nine months ended September 30, 2016.

As of September 30, 2016 and December 31, 2015, the Company had warrants to purchase 222,775 shares of common stock issued and outstanding. In October 2016, upon the closing of the IPO, all of the outstanding warrants to purchase 222,775 shares of common stock net exercised, in accordance with their terms, into 221,573 shares of common stock.

Reserve for future issuance

The Company has reserved for future issuances the following number of shares of common stock:

	<u>As of September 30, 2016</u>	<u>As of December 31, 2015</u>
Conversion of Series A Preferred Stock	4,920,074	4,920,074
Conversion of Series B-1 Preferred Stock	4,509,228	4,509,228
Conversion of Series B-2 Preferred Stock	4,194,631	—
Stock-based compensation awards	2,196,603	1,513,727
Warrants to purchase Common Stock	222,775	222,775
Total	<u>16,043,311</u>	<u>11,165,804</u>

10. Revenue Recognition

Merck Collaboration and License Agreement

In April 2013, the Company entered into a multi-target collaboration and license agreement with Merck to use its proprietary drug discovery technology platform to identify orally available cyclic peptides for non-complement program targets nominated by Merck and provide specific research and development services. Under the 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. In April 2015, the Merck Agreement was amended to extend the research term of the collaboration to April 2016.

At the signing of the Merck Agreement, Merck made an upfront non-refundable, technology license fee of \$4.5 million to the Company. In addition, during the research term, which ended in April 2016, the Merck Agreement provided for reimbursement of research and development services provided by the Company in accordance with pre-specified limits for the number of the Company's 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 target, for which the Company had received \$3.5 million in preclinical milestone payments as of September 30, 2016.

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 & 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 are being accounted for as a single combined unit of accounting at the outset of the arrangement.

The Company has recognized revenue in connection with the upfront non-refundable license fee ratably over the research term of the Merck Agreement. Payments for research and development services and reimbursement for certain lab supplies and reagents have been recognized as services are performed, and milestone payments are recognized as these milestones are achieved, assuming all other revenue recognition criteria are met.

The Company has determined that the \$3.5 million in milestone payments received through September 30, 2016 were substantive in nature as they were commensurate with the enhancement of value under the Merck Agreement and because the Company concluded at the start of the collaboration that there was no certainty that these milestones would be successful. 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 as no further performance obligations exist for the Company under the Merck Agreement.

[Table of Contents](#)

The Company recognized revenue of \$0 and \$0.2 million during the three months ended September 30, 2016 and 2015, respectively, and \$4.2 million and \$0.8 million during the nine months ended September 30, 2016 and 2015, respectively, related to upfront, non-refundable payments. The Company recognized revenue of \$0 and \$0.8 million during the three months ended September 30, 2016 and 2015, respectively, and \$0.6 million and \$2.3 million during the nine months ended September 30, 2016 and 2015, respectively, related to research and development services performed under the agreement. In addition, the Company also recognized approximately \$0 and \$0.1 million during the three months ended September 30, 2016 and 2015, respectively, and \$0.1 million and \$0.2 million during the nine months ended September 30, 2016 and 2015, respectively, in revenue related to reimbursable expenses under the terms of the Merck Agreement.

As of September 30, 2016 and December 31, 2015, the Company had \$0 and \$1.9 million, respectively, of deferred revenue, and, \$0 and \$6,000, respectively, of billed and unbilled accounts receivable pursuant to the agreement with Merck.

11. Stock Incentive Plan

The Company maintains the 2010 Stock Incentive Plan (the "2010 Plan") for employees, consultants, advisors, and directors. The 2010 Plan provides for the grant of incentive, non-qualified stock options and restricted stock grants as determined by the Board of Directors. The Company has reserved 2,495,607 shares of common stock under the 2010 Plan. As of September 30, 2016 and December 31, 2015, the Company has 101,109, and 678,781, respectively, of shares available for future issuance under the 2010 Plan. Shares of common stock issued upon exercise of stock options are generally issued from new shares of the Company. The 2010 Plan provides that the exercise price of incentive stock options cannot be less than 100% of the fair market value of the common stock on the date of the award for participants who own less than 10% of the total combined voting power of stock of the Company, and not less than 110% for participants who own more than 10% of the Company's voting power. Options and restricted stock granted under the 2010 Plan vest over periods as determined by the Board of Directors, which generally are equal to four years. Options generally expire ten years from the date of grant.

Total stock-based compensation expense recorded in research and development and general and administrative expenses, respectively, for employees, directors and non-employees during the three and nine months ended September 30, 2016 and 2015 is as follows (in thousands):

	Three Months Ended September 30,		Nine Months Ended September 30,	
	2016	2015	2016	2015
Research and development	\$ 149	\$ 18	\$ 309	\$ 67
General and administrative	102	12	212	54
Total	<u>\$ 251</u>	<u>\$ 30</u>	<u>\$ 521</u>	<u>\$ 121</u>

As of September 30, 2016 and December 31, 2015, the total unrecognized compensation expense related to non-vested, employee and non-employee director stock options, net of related forfeiture estimates, was \$4.8 million and \$1.2 million, respectively. As of September 30, 2016 and December 31, 2015, the total unrecognized compensation cost related to non-vested, non-employee stock options, net of related forfeiture estimates, was \$0.1 million and \$0, respectively. The Company expects to recognize its remaining unrecognized stock-based compensation expense over a weighted-average period of approximately three years. The weighted-average grant date fair value per share for awards granted was \$0.58 during the three months ended September 30, 2016, and was \$0.48 and \$0.26 during the nine months ended September 30, 2016 and 2015, respectively. The Company did not grant any awards to employee and non-employee directors during the three months ended September 30, 2015.

[Table of Contents](#)

The fair value of each stock option granted to employees and directors was estimated on the date of grant using the Black-Scholes option-pricing model, with the following range of assumptions for the three and nine months ended September 30, 2016 and 2015 is as follows (in thousands):

	Three Months Ended September 30,		Nine Months Ended September 30,	
	2016	2015	2016	2015
Risk free interest rate	1.29%	N/A	1.23 - 1.59%	1.76%
Expected dividend yield	—	N/A	—	—
Expected term (in years)	6.0	N/A	6.0 - 6.1	6.0
Expected volatility	86.99% - 87.17%	N/A	74.12% - 87.17%	73.32%

The following table summarizes the activity of the Company's stock option plan for the nine months ended September 30, 2016, (intrinsic values in thousands):

	Number of Shares	Weighted-Average Exercise Price	Average Remaining Contractual Life (in years)	Aggregate Intrinsic Value
Outstanding at December 31, 2014	290,948	\$ 1.82	8.8	\$ 306
Granted	656,292	\$ 2.87		
Exercised	(51,508)	\$ 1.61		
Cancelled or forfeited	(60,786)	\$ 1.75		
Outstanding at December 31, 2015	834,946	\$ 2.66	9.1	\$ 174
Granted	1,464,972	\$ 4.55		
Exercised	(57,354)	\$ 1.89		
Cancelled or forfeited	(147,070)	\$ 2.87		
Outstanding at September 30, 2016	2,095,494	\$ 3.99	9.4	\$ 3,429
Exercisable at September 30, 2016	232,773	\$ 2.59	8.3	\$ 704
Vested or expected to vest at September 30, 2016	1,937,054	\$ 3.92	9.4	\$ 3,221

The total intrinsic value of options exercised was \$47,000 and \$36,000 during the three months ended September 30, 2016 and 2015, respectively, and \$0.1 million and \$36,000 during the nine months ended September 30, 2016 and 2015, respectively.

Stock Option Grants to Non-Employees

During the nine months ended September 30, 2016 and 2015, the Company granted 28,571 and 14,285, respectively, of non-qualified stock options to non-employees, excluding grants to non-employee directors. The Company did not grant any non-qualified stock options to non-employees during the three months ended September 30, 2016 and 2015. The unvested shares held by non-employees are revalued using the Company's estimate of fair value at each reporting period through the remaining vesting period. Stock-based compensation expense related to stock option grants to non-employees was \$22,000 and \$7,000 during three months ended September 30, 2016 and 2015, respectively, and was \$0.1 million and \$0.1 million during the nine months ended September 30, 2016 and 2015, respectively. The fair value of each non-qualified stock option granted to non-employees was estimated on the date of grant and at each revaluation date using the Black-Scholes option-pricing model, with the following range of assumptions, excluding performance-based awards, during the nine months ended September 30, 2016 and 2015 is as follows (in thousands):

[Table of Contents](#)

	Nine Months Ended September 30,	
	2016	2015
Risk free interest rate	1.46% - 1.76%	1.93% - 2.31%
Expected dividend yield	—	—
Expected term (in years)	9.4 - 10.0	9.2 - 10.0
Expected volatility	78.43% - 89.47%	75.34% - 79.89%

Performance-based Stock Options

In December 2015, the Company granted 5,714 non-qualified stock options to a non-employee consultant of the Company, which contain performance-based vesting criteria. Milestone events are specific to the Company's development and progression in clinical trial studies. Stock-based compensation expense associated with this performance-based stock options is recognized if the achievement of the performance condition is considered probable using management's best estimates. As of December 31, 2015, management determined that it was not probable these conditions would be met, and therefore no stock-based compensation expense relating to performance-based awards was recorded. As of September 30, 2016, management determined these conditions were met, and therefore stock-based compensation expense relating to this performance-based award was recorded during the nine months ended September 30, 2016.

In February 2016, the Company granted 29,285 shares of qualified stock options to an employee of the Company. Vesting of the stock option award only commences upon the occurrence of a Trigger Event, which is defined in the employee's equity agreement as the consummation of either (i) an initial public offering of the Company's common stock and (ii) a private placement financing, in one or a series of related closings, resulting in gross proceeds to the Company of at least \$20 million (the "Trigger Event"). Stock-based compensation expense associated with this performance-based stock options is recognized if the achievement of the performance condition is considered probable using management's best estimates. As of September 30, 2016, management determined that it was not probable the Trigger Event would be met, and therefore stock-based compensation expense relating to this performance-based award was not recorded during the nine months ended September 30, 2016. In October 2016, upon the closing of the initial public offering, the vesting of this stock option award commenced.

12. Income Taxes

Deferred tax assets and deferred tax liabilities are recognized based on temporary differences between the financial reporting and tax basis of assets and liabilities using statutory rates. A valuation allowance is recorded against deferred tax assets if it is more likely than not that some or all of the deferred tax asset will not be realized. There were no significant income tax provisions or benefits for the three and nine months ended September 30, 2016 and 2015. Due to uncertainty surrounding the realization of its favorable tax attributes in future tax returns, the Company has recorded a full valuation allowance against the Company's otherwise recognizable net deferred tax assets.

13. Net loss Per Share Attributable to Common Stockholders

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, 2016 and 2015 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.

Basic and diluted net loss per common share are calculated as follows:

[Table of Contents](#)

	Three Months Ended September 30,		Nine Months Ended September 30,	
	2016	2015	2016	2015
Numerator:				
Net loss	\$ (8,114)	\$ (3,932)	\$ (17,976)	\$ (9,380)
Gain on extinguishment of redeemable convertible preferred shares	—	1,673	—	1,673
Net loss attributable to common stockholders — basic and diluted	<u>\$ (8,114)</u>	<u>\$ (2,259)</u>	<u>\$ (17,976)</u>	<u>\$ (7,707)</u>
Denominator:				
Weighted-average common shares used in net loss per share attributable to common stockholders - basic and diluted	570,558	494,302	549,138	486,479
Net loss per share attributable to common stockholders - basic and diluted	<u>\$ (14.22)</u>	<u>\$ (4.57)</u>	<u>\$ (32.73)</u>	<u>\$ (15.84)</u>

The following Common Stock equivalents, presented on an as converted basis, were excluded from the calculation of net loss per share for the periods presented, due to their anti-dilutive effect:

	Three Months Ended September 30,		Nine Months Ended September 30,	
	2016	2015	2016	2015
Convertible preferred stock	13,623,933	9,429,302	13,623,933	9,429,302
Common stock warrants	222,775	222,775	222,775	222,775
Outstanding stock options	2,095,494	231,795	2,095,494	231,795
Total	<u>15,942,202</u>	<u>9,883,872</u>	<u>15,942,202</u>	<u>9,883,872</u>

14. Subsequent Events:

On October 14, 2016, the Company's stockholders approved the 2016 Stock Award and Incentive Plan (the "2016 Plan"), which became effective on October 25, 2016. A total of 1,300,000 shares of common stock were reserved for issuance under the 2016 Plan, plus 101,109 shares originally available for future issuance under the 2010 Plan. In addition, the number of shares of common stock that may be issued under the 2016 Plan will automatically increase on each January 1, beginning on January 1, 2017 and ending on January 1, 2026, equal to the lesser of (i) 2,000,000 shares of common stock, (ii) 4% of the Company's shares of common stock outstanding on the immediately preceding December 31 or (iii) an amount determined by the administrator of the 2016 Plan appointed by the Company's board of directors.

On October 14, 2016, the Company's stockholders approved the 2016 Employee Stock Purchase Plan (the "ESPP"), which became effective on October 25, 2016. A total of 175,000 shares of common stock were reserved for issuance under the ESPP. In addition, the number of shares of common stock that may be issued under the ESPP will automatically increase on each January 1, beginning on January 1, 2017 and ending on January 1, 2026, equal to the lesser of (i) 300,000 shares of common stock, (ii) 1% of the Company's shares of common stock outstanding on the immediately preceding December 31 or (iii) an amount determined by the administrator of the ESPP appointed by the Company's board of directors.

On October 14, 2016, the Company amended its certificate of incorporation to effect a one-for-seven reverse stock split of its issued and outstanding shares of common stock and a proportional adjustment to the existing conversion ratios for each series of the Company's convertible preferred stock. Accordingly, all share and per share amounts for all periods presented in the accompanying interim condensed consolidated financial statements and notes thereto have been adjusted retroactively, where applicable, to reflect this reverse stock split and adjustment of the preferred stock conversion ratios.

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 \$82.9 million after deducting \$6.4 million of underwriting discounts and commissions and offering costs of \$2.3 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 discounts and commissions.

[Table of Contents](#)

The shares began trading on the NASDAQ Global Market on October 26, 2016. Upon the closing of the IPO, all of the outstanding shares of convertible preferred stock automatically converted into 13,623,933 shares of common stock at the applicable conversion ratio then in effect and the outstanding warrants to purchase 222,775 shares of common stock 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 and analysis of our financial condition and results of operations should be read together with our unaudited condensed consolidated financial statements and related notes appearing elsewhere in this Quarterly Report on Form 10-Q and the audited financial information and the notes thereto included in our final prospectus for our initial public offering filed pursuant to Rule 424(b) under the Securities Act of 1933, as amended, or the Securities Act, with the Securities and Exchange Commission, or the SEC on October 26, 2016, or the Prospectus.

Our actual results and timing of certain events may differ materially from the results discussed, projected, anticipated, or indicated in any forward-looking statements. We caution you that forward-looking statements are not guarantees of future performance and that our actual results of operations, financial condition and liquidity, and the development of the industry in which we operate may differ materially from the forward-looking statements contained in this Quarterly Report. In addition, even if our results of operations, financial condition and liquidity, and the development of the industry in which we operate are consistent with the forward-looking statements contained in this Quarterly Report, they may not be predictive of results or developments in future periods.

The following information and any forward-looking statements should be considered in light of factors discussed elsewhere in the Quarterly Report on Form 10-Q, including those risks identified under Part II, Item 1A. Risk Factors.

We caution readers not to place undue reliance on any forward-looking statements made by us, which speak only as of the date they are made. We disclaim any obligation, except as specifically required by law and the rules of the SEC, to publicly update or revise any such statements to reflect any change in our expectations or in events, conditions or circumstances on which any such statements may be based, or that may affect the likelihood that actual results will differ from those set forth in the forward-looking statements.

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.

[Table of Contents](#)

We are developing our lead product candidate, RA101495, a convenient self-administered subcutaneous, or 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 expect to initiate our Phase 2 clinical program for RA101495 in PNH patients in the first quarter of 2017 and release data in the second half of 2017. We are also developing RA101495, administered SC, to treat other debilitating complement-mediated diseases such as refractory generalized myasthenia gravis, or rMG, and lupus nephritis, or LN. We expect to initiate a Phase 2 clinical trial with RA101495 for rMG and a Phase 1b clinical trial in LN in the second half of 2017. We also have preclinical programs targeting selective inhibition of other complement factors for diseases with no approved therapies, including a Factor D program for ophthalmologic and renal diseases, an oral, small molecule C5 inhibitor and a C1s program for certain autoimmune and central nervous system, or CNS, 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., or 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 private placement of our securities and revenue from our collaboration with Merck. As of September 30, 2016, we had raised an aggregate of \$103.5 million comprised of \$86.0 million gross proceeds from private placements of our securities and \$17.5 million in payments in connection with our collaboration and license agreement with Merck, or the Merck Agreement.

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 \$82.9 million after deducting \$6.4 million of underwriting discounts and commissions and offering costs of \$2.3 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 discounts and commissions.

As of September 30, 2016, we had an accumulated deficit of \$57.9 million. Our net losses were \$8.1 million and \$3.9 million for the three months ended September 30, 2016 and 2015, respectively, and \$18.0 million and \$9.4 million for the nine months ended September 30, 2016 and 2015, 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;
- 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.

[Table of Contents](#)

Because of the numerous risks and uncertainties associated with product development, we are unable to predict the timing or amount of increased expenses or when or if we will be able to achieve or maintain profitability. Even if we are able to generate product sales, we may not become profitable. If we fail to become profitable or are unable to sustain profitability on a continuing basis, then we may be unable to continue our operations at planned levels and be forced to reduce or terminate our operations.

Financial Overview

Revenue

We have derived all of our revenue to date from our collaboration and license agreement with Merck, or 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, or 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, 2016. 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 the section entitled “—Revenue Recognition—Merck Collaboration and License Agreement” within the Prospectus. For additional information about our revenue recognition policy, see the section entitled “—Critical Accounting Policies and Significant Judgments and Estimates—Revenue” within the Prospectus.

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

Research and Development Expenses

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

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

[Table of Contents](#)

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.

We have not provided program costs since inception because historically we have not tracked or recorded our research and development expenses on a program-by-program basis. We use our employee and infrastructure resources across multiple research and development programs directed toward developing our Extreme Diversity platform and for identifying and developing product candidates. We manage certain activities such as contract research and manufacturing of RA101495 and our discovery programs through our third-party vendors, and do not track the costs of these activities on a program-by-program basis.

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

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

A change in the outcome of any of these factors could mean a significant change in the costs and timing associated with the development of our current and future preclinical and clinical product candidates. For example, if the FDA, or another regulatory authority were to require us to conduct clinical trials beyond those that we currently anticipate will be required for the completion of clinical development, or if we experience significant delays in enrollment in any of our preclinical 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.

[Table of Contents](#)

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, or 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 expense incurred on debt instruments, amortized debt discount, interest income earned on our cash and cash equivalents, non-cash changes in fair value of the derivative liability associated with the Convertible Notes, loss on debt extinguishment and the increase in fair value of preferred stock tranche rights, or Preferred Stock Tranche Rights. The debt discount primarily resulted from recording the relative fair value of warrants and the fair value of bifurcated features embedded in the Convertible Notes issued in April 2015. The debt discount has been amortized to interest expense over the life of the Convertible Note.

Critical Accounting Policies and Significant Judgments and Estimates

Our management's discussion and analysis of our financial condition and results of operations is based on our financial statements, which we have prepared in accordance with U.S. generally accepted accounting principles. We believe that several accounting policies are important to understanding our historical and future performance. We refer to these policies as critical because these specific areas generally require us to make judgments and estimates about matters that are uncertain at the time we make the estimate, and different estimates—which also would have been reasonable—could have been used. On an ongoing basis, we evaluate our estimates and judgments, including those described in greater detail below. We base our estimates on historical experience and other market-specific or other relevant assumptions that we believe to be reasonable under the circumstances, the results of which form the basis for making judgments about the carrying value of assets and liabilities that are not readily apparent from other sources. Actual results may differ from these estimates under different assumptions or conditions.

We believe that our most critical accounting policies are those relating to revenue recognition, accrued research and development expenses and stock-based compensation. There have been no significant changes to our accounting policies discussed in our Prospectus.

Result of Operations

Comparison of the Three Months Ended September 30, 2016 and 2015

The following table summarizes our results of operations for the three months ended September 30, 2016 and 2015, together with the dollar change in those items:

[Table of Contents](#)

	Three Months Ended September 30,		Period-to-Period Change
	2016	2015	
Revenue	\$ —	\$ 1,096	\$ (1,096)
Operating expenses:		(in thousands)	
Research & development	7,079	4,027	3,052
General & administrative	1,042	539	503
Total operating expenses	8,121	4,566	3,555
Loss from operations	(8,121)	(3,470)	(4,651)
Other income (expense), net	7	(462)	469
Net loss	\$ (8,114)	\$ (3,932)	\$ (4,182)

Revenue

Revenue decreased by \$1.1 million to \$0 for the three months ended September 30, 2016, from \$1.1 million for the three months ended September 30, 2015. This decrease is primarily attributable to the expiration of the research term of the Merck Agreement in April, 2016.

Research and Development Expenses

Research and development expenses increased by \$3.1 million to \$7.1 million for the three months ended September 30, 2016, from \$4.0 million for the three months ended September 30, 2015. This increase is primarily attributable to \$1.4 million increase in CRO and CMO expenses in connection with our pre-clinical studies and clinical trials for RA101495, \$0.9 million increase in employee-related costs associated with salaries, bonus, benefits and non-cash stock-based compensation, including costs for additional personnel to support our increased research and development activities, \$0.4 million increase in facilities costs including rent, moving expenses related to our new facility, and depreciation expense, \$0.3 million increase in consulting expenses and \$0.1 million increase in lab-supply and reagent expenses.

General and Administrative Expenses

General and administrative expenses increased by \$0.5 million to \$1.0 million for the three months ended September 30, 2016, from \$0.5 million for the three months ended September 30, 2015. The increase in general and administrative expenses was primarily attributable to \$0.3 million increase in employee-related costs, including salary, bonus, benefits and non-cash stock-based compensation for executive and administrative personnel including for additional personnel to support our increased activities, \$0.1 million increase in consulting and professional fees, including increased market research, accounting and audit fees, and approximately \$0.1 million in increased facilities related costs.

Other Income (Expense), Net

Other income (expense), net increased by \$0.5 million to \$7,000 in other income, net during the three months ended September 30, 2016 from \$0.5 million in other expense, net for the three months ended September 30, 2015. This increase was primarily attributable to \$0.6 million in other expense related to the loss upon debt extinguishment of the Convertible Notes recognized in the three months ended September 30, 2015, partially offset by the increase in the fair value of the Series B-2 Preferred Stock Tranche Rights of approximately \$0.1 million, recognized in the three months ended September 30, 2015.

[Table of Contents](#)**Comparison of the Nine Months Ended September 30, 2016 and 2015**

The following table summarizes our results of operations for the nine months ended September 30, 2016 and 2015, together with the dollar change in those items:

	Nine Months Ended September 30,		Period-to-Period Change
	2016	2015	
Revenue	\$ 4,928	\$ 3,273	\$ 1,655
Operating expenses:		(in thousands)	
Research & development	18,541	10,593	7,948
General & administrative	3,418	1,549	1,869
Total operating expenses	21,959	12,142	9,817
Loss from operations	(17,031)	(8,869)	(8,162)
Other expense, net	(945)	(511)	(434)
Net loss	\$ (17,976)	\$ (9,380)	\$ (8,596)

Revenue

Revenue increased by \$1.6 million to \$4.9 million for the nine months ended September 30, 2016, from \$3.3 million for the nine months ended September 30, 2015. This increase is primarily attributable to the recognition of a \$3.0 million milestone payment from Merck in June 2016 and an increase of \$0.4 million in connection with recognition of the remaining deferred revenue related to the upfront non-refundable, license fee earned at the expiration of the research term of the Merck Agreement, on March 31, 2016. These increases were offset in part by \$1.6 million decrease in FTE revenue related to a decrease in the number of FTEs providing research and development services under the Merck Agreement and \$0.1 million decrease related to lower reimbursable lab supply and reagent expenses for the nine months ended September 30, 2016.

Research and Development Expenses

Research and development expenses increased by \$7.9 million to \$18.5 million for the nine months ended September 30, 2016, from \$10.6 million for the nine months ended September 30, 2015. This increase is primarily attributable to \$3.6 million increase in CRO and CMO expenses in connection with our pre-clinical studies and clinical trials for RA101495, \$2.0 million increase in employee-related costs associated with salaries, bonus, benefits, travel and non-cash stock-based compensation including costs for additional personnel to support our increased research and development activities, \$1.3 million increase in facilities costs including rent, moving expenses related to our new facility, and depreciation expense, \$0.6 million increase in consulting expenses and \$0.4 million increase in lab-supply and reagent expenses.

General and Administrative Expenses

General and administrative expenses increased by \$1.9 million to \$3.4 million for the nine months ended September 30, 2016, from \$1.5 million for the nine months ended September 30, 2015. The increase in general and administrative expenses was primarily attributable to \$0.8 million increase in employee-related costs, including salary, bonus, benefits, travel, insurance and non-cash stock-based compensation for executive and administrative personnel including for additional personnel to support our increased activities, \$0.8 million increase in consulting and professional fees, including increased market research, accounting, legal and audit fees and approximately \$0.3 million in increased facilities related costs and depreciation.

Other Expense, Net

Other expense, net increased by \$0.4 million to \$0.9 million in other expense, net during the nine months ended September 30, 2016 from \$0.5 million in other expense, net for the nine months ended September 30, 2015. This increase was primarily attributable to increase in the fair value of the Series B-2 Preferred Stock Tranche Rights of \$1.0 million recognized during the nine months ended September 30, 2016, offset by other expense of \$0.6 million related to the loss upon debt extinguishment of the Convertible Notes during the nine months ended September 30, 2015.

Liquidity and Capital Resources

Overview

We have funded our operations from inception through September 30, 2016 primarily through gross proceeds of \$86.0 million from the sale of our preferred stock and the issuance of convertible notes that subsequently converted into preferred stock and \$17.5 million from payments from Merck in connection with the Merck Agreement. As of September 30, 2016, we had cash and cash equivalents of \$31.2 million.

Cash Flows

The following table provides information regarding our cash flows for the nine months ended September 30, 2016 and 2015:

	Nine Months Ended September 30,	
	2016	2015
	(in thousands)	
Net cash (used in) provided by:		
Operating activities	\$ (12,819)	\$ (8,815)
Investing activities	(4,358)	(1,485)
Financing activities	28,987	28,681
Net increase in cash	<u>\$ 11,810</u>	<u>\$ 18,381</u>

Net Cash Used in Operating Activities

The use of cash in all periods resulted primarily from our net losses adjusted for non-cash charges and changes in components of working capital.

Net cash used in operating activities was \$12.8 million during the nine months ended September 30, 2016 compared to \$8.8 million during the nine months September 30, 2015. The increase in net cash used in operations was primarily due to an increase in our net loss of \$8.6 million for the nine months ended September 30, 2016, as compared to the nine months ended September 30, 2015 due to an increase in our operating expenses in connection with our lead program RA101495 and other research and development pipeline.

Net Cash Used in Investing Activities

Net cash used in investing activities was \$4.4 million for the nine months ended September 30, 2016 compared to \$1.5 million during the nine months September 30, 2015. The increase in cash used in investing

[Table of Contents](#)

activities was primarily due to increased purchases of property and equipment related to the cost of new leasehold improvements in our new leased facility in Cambridge, Massachusetts.

Net Cash Provided by Financing Activities

Net cash provided by financing activities was \$29.0 million during the nine months ended September 30, 2016 compared to \$28.7 million during the nine months September 30, 2015. The increase in cash provided by financing activities was due to net proceeds of \$29.2 million related to the issuance of Series B-2 preferred stock during the nine months ended September 30, 2016, offset by cash provided by the net proceeds of \$28.9 million related to the issuance of convertible notes and Series B-1 preferred stock during the nine months ended September 30, 2015.

Funding Requirements

We expect our expenses to increase in connection with our ongoing activities, particularly as we initiate Phase 2 clinical trials of RA101495 in PNH, initiate clinical trial of RA101495 in additional indications MG 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, with the closing of our initial public offering, we expect to incur additional costs associated with 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 \$82.9 million after deducting \$6.4 million of underwriting discounts and commissions and offering costs of \$2.3 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 discounts and commissions. We believe that the proceeds from the IPO, together with our existing cash and cash equivalents as of September 30, 2016, will enable us to fund our operating expenses and capital expenditure requirements into the third 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

[Table of Contents](#)

- 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, your ownership interest will be diluted, and the terms of these securities may include liquidation or other preferences that adversely affect your rights as 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 Obligations

The disclosure of our contractual obligations and commitments is set forth under the heading “Management’s Discussion and Analysis of Financial Condition and Results of Operations—Contractual Obligations” in our final prospectus filed under the Securities Act with the Securities and Exchange Commission pursuant to Rule 424(b)(4) on October 26, 2016.

Off-Balance Sheet Arrangements

We did not have during the periods presented, and we do not currently have, any off-balance sheet arrangements, as defined under the applicable regulations of the SEC.

Item 3. Quantitative and Qualitative Disclosures About Market Risk

We are exposed to market risk related to changes in interest rates. As of September 30, 2016, we had cash and cash equivalents of \$32.2 million, respectively, consisting primarily of money market funds. Our primary

[Table of Contents](#)

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

Item 4. 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 submit under the 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 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, 2016.

Changes in Internal Control over Financial Reporting

No change in our internal control over financial reporting (as defined in Rules 13a-15(f) and 15d-15(f) under the Exchange Act) occurred during the three months ended September 30, 2016 that has materially affected, or is reasonably likely to materially affect, our internal control over financial reporting.

Part II — Other Information

Item 1. Legal Proceedings.

From time to time, we may be involved in lawsuits, claims, investigations and proceedings, consisting of intellectual property, commercial, employment and other matters which arise in the ordinary course of business. We are not currently a party to any material legal proceedings.

Item 1A. Risk Factors

Careful consideration should be given to the following risk factors, in addition to the other information set forth in this Quarterly Report on Form 10-Q and in other documents that we file with the SEC, in evaluating the Company and our business. Investing in our common stock involves a high degree of risk. If any of the following risks and uncertainties actually occurs, our business, prospects, financial condition and results of operations could be materially and adversely affected. The risks described below are not intended to be exhaustive and are not the only risks facing the Company. New risk factors can emerge from time to time, and it is not possible to predict the impact that any factor or combination of factors may have on our business, prospects, financial condition and results of operations. Certain statements below are forward-looking statements. See “Information Regarding Forward-Looking Statements” in this Quarterly Report on Form 10-Q.

Risks Related to Our Financial Position and Need for Additional Capital

We have a history of significant operating losses and expect to incur significant and increasing losses for the foreseeable future; we may never achieve or maintain profitability and investors may lose their entire investment.

We do not expect to generate revenue or profitability that is necessary to finance our operations in the short term. We incurred net losses of \$5.5 million and \$13.9 million for the years ended December 31, 2014 and 2015, respectively, and \$9.4 million and \$18.0 million for the nine months ended September 30, 2015 and 2016, respectively. In addition, our accumulated deficit as of December 31, 2014 and 2015 was \$26.0 million and \$39.9 million, respectively, and \$57.9 million as of September 30, 2016. To date, we have not commercialized any products or generated any revenues from the sale of products, and absent the realization of sufficient revenues from product sales, we may never attain profitability in the future. We have devoted substantially all of our financial resources and efforts to research and development, including preclinical studies and our clinical trials. Our net losses may fluctuate significantly from quarter to quarter and year to year. Net losses and negative cash flows have had, and will continue to have, an adverse effect on our stockholders' equity and working capital.

We anticipate that our expenses will increase substantially if and as we:

- continue to develop and conduct clinical trials with respect to our lead product candidate, RA101495;
- initiate and continue research, preclinical and clinical development efforts for any future product candidates;
- seek to identify additional product candidates;
- seek regulatory and marketing approvals for our product candidates that successfully complete clinical trials, if any;
- establish sales, marketing, distribution and other commercial infrastructure in the future to commercialize various products for which we may obtain marketing approval, if any;
- require the manufacture of larger quantities of product candidates for clinical development and, potentially, commercialization;
- maintain, expand and protect our intellectual property portfolio;
- hire and retain additional personnel, such as clinical, quality control and scientific personnel;
- add operational, financial and management information systems and personnel, including personnel to support our product development and help us comply with our obligations as a public company; and
- add equipment and physical infrastructure to support our research and development.

Our ability to become and remain profitable depends on our ability to generate revenue. We do not expect to generate significant revenue unless and until we are, or any future collaborator is, able to obtain marketing

[Table of Contents](#)

approval for, and successfully commercialize, one or more of our product candidates. Successful commercialization will require achievement of key milestones, including completing clinical trials of our product candidates, obtaining marketing approval for these product candidates, manufacturing, marketing and selling those products for which we, or any of our future collaborators, may obtain marketing approval, satisfying any post-marketing requirements and obtaining reimbursement for our products from private insurance or government payors. Because of the uncertainties and risks associated with these activities, we are unable to accurately predict the timing and amount of revenues, and if or when we might achieve profitability. We and any future collaborators may never succeed in these activities and, even if we do, or any future collaborators do, we may never generate revenues that are large enough for us to achieve profitability. Even if we do achieve profitability, we may not be able to sustain or increase profitability on a quarterly or annual basis.

Because our only source of revenue to date, our research collaboration with Merck & Co., or Merck, has reached the end of its research term and identified a product candidate, to the extent we continue to receive revenue from this collaboration, we must rely on Merck's efforts to develop and commercialize that target candidate, which we do not control.

Our failure to become and remain profitable would depress the market price of our common stock and could impair our ability to raise capital, expand our business, diversify our product offerings or continue our operations. If we continue to suffer losses as we have in the past, investors may not receive any return on their investment and may lose their entire investment.

Our independent registered public accounting firm had indicated that our financial condition raises substantial doubt as to our ability to continue as a going concern.

Prior to the closing of our initial public offering in October 2016, our independent registered public accounting firm has included in its audit opinion for the year ended December 31, 2015 a statement that there was substantial doubt as to our ability to continue as a going concern as a result of our recurring losses and financial condition as of December 31, 2015. The reaction of investors to the inclusion of a going concern statement by our auditors, may materially adversely affect our share price and our ability to raise new capital or enter into strategic alliances.

We will need substantial additional funding, and if we are unable to raise capital when needed, we could be forced to delay, reduce or eliminate our product discovery and development programs or commercialization efforts.

Developing pharmaceutical products, including conducting preclinical studies and clinical trials, is a very time-consuming, expensive and uncertain process that takes years to complete. For example, in the years ended December 31, 2014 and December 31, 2015, we used \$7.6 million and \$12.0 million, respectively, in net cash for our operating activities, and in the nine months ended September 30, 2016, we used \$12.8 million in net cash for our operating activities, substantially all of which related to research and development activities. We expect our expenses to increase in connection with our ongoing activities, particularly as we initiate new clinical trials of, initiate new research and preclinical development efforts for and seek marketing approval for, our current product candidates or any product candidates that we acquire, if any. In addition, if we obtain marketing approval for any of our product candidates, we may incur significant commercialization expenses related to product sales, marketing, manufacturing and distribution to the extent that such sales, marketing, manufacturing and distribution are not the responsibility of a future collaborator. Furthermore, as a public company, we will incur significant additional costs associated with 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 may be forced to delay, reduce or eliminate our research and development programs or any future commercialization efforts.

We will be required to expend significant funds in order to advance the development of RA101495, as well as other product candidates we may seek to develop. In addition, while we may seek one or more collaborators for future development of our product candidates for one or more indications, we may not be able to enter into a collaboration for any of our product candidates for such indications on suitable terms, on a timely basis or at all. In any event, our existing cash and cash equivalents will not be sufficient to fund all of the efforts that we plan to

[Table of Contents](#)

undertake or to fund the completion of development of any of our product candidates. Accordingly, we will be required to obtain further funding through public or private equity offerings, debt financings, collaborations and licensing arrangements or other sources. We do not have any committed external source of funds. 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.

Our future funding requirements, both short-term and long-term, will depend on many factors, including:

- the scope, progress, timing, costs and results of clinical trials of, and research and preclinical development efforts for, our current and future product candidates;
- our ability to enter into, and the terms and timing of, any collaborations, licensing 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 including enforcing and defending intellectual property related claims; and
- the costs of operating as a public company.

Raising additional capital may cause dilution to our stockholders, restrict our operations or require us to relinquish rights to our technologies or product candidates.

We expect our expenses to increase in connection with our planned operations. To the extent that we raise additional capital through the sale of common stock, convertible securities or other equity securities, the ownership interest of our existing stockholders may be diluted, and the terms of these securities could include liquidation or other preferences and anti-dilution protections that could adversely affect the rights of our common stockholders. In addition, debt financing, if available, may result in fixed payment obligations and may involve agreements that include restrictive covenants that limit our ability to take specific actions, such as incurring additional debt, making capital expenditures, creating liens, redeeming stock or declaring dividends, that could adversely impact our ability to conduct our business. In addition, securing financing could require a substantial amount of time and attention from our management and may divert a disproportionate amount of their attention away from day-to-day activities, which may adversely affect our management's ability to oversee the development of our product candidates.

If we raise additional funds through collaborations or marketing, distribution or licensing arrangements with third parties, we may have to relinquish valuable rights to our technologies, future revenue streams or product candidates or grant licenses on terms that may not be favorable to us. If we are unable to raise additional funds 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.

We have a limited operating history and no history of commercializing pharmaceutical products, which may make it difficult to evaluate the prospects for our future viability.

We commenced operations in 2008. Our operations to date have been limited to financing and staffing our company, developing our technology and conducting preclinical research and early-stage clinical trials for our product candidates. We have not yet demonstrated an ability to successfully conduct late-stage clinical trials, obtain marketing approvals, manufacture a commercial-scale product, or arrange for a third party to do so on our behalf, or conduct sales and marketing activities necessary for successful product commercialization. Accordingly, you should consider our prospects in light of the costs, uncertainties, delays and difficulties frequently encountered by companies in the early stages of development, especially clinical-stage biopharmaceutical companies such as ours.

[Table of Contents](#)

Any predictions you make about our future success or viability may not be as accurate as they could be if we had a longer operating history or a history of successfully developing and commercializing pharmaceutical products.

We may encounter unforeseen expenses, difficulties, complications, delays and other known or unknown factors in achieving our business objectives. We will eventually need to transition from a company with a development focus to a company capable of supporting commercial activities. We may not be successful in such a transition.

We expect our financial condition and operating results to continue to fluctuate significantly from quarter to quarter and year to year due to a variety of factors, many of which are beyond our control. Accordingly, you should not rely upon the results of any quarterly or annual periods as indications of future operating performance.

Risks Related to the Discovery, Development and Commercialization of Our Product Candidates

We are at a very early stage in our development efforts, our approach is unproven and we may not be able to successfully develop and commercialize any product candidates.

RA101495 is a novel therapeutic compound and its potential therapeutic benefit is unproven. There is only one approved therapy inhibiting C5. Our product candidates may not demonstrate in patients any or all of the pharmacological benefits we believe they may possess or compare favorably to the approved C5 inhibitor therapy. We have not yet succeeded and may never succeed in demonstrating efficacy and safety for these or any other product candidates in clinical trials or in obtaining marketing approval thereafter. For example, although we have evaluated RA101495 in preclinical studies and have evaluated RA101495 in an early-stage clinical trial, we have not yet advanced RA101495 into Phase 2 or Phase 3 clinical development, nor have we obtained regulatory approval to sell any product based on our therapeutic approaches.

Our development plans include exploring the potential of complement inhibition, including C5 inhibition, to treat complement-mediated diseases for which complement inhibition has not been fully validated. This is an unproven approach to the treatment of diseases such as refractory generalized myasthenia gravis, or rMG, and lupus nephritis, or LN. The scientific evidence to support the feasibility of developing products to treat such disease by C5 inhibition is both preliminary and limited. Accordingly, our focus on treating these diseases may not result in the discovery and development of commercially viable products.

If we are unsuccessful in our development efforts, we may not be able to advance the development of our product candidates, commercialize products, raise capital, expand our business or continue our operations.

Our business depends heavily upon the success of RA101495, which is still under development. If we are unable to obtain regulatory approval for or successfully commercialize RA101495, our business will be materially harmed.

We currently have no products approved for sale and are investing a significant portion of our efforts and financial resources in the development of our lead product candidate, RA101495. Successful continued development and ultimate regulatory approval of RA101495 for paroxysmal nocturnal hemoglobinuria, or PNH, and, in the future, a range of debilitating autoimmune diseases including rMG and LN is critical to the future success of our business. We will need to raise sufficient funds for, and successfully enroll and complete, our clinical development program for RA101495 in PNH. The future regulatory and commercial success of this product candidate is subject to a number of risks, including the following:

- we may not have sufficient financial and other resources to initiate or complete the necessary clinical trials for RA101495;
- we may not be able to obtain adequate evidence of clinical efficacy and safety for RA101495 in PNH;
- we do not know the degree to which RA101495 will be accepted as a therapy, if approved;
- in our clinical programs, we may experience variability in patients, adjustments to clinical trial procedures and the need for additional clinical trial sites, which could delay our clinical trial progress;
- the results of our clinical trials may not meet the level of statistical or clinical significance required by the

[Table of Contents](#)

FDA, the European Medicines Agency, or EMA, or comparable foreign regulatory bodies for marketing approval;

- we may have difficulty enrolling patients in trials if, for instance, a current or future effective standard of care limits the desire of patients, physicians, or regulatory agencies to participate in or support clinical trials;
- patients in our clinical trials may die or suffer other adverse effects for reasons that may or may not be related to RA101495, which could delay or prevent further clinical development;
- the standards implemented by regulatory agencies may change at any time;
- the FDA, EMA or foreign regulatory agencies may require efficacy endpoints for a Phase 2 clinical trial for the treatment of PNH, rMG and LN that differ from the endpoints of our planned current or future trials, which may require us to conduct additional clinical trials;
- the mechanism of action of RA101495 is complex and we cannot guarantee the degree to which it will translate into a medical benefit in any indications;
- if approved for PNH, rMG and LN, RA101495 will likely compete with the off-label use of currently marketed products and other therapies in development;
- our intellectual property rights may not be patentable, valid or enforceable; and
- we may not be able to obtain, maintain, defend or enforce our patents and other intellectual property rights.

Of the large number of drugs in development in the pharmaceutical industry, only a small percentage result in the submission of a new drug application, or NDA, to the FDA and even fewer are approved for commercialization. Furthermore, even if we do receive regulatory approval to market RA101495, any such approval may be subject to limitations on the indicated uses or patient populations for which we may market the product. Accordingly, even if we are able to obtain the requisite financing to continue to fund our development programs, we cannot assure you that RA101495 will be successfully developed or commercialized. If we or any of our future development partners are unable to develop, or obtain regulatory approval for, or, if approved, successfully commercialize RA101495, we may not be able to generate sufficient revenue to continue our business.

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 in early clinical trials, RO7112689, a monoclonal antibody inhibitor of C5 being developed by F. Hoffman-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 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. 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 Roche that is in Phase 3 clinical trials, LFG316, an anti-C5 monoclonal antibody being developed by Novartis Pharma that is in Phase 2 clinical trials, Zimura, a C5 inhibitor being developed by Ophthotech that is entering Phase 2/3 clinical

[Table of Contents](#)

trials, and other product candidates that 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.

If clinical trials of our product candidates fail to satisfactorily demonstrate safety and efficacy to the FDA and other regulators, we, or any future collaborators, may incur additional costs or experience delays in completing, or ultimately be unable to complete, the development and commercialization of these product candidates.

We, and any future collaborators, are not permitted to commercialize, market, promote or sell any product candidate in the United States without obtaining marketing approval from the FDA. Foreign regulatory authorities, such as the EMA, impose similar requirements. We have not previously submitted a NDA to the FDA or similar drug approval filings to comparable foreign regulatory authorities for any of our product candidates. We, and any future collaborators, must complete extensive preclinical development and clinical trials to demonstrate the safety and efficacy of our product candidates in humans before we will be able to obtain these approvals.

Clinical testing is expensive, is difficult to design and implement, can take many years to complete and is inherently uncertain as to outcome. We cannot guarantee that any clinical trials will be conducted as planned or completed on schedule, if at all. The clinical development of our product candidates is susceptible to the risk of failure inherent at any stage of product development, including failure to demonstrate efficacy in a clinical trial or across a broad population of patients, the occurrence of adverse events that are severe or medically or commercially unacceptable, failure to comply with protocols or applicable regulatory requirements and determination by the FDA or any comparable foreign regulatory authority that a product candidate may not continue development or is not approvable. It is possible that even if one or more of our product candidates has a beneficial effect, that effect will not be detected during clinical evaluation as a result of one or more of a variety of factors, including the size, duration, design, measurements, conduct or analysis of our clinical trials. Conversely, as a result of the same factors, our clinical trials may indicate an apparent positive effect of a product candidate that is greater than the actual positive effect, if any. Similarly, in our clinical trials we may fail to detect toxicity or intolerance caused by our product candidates, or mistakenly believe that our product candidates are toxic or not well tolerated when that is not in fact the case.

Any inability to successfully complete preclinical and clinical development could result in additional costs to us, or any future collaborators, and impair our ability to generate revenues from product sales, regulatory and commercialization milestones and royalties. Moreover, if we, or any future collaborators, are required to conduct additional clinical trials or other testing of our product candidates beyond the trials and testing that we or they contemplate, if we or they are unable to successfully complete clinical trials of our product candidates or other

[Table of Contents](#)

testing or the results of these trials or tests are unfavorable, uncertain or are only modestly favorable, or there are unacceptable safety concerns associated with our product candidates, we, or any future collaborators may:

- incur additional unplanned costs;
- be delayed in obtaining marketing approval for our product candidates;
- not obtain marketing approval at all;
- obtain approval for indications or patient populations that are not as broad as intended or desired;
- obtain approval with labeling that includes significant use or distribution restrictions or significant safety warnings, including boxed warnings;
- be subject to additional post-marketing testing or other requirements; or
- be required to remove the product from the market after obtaining marketing approval.

Our failure to successfully complete clinical trials of our product candidates and to demonstrate the efficacy and safety necessary to obtain regulatory approval to market any of our product candidates would significantly harm our business.

Our product candidates may cause undesirable side effects or have other properties that could delay or prevent their regulatory approval, limit the commercial profile of an approved label, or result in significant negative consequences following marketing approval, if any.

Undesirable side effects caused by our product candidates could cause us or regulatory authorities to interrupt, delay or halt clinical trials and could result in a more restrictive label or the delay or denial of regulatory approval by the FDA or comparable foreign regulatory authorities. Results of our clinical trials could reveal a high and unacceptable severity and prevalence of side effects or unexpected characteristics. To date, subjects exposed to our product candidate RA101495 in our Phase 1 clinical trial have experienced drug-related side effects including injection site erythema, which was reported in patients receiving the highest dose, fatigue, headache, dizziness, rash and upper respiratory tract infection.

If unacceptable side effects arise in the development of our product candidates, we, the FDA or comparable foreign regulatory authorities, the Institutional Review Boards, or IRBs, or independent ethics committees at the institutions in which our studies are conducted, or the Data Safety Monitoring Board, or DSMB, could suspend or terminate our clinical trials or the FDA or comparable foreign regulatory authorities could order us to cease clinical trials or deny approval of our product candidates for any or all targeted indications. Treatment-related side effects could also affect patient recruitment or the ability of enrolled patients to complete the trial or result in potential product liability claims. In addition, these side effects may not be appropriately recognized or managed by the treating medical staff. We expect to have to train medical personnel using our product candidates to understand the side effect profiles for our clinical trials and upon any commercialization of any of our product candidates. Inadequate training in recognizing or managing the potential side effects of our product candidates could result in patient injury or death. Any of these occurrences may harm our business, financial condition and prospects significantly.

Moreover, clinical trials of our product candidates are conducted in carefully defined sets of patients who have agreed to enter into clinical trials. Consequently, it is possible that our clinical trials, or those of any future collaborator, may indicate an apparent positive effect of a product candidate that is greater than the actual positive effect, if any, or alternatively fail to identify undesirable side effects. If, following approval of a product candidate, we, or others, discover that the product is less effective than previously believed or causes undesirable side effects that were not previously identified, any of the following adverse events could occur:

- regulatory authorities may withdraw their approval of the product or seize the product;
- we, or any future collaborators, may need to recall the product, or be required to change the way the product is administered or conduct additional clinical trials;
- additional restrictions may be imposed on the marketing of, or the manufacturing processes for, the particular product;
- we may be subject to fines, injunctions or the imposition of civil or criminal penalties;
- regulatory authorities may require the addition of labeling statements, such as a “black box” warning or a contraindication;

[Table of Contents](#)

- we, or any future collaborators, may be required to create a Medication Guide outlining the risks of the previously unidentified side effects for distribution to patients;
- we, or any future collaborators, could be sued and held liable for harm caused to patients;
- the product may become less competitive; and
- our reputation may suffer.

Any of these events could harm our business and operations, and could negatively impact our stock price.

If we fail to develop and commercialize other product candidates, we may be unable to grow our business.

Although the development and commercialization of RA101495 is our primary focus, as part of our longer-term growth strategy, we plan to evaluate the development and commercialization of other therapies for complement-mediated diseases, including rare blood, neurologic, ophthalmologic, renal and inflammatory diseases. We will evaluate internal opportunities from our current product candidates, and also may choose to in-license or acquire other product candidates as well as commercial products to treat patients suffering from immune-mediated or orphan or other disorders with high unmet medical needs and limited treatment options. These other product candidates will require additional, time-consuming development efforts prior to commercial sale, including preclinical studies, clinical trials and approval by the FDA and/or applicable foreign regulatory authorities. All product candidates are prone to the risks of failure that are inherent in pharmaceutical product development, including the possibility that the product candidate will not be shown to be sufficiently safe and effective for approval by regulatory authorities. In addition, we cannot assure you that any such products that are approved will be manufactured or produced economically, successfully commercialized or widely accepted in the marketplace or be more effective than other commercially available alternatives.

Our preclinical programs may not produce product candidates that are suitable for clinical trials or that can be successfully commercialized or generate revenue through partnerships.

We must successfully complete preclinical testing for RA101495 and our other programs, which may include demonstrating activity and comprehensive studies to show the lack of toxicity and other adverse effects in established animal models, before commencing clinical trials for any product candidate. Many pharmaceutical and biological products do not successfully complete preclinical testing and, even if preclinical testing is successfully completed, may fail in clinical trials. In addition, there can be no assurance that positive results from preclinical studies will be predictive of results obtained from subsequent preclinical studies or clinical trials. We also cannot be certain that any product candidates that do advance into clinical trials will successfully demonstrate safety and efficacy in clinical trials. Even if we achieve positive results in early clinical trials, they may not be predictive of the results in later trials.

We may expend our limited resources to pursue a particular product candidate or indication and fail to capitalize on product candidates or indications that may be more profitable or for which there is a greater likelihood of success.

Because we have limited financial and managerial resources, we intend to focus on developing product candidates for specific indications that we identify as most likely to succeed, in terms of both their potential for marketing approval and commercialization. As a result, we may forego or delay pursuit of opportunities with other product candidates or for other indications that may prove to have greater commercial potential.

Our resource allocation decisions may cause us to fail to capitalize on viable commercial products or profitable market opportunities. Our spending on current and future research and development programs and product candidates for specific indications may not yield any commercially viable product candidates. If we do not accurately evaluate the commercial potential or target market for a particular product candidate, we may relinquish valuable rights to that product candidate through collaboration, licensing or other royalty arrangements in cases in which it would have been more advantageous for us to retain sole development and commercialization rights to the product candidate.

[Table of Contents](#)

If the FDA or comparable foreign regulatory authorities approve generic versions of any of our product candidates that receive marketing approval, or such authorities do not grant such products appropriate periods of data exclusivity before approving generic versions of such products, the sales of such products could be adversely affected.

Once a NDA is approved, the product covered thereby becomes a “reference-listed drug” in the FDA’s publication, “Approved Drug Products with Therapeutic Equivalence Evaluations,” or the Orange Book. Manufacturers may seek approval of generic versions of reference-listed drugs through submission of abbreviated new drug applications, or ANDAs, in the United States. In support of an ANDA, a generic manufacturer generally must show that its product has the same active ingredient(s), dosage form, strength, route of administration and conditions of use or labeling as the reference-listed drug and that the generic version is bioequivalent to the reference-listed drug, meaning, in part, that it is absorbed in the body at the same rate and to the same extent. Generic products may be significantly less costly to bring to market than the reference-listed drug and companies that produce generic products are generally able to offer them at lower prices. Thus, following the introduction of a generic drug, a significant percentage of the sales of any branded product or reference-listed drug may be typically lost to the generic product.

The FDA may not approve an ANDA for a generic product until any applicable period of non-patent exclusivity for the reference-listed drug has expired. The Federal Food, Drug, and Cosmetic Act, or FDCA, provides a period of five years of non-patent exclusivity for a new drug containing a new chemical entity, or NCE. Specifically, in cases where such exclusivity has been granted, an ANDA may not be filed with the FDA until the expiration of five years unless the submission is accompanied by a Paragraph IV certification that a patent covering the reference-listed drug is either invalid or will not be infringed by the generic product, in which case the applicant may submit its application four years following approval of the reference-listed drug. It is unclear whether the FDA will treat the active ingredients in our product candidates as NCEs and, therefore, afford them five years of NCE data exclusivity if they are approved. If any product we develop does not receive five years of NCE exclusivity, the FDA may approve generic versions of such product three years after its date of approval, subject to the requirement that the ANDA applicant certifies to any patents listed for our products in the Orange Book. Three year exclusivity is given to a drug if the NDA includes reports of one or more new clinical investigations, other than bioavailability or bioequivalence studies, that were conducted by or for the applicant and are essential to the approval of the NDA. Manufacturers may seek to launch these generic products following the expiration of the applicable marketing exclusivity period, even if we still have patent protection for our product.

Competition that our products may face from generic versions of our products could negatively impact our future revenue, profitability and cash flows and substantially limit our ability to obtain a return on our investments in those product candidates.

Even if we complete the necessary preclinical studies and clinical trials, the marketing approval process is expensive, time consuming and uncertain and may prevent us or any future collaborators from obtaining approvals for the commercialization of some or all of our product candidates. As a result, we cannot predict when or if, and in which territories, we, or any future collaborators, will obtain marketing approval to commercialize a product candidate.

The research, testing, manufacturing, labeling, approval, selling, marketing, promotion and distribution of products are subject to extensive regulation by the FDA and comparable foreign regulatory authorities. We, and any future collaborators, are not permitted to market our product candidates in the United States or in other countries until we, or they, receive approval of a NDA from the FDA or marketing approval from applicable regulatory authorities outside the United States. Our product candidates are in various stages of development and are subject to the risks of failure inherent in drug development. We have not submitted an application for or received marketing approval for any of our product candidates in the United States or in any other jurisdiction. We have limited experience in conducting and managing the clinical trials necessary to obtain marketing approvals, including FDA approval of a NDA.

The process of obtaining marketing approvals, both in the United States and abroad, is lengthy, expensive and uncertain. It may take many years, if approval is obtained at all, and can vary substantially based upon a variety of factors, including the type, complexity and novelty of the product candidates involved. Securing marketing

[Table of Contents](#)

approval requires the submission of extensive preclinical and clinical data and supporting information to regulatory authorities for each therapeutic indication to establish the product candidate's safety and efficacy. Securing marketing approval also requires the submission of information about the product manufacturing process to, and inspection of manufacturing facilities by, the regulatory authorities. The FDA or other regulatory authorities may determine that our product candidates are not safe and effective, only moderately effective or have undesirable or unintended side effects, toxicities or other characteristics that preclude our obtaining marketing approval or prevent or limit commercial use. Any marketing approval we ultimately obtain may be limited or subject to restrictions or post-approval commitments that render the approved product not commercially viable.

In addition, changes in marketing approval policies during the development period, changes in or the enactment or promulgation of additional statutes, regulations or guidance or changes in regulatory review for each submitted product application, may cause delays in the approval or rejection of an application. Regulatory authorities have substantial discretion in the approval process and may refuse to accept any application or may decide that our data are insufficient for approval and require additional preclinical, clinical or other studies. In addition, varying interpretations of the data obtained from preclinical and clinical testing could delay, limit or prevent marketing approval of a product candidate. Any marketing approval we, or any future collaborators, ultimately obtain may be limited or subject to restrictions or post-approval commitments that render the approved product not commercially viable.

Moreover, principal investigators for our clinical trials may serve as scientific advisors or consultants to us from time to time and receive compensation in connection with such services. Under certain circumstances, we may be required to report some of these relationships to the FDA or other regulatory authority. The FDA or other regulatory authority may conclude that a financial relationship between us and a principal investigator has created a conflict of interest or otherwise affected interpretation of the study. The FDA or other regulatory authority may therefore question the integrity of the data generated at the applicable clinical trial site and the utility of the clinical trial itself may be jeopardized. This could result in a delay in approval, or rejection, of our marketing applications by the FDA or other regulatory authority, as the case may be, and may ultimately lead to the denial of marketing approval of one or more of our product candidates.

Any delay in obtaining or failure to obtain required approvals could negatively impact our ability or that of any future collaborators to generate revenue from the particular product candidate, which likely would result in significant harm to our financial position and adversely impact our stock price.

If we encounter difficulties enrolling patients in our clinical trials, our clinical development activities could be delayed or otherwise adversely affected.

We may not be able to initiate or continue clinical trials required by the FDA, EMA or other foreign regulatory agencies for RA101495 if we are unable to locate and enroll a sufficient number of eligible patients to participate in these clinical trials. We will be required to identify and enroll a sufficient number of patients with PNH, rMG and LN for each of our planned clinical trials of RA101495 in these indications. Each of these is a rare disease or indication with relatively small patient populations, which could result in slow enrollment of clinical trial participants. For example, we estimate that there are approximately 16,000 PNH patients worldwide, approximately 9,000 rMG patients in the United States and approximately 63,000 LN patients in the United States.

Patient enrollment is affected by other factors, including:

- severity of the disease under investigation;
- design of the clinical trial protocol;
- size and nature of the patient population;
- eligibility criteria for the trial in question;
- perceived risks and benefits of the product candidate under trial;
- proximity and availability of clinical trial sites for prospective patients;
- availability of competing therapies and clinical trials;
- clinicians' and patients' perceptions as to the potential advantages of the drug being studied in relation to other available therapies, including standard-of-care and any new drugs that may be approved for the

[Table of Contents](#)

indications we are investigating;

- efforts to facilitate timely enrollment in clinical trials;
- patient referral practices of physicians; and
- our ability to monitor patients adequately during and after treatment.

Further, there are only a limited number of specialist physicians who treat patients with these diseases, and major clinical centers are concentrated in a few geographic regions. We also may encounter difficulties in identifying and enrolling such patients with a stage of disease appropriate for our ongoing or future clinical trials. In addition, the process of finding and diagnosing patients may prove costly. Our inability to enroll a sufficient number of patients for any of our clinical trials would result in significant delays or may require us to abandon one or more clinical trials.

Ingredients, excipients and other materials necessary to manufacture RA101495 may not be available on commercially reasonable terms, or at all, which may adversely affect the development and commercialization of RA101495.

We and our third-party manufacturers must obtain from third-party suppliers the active pharmaceutical ingredients, excipients and primary and secondary packaging materials necessary for our contract manufacturers to produce RA101495 for our clinical trials and, to the extent approved or commercialized, for commercial distribution. There is no guarantee that we would be able to enter into all the necessary agreements with third-party suppliers that we require for the supply of such materials on commercially reasonable terms or at all. Even if we were able to secure such agreements or guarantees, our suppliers may be unable or choose not to provide us the ingredients, excipients or materials in a timely manner or in the quantities required. If we or our third-party manufacturers are unable to obtain the quantities of these ingredients, excipients or materials that are necessary for the manufacture of commercial supplies of RA101495, our ability to generate revenue from the sale of RA101495 would be materially and adversely affected. Further, if we or our third-party manufacturers are unable to obtain active pharmaceutical ingredients, excipients and materials as necessary for our clinical trials or for the manufacture of commercial supplies of our product candidates, if approved, potential regulatory approval or commercialization would be delayed, which would materially and adversely affect our ability to generate revenue from the sale of our product candidates.

Even if one of our product candidates receives marketing approval, it may fail to achieve the degree of market acceptance by physicians, patients, third-party payors and others in the medical community necessary for commercial success, in which case we may not generate significant revenues or become profitable.

We have never commercialized a product, and even if one of our product candidates is approved by the appropriate regulatory authorities for marketing and sale, it may nonetheless fail to gain sufficient market acceptance by physicians, patients, third-party payors and others in the medical community. Physicians are often reluctant to switch their patients from existing therapies even when new and potentially more effective or convenient treatments enter the market. Further, patients often acclimate to the therapy that they are currently taking and do not want to switch unless their physicians recommend switching products or they are required to switch therapies due to lack of reimbursement for existing therapies. Eculizumab is the only drug approved for the treatment of PNH, and even if we are able to obtain marketing approval of RA101495 for the treatment of PNH, we may not be able to successfully convince physicians or patients to switch from eculizumab to RA101495. In addition, even if we are able to demonstrate our product candidates' safety and efficacy to the FDA and other regulators, safety concerns in the medical community may hinder market acceptance.

Efforts to educate the medical community and third-party payors on the benefits of our product candidates may require significant resources, including management time and financial resources, and may not be successful. If any of our product candidates is approved but does not achieve an adequate level of market acceptance, we may not generate significant revenues and we may not become profitable. The degree of market acceptance of our product candidates, if approved for commercial sale, will depend on a number of factors, including:

- the efficacy and safety of the product;
- the potential advantages of the product compared to competitive therapies;
- the prevalence and severity of any side effects;

[Table of Contents](#)

- whether the product is designated under physician treatment guidelines as a first-, second- or third-line therapy;
- our ability, or the ability of any future collaborators, to offer the product for sale at competitive prices;
- the product's convenience and ease of administration compared to alternative treatments;
- the willingness of the target patient population to try, and of physicians to prescribe, the product;
- limitations or warnings, including distribution or use restrictions contained in the product's approved labeling;
- the strength of sales, marketing and distribution support;
- changes in the standard of care for the targeted indications for the product; and
- availability and amount of coverage and reimbursement from government payors, managed care plans and other third-party payors.

Even if we, or any future collaborators, are able to commercialize any product candidate that we, or they, develop, the product may become subject to unfavorable pricing regulations or third-party payor coverage and reimbursement policies, any of which could harm our business.

Patients who are provided medical treatment for their conditions generally rely on third party payors to reimburse all or part of the costs associated with their treatment. Therefore, our ability, and the ability of any future collaborators to commercialize any of our product candidates will depend in part on the extent to which coverage and reimbursement for these products and related treatments will be available from third party payors including government health administration authorities and private health coverage insurers. Third-party payors decide which medications they will cover and establish reimbursement levels. We cannot be certain that reimbursement will be available for RA101495 or any of our product candidates. Also, we cannot be certain that reimbursement policies will not reduce the demand for, or the price paid for, our products. The insurance coverage and reimbursement status of newly-approved products for orphan diseases is particularly uncertain, and failure to obtain or maintain adequate coverage and reimbursement for RA101495 or any other product candidates could limit our ability to generate revenue.

If coverage and reimbursement are not available, or reimbursement is available only to limited levels, we, or any future collaborators, may not be able to successfully commercialize our product candidates. Even if coverage is provided, the approved reimbursement amount may not be high enough to allow us, or any future collaborators, to establish or maintain pricing sufficient to realize a sufficient return on our or their investments. In the United States, no uniform policy of coverage and reimbursement for products exists among third-party payors and coverage and reimbursement for products can differ significantly from payor to payor. As a result, the coverage determination process is often a time-consuming and costly process that will require us to provide scientific and clinical support for the use of our products to each payor separately, with no assurance that coverage and adequate reimbursement will be applied consistently or obtained in the first instance.

There is significant uncertainty related to third-party payor coverage and reimbursement of newly approved drugs. Marketing approvals, pricing and reimbursement for new drug products vary widely from country to country. Some countries require approval of the sale price of a drug before it can be marketed. In many countries, the pricing review period begins after marketing or product licensing approval is granted. In some foreign markets, prescription pharmaceutical pricing remains subject to continuing governmental control even after initial approval is granted. As a result, we, or any future collaborators, might obtain marketing approval for a product in a particular country, but then be subject to price regulations that delay commercial launch of the product, possibly for lengthy time periods, which may negatively impact the revenues we are able to generate from the sale of the product in that country. Adverse pricing limitations may hinder our ability or the ability of any future collaborators to recoup our or their investment in one or more product candidates, even if our product candidates obtain marketing approval.

The healthcare industry is acutely focused on cost containment, both in the United States and elsewhere. Government authorities and other third-party payors have attempted to control costs by limiting coverage and the amount of reimbursement for particular medications, which could affect our ability or that of any future collaborators to sell our product candidates profitably. These payors may not view our products, if any, as cost-effective, and coverage and reimbursement may not be available to our customers, or those of any future collaborators, or may not be sufficient to allow our products, if any, to be marketed on a competitive basis. Cost-control initiatives could cause us, or any future collaborators, to decrease the price we, or they, might establish for

[Table of Contents](#)

products, which could result in lower than anticipated product revenues. If the prices for our products, if any, decrease or if governmental and other third-party payors do not provide coverage or adequate reimbursement, our prospects for revenue and profitability will suffer.

There may also be delays in obtaining coverage and reimbursement for newly approved drugs, and coverage may be more limited than the indications for which the drug is approved by the FDA or comparable foreign regulatory authorities. Moreover, eligibility for reimbursement does not imply that any drug will be paid for in all cases or at a rate that covers our costs, including research, development, manufacture, sale and distribution. Reimbursement rates may vary, by way of example, according to the use of the product and the clinical setting in which it is used. Reimbursement rates may also be based on reimbursement levels already set for lower cost drugs or may be incorporated into existing payments for other services.

In addition, increasingly, third-party payors are requiring higher levels of evidence of the benefits and clinical outcomes of new technologies and are challenging the prices charged. We cannot be sure that coverage will be available for any product candidate that we, or any future collaborator, commercialize and, if available, that the reimbursement rates will be adequate. Further, the net reimbursement for drug products may be subject to additional reductions if there are changes to laws that presently restrict imports of drugs from countries where they may be sold at lower prices than in the United States. An inability to promptly obtain coverage and adequate payment rates from both government-funded and private payors for any of our product candidates for which we, or any future collaborator, obtain marketing approval could significantly harm our operating results, our ability to raise capital needed to commercialize products and our overall financial condition.

If any product liability lawsuits are successfully brought against us or any of our collaborative partners, we may incur substantial liabilities and may be required to limit commercialization of our product candidates.

We face an inherent risk of product liability lawsuits related to the testing of our product candidates in seriously ill patients and will face an even greater risk if product candidates are approved by regulatory authorities and introduced commercially. Product liability claims may be brought against us or our partners by participants enrolled in our clinical trials, patients, health care providers or others using, administering or selling any of our future approved products. If we cannot successfully defend ourselves against any such claims, we may incur substantial liabilities, which may result in:

- decreased demand for any of our future approved products;
- injury to our reputation;
- withdrawal of clinical trial participants;
- termination of clinical trial sites or entire trial programs;
- significant litigation costs;
- substantial monetary awards to or costly settlements with patients or other claimants;
- product recalls or a change in the indications for which they may be used;
- loss of revenue;
- diversion of management and scientific resources from our business operations; and
- the inability to commercialize our product candidates.

If any of our product candidates are approved for commercial sale, we will be highly dependent upon consumer perceptions of us and the safety and quality of our products. We could be adversely affected if we are subject to negative publicity associated with illness or other adverse effects resulting from patients' use or misuse of our products or any similar products distributed by other companies.

Although we maintain product liability insurance coverage in the amount of up to \$5.0 million in the aggregate and clinical trial liability insurance of \$5.0 million in the aggregate, this insurance may not fully cover potential liabilities that we may incur. The cost of any product liability litigation or other proceeding, even if resolved in our favor, could be substantial. We will need to increase our insurance coverage if we commercialize any product that receives marketing approval. In addition, insurance coverage is becoming increasingly expensive. If we are unable to maintain sufficient insurance coverage at an acceptable cost or to otherwise protect against potential product liability claims, it could prevent or inhibit the development and commercial production and sale of our product candidates, which could harm our business, financial condition, results of operations and prospects.

[Table of Contents](#)

We currently have no marketing, sales or distribution infrastructure with respect to our product candidates. If we are unable to develop our sales, marketing and distribution capability on our own or through collaborations with marketing partners, we will not be successful in commercializing our product candidates.

We currently have no marketing, sales or distribution capabilities and have limited sales or marketing experience within our organization. If our product candidate RA101495 is approved, we intend either to establish a sales and marketing organization with technical expertise and supporting distribution capabilities to commercialize RA101495, or to outsource this function to a third party. Either of these options would be expensive and time consuming. Some or all of these costs may be incurred in advance of any approval of RA101495. In addition, we may not be able to hire a sales force in the United States or other target market that is sufficient in size or has adequate expertise in the medical markets that we intend to target. These risks may be particularly pronounced due to our focus on our lead indications of PNH, rMG and LN, each of which is a rare disease with relatively small patient populations. Any failure or delay in the development of our or third parties' internal sales, marketing and distribution capabilities would adversely impact the commercialization of RA101495 and other future product candidates.

With respect to our existing and future product candidates, we may choose to collaborate with third parties that have direct sales forces and established distribution systems, either to augment or to serve as an alternative to our own sales force and distribution systems. Our product revenue may be lower than if it directly marketed or sold any approved products. In addition, any revenue we receive will depend in whole or in part upon the efforts of these third parties, which may not be successful and are generally not within our control. If we are unable to enter into these arrangements on acceptable terms or at all, we may not be able to successfully commercialize any approved products. If we are not successful in commercializing any approved products, our future product revenue will suffer and we may incur significant additional losses.

The route of administration, formulation or dose for RA101495, which we are currently developing for SC self-administration, may be inadequate.

We are currently developing RA101495 for SC self-administration. Unsatisfactory drug availability due to problems relating to this route of administration or the ability of the drug to bind to its target is another potential cause of lack of efficacy of RA101495 if and when it is commercialized. C5, the target of RA101495 is predominantly found in blood. For PNH, RA101495 will be administered subcutaneously. In our recently completed Phase 1 study of RA101495 in single-ascending dose cohorts and a multiple-dose cohort, single and repeat SC doses of RA101495 were safe and well tolerated in healthy volunteers. However, if daily subcutaneous administration proves to be unfeasible, then we may need to research additional doses, formulations or routes of administration, which could delay commercialization of RA101495 and result in significant additional costs to us. Additionally, while we may offer training in SC injections, reliance on patient self-administration may lead to higher rates of user error due to poor administration procedure by patients and reduced patient compliance as compared with administration by healthcare professionals.

If we, or any future collaborators, experience any of a number of possible unforeseen events in connection with clinical trials of our product candidates, potential clinical development, marketing approval or commercialization of our product candidates could be delayed or prevented.

We, or any future collaborators, may experience numerous unforeseen events during, or as a result of, clinical trials that could delay or prevent clinical development, marketing approval or commercialization of our product candidates, including:

- our product candidates may produce unfavorable or inconclusive results;
- regulators may require us or any future collaborators, to conduct additional clinical trials or abandon product development programs;
- the number of patients required for clinical trials of our product candidates may be larger than we, or any future collaborators, anticipate, patient enrollment in these clinical trials may be slower than we, or any future collaborators, may anticipate or participants may drop out of these clinical trials at a higher rate than we, or any future collaborators, anticipate;
- the cost of planned clinical trials of our product candidates may be greater than we anticipate;

[Table of Contents](#)

- our third-party contractors or those of any future collaborators, including those manufacturing our product candidates or components or ingredients thereof or conducting clinical trials on our behalf or on behalf of any future collaborators, may fail to comply with regulatory requirements or meet their contractual obligations to us or any future collaborators in a timely manner or at all;
- regulators, IRBs or independent ethics committees may not authorize us, any future collaborators or our or their investigators to commence a clinical trial or conduct a clinical trial at a prospective trial site;
- delays in reaching or fail to reach agreement on acceptable clinical trial contracts or clinical trial protocols with prospective trial sites;
- patients who enroll in a clinical trial may misrepresent their eligibility to do so or may otherwise not comply with the clinical trial protocol, resulting in the need to drop the patients from the clinical trial, increase the needed enrollment size for the clinical trial or extend the clinical trial's duration;
- delay, suspension or termination of clinical trials of our product candidates for various reasons, including a finding that the participants are being exposed to unacceptable health risks, undesirable side effects or other unexpected characteristics of the product candidate;
- regulators, IRBs or independent ethics committees may require that we, or any future collaborators, or our or their investigators suspend or terminate clinical research for various reasons, including noncompliance with regulatory requirements or their standards of conduct, a finding that the participants are being exposed to unacceptable health risks, undesirable side effects or other unexpected characteristics of the product candidate or findings of undesirable effects caused by a chemically or mechanistically similar product or product candidate;
- the FDA or comparable foreign regulatory authorities may disagree with our, or any future collaborators', clinical trial designs or our or their interpretation of data from preclinical studies and clinical trials;
- the FDA or comparable foreign regulatory authorities may fail to approve or subsequently find fault with the manufacturing processes or facilities of third-party manufacturers with which we, or any future collaborators, enter into agreements for clinical and commercial supplies;
- the supply or quality of raw materials or manufactured product candidates or other materials necessary to conduct clinical trials of our product candidates may be insufficient, inadequate or not available at an acceptable cost, or we may experience interruptions in supply; and
- the approval policies or regulations of the FDA or comparable foreign regulatory authorities may significantly change in a manner rendering our clinical data insufficient to obtain marketing approval.

We or any future collaborators could also encounter delays if a clinical trial is suspended or terminated by us or our collaborators, by the IRBs or independent ethics committees of the institutions in which such trials are being conducted, by the DSMB for such trial or by the FDA or other regulatory authorities. Such authorities may suspend or terminate a clinical trial due to a number of factors, including failure to conduct the clinical trial in accordance with regulatory requirements or our clinical protocols, inspection of the clinical trial operations or trial site by the FDA or other regulatory authorities resulting in the imposition of a clinical hold, unforeseen safety issues or adverse side effects, failure to demonstrate a benefit from using a drug, changes in governmental regulations or administrative actions or lack of adequate funding to continue the clinical trial.

Further, conducting clinical trials in foreign countries, as we plan to do for our product candidates, presents additional risks that may delay completion of our clinical trials. These risks include the failure of enrolled patients in foreign countries to adhere to clinical protocol as a result of differences in healthcare services or cultural customs, managing additional administrative burdens associated with foreign regulatory schemes, as well as political and economic risks relevant to such foreign countries.

Product development costs for us, or any future collaborators, will increase if we, or they, experience delays in testing or pursuing marketing approvals and we, or they, may be required to obtain additional funds to complete clinical trials and prepare for possible commercialization of our product candidates. We do not know whether any preclinical tests or clinical trials will begin as planned, will need to be restructured, or will be completed on schedule or at all. Significant preclinical study or clinical trial delays also could shorten any periods during which we, or any future collaborators, may have the exclusive right to commercialize our product candidates or allow our competitors, or the competitors of any future collaborators, to bring products to market before we, or any future collaborators, do and impair our ability, or the ability of any future collaborators, to successfully commercialize our product candidates and may harm our business and results of operations. In addition, many of the

[Table of Contents](#)

factors that lead to clinical trial delays may ultimately lead to the denial of marketing approval of any of our product candidates.

Results of preclinical studies and early clinical trials may not be predictive of results of future clinical trials.

The outcome of preclinical studies and early clinical trials may not be predictive of the success of later clinical trials, and interim results of clinical trials do not necessarily predict success in the results of completed clinical trials. Many companies in the pharmaceutical and biotechnology industries have suffered significant setbacks in late-stage clinical trials after achieving positive results in earlier development, and we could face similar setbacks. The design of a clinical trial can determine whether its results will support approval of a product and flaws in the design of a clinical trial may not become apparent until the clinical trial is well advanced. We have limited experience in designing clinical trials and may be unable to design and execute a clinical trial to support marketing approval. In addition, preclinical and clinical data are often susceptible to varying interpretations and analyses. Many companies that believed their product candidates performed satisfactorily in preclinical studies and clinical trials have nonetheless failed to obtain marketing approval for the product candidates. Even if we, or any future collaborators, believe that the results of clinical trials for our product candidates warrant marketing approval, the FDA or comparable foreign regulatory authorities may disagree and may not grant marketing approval of our product candidates.

In some instances, there can be significant variability in safety or efficacy results between different clinical trials of the same product candidate due to numerous factors, including changes in trial procedures set forth in protocols, differences in the size and type of the patient populations, changes in and adherence to the dosing regimen and other clinical trial protocols and the rate of dropout among clinical trial participants. If we fail to receive positive results in clinical trials of our product candidates, the development timeline and regulatory approval and commercialization prospects for our most advanced product candidates, and, correspondingly, our business and financial prospects would be negatively impacted.

Chronic dosing of patients with RA101495 could lead to an immune response that causes adverse reactions or impairs the activity and/or efficacy of the drug, or causes other side effects.

There is a risk that chronic dosing of patients with RA101495 may lead to an immune response that causes adverse reactions or impairs the activity and/or efficacy of the drug. Patients may develop an allergic reaction to the drug and/or develop antibodies directed at the drug. Impaired drug activity could be caused by neutralization of the drug's inhibitory activity or by an increased rate of clearance of the drug from circulation. For example, one potential side effect of RA101495 that has occurred in patients receiving eculizumab, a humanized antibody against C5, is an increased incidence of meningococcal infections as a result of inhibition of the terminal complement system in a manner similar to RA101495. As a result, patients receiving RA101495 may also require immunization with a meningococcal vaccine and prophylactic antibiotics.

Any immune response that causes adverse reactions or impairs the activity of the drug could cause a delay in or termination of our development of RA101495, which would have a material adverse effect on our financial condition and results of operation.

Risks Related to Regulatory Approval and Marketing of Our Product Candidates and Other Legal Compliance Matters

We plan to seek orphan drug designation for RA101495, but we may be unable to obtain such designation or to maintain the benefits associated with orphan drug status, including market exclusivity, even if that designation is granted.

We plan to seek orphan drug designation for RA101495 in specific orphan indications in which there is a medically plausible basis for its use and may seek orphan drug designation for other preclinical product candidates in our pipeline or that we may develop. In the United States, orphan drug designation entitles a party to financial incentives such as opportunities for grant funding towards clinical trial costs, tax advantages, and user-fee waivers. After the FDA grants orphan drug designation, the generic identity of the drug and its potential orphan use are

[Table of Contents](#)

disclosed publicly by the FDA. Orphan drug designation does not convey any advantage in, or shorten the duration of, the regulatory review and approval process. Although we intend to seek orphan drug designation for RA101495, we may never receive such designation. Moreover, obtaining orphan drug designation for one indication for RA101495 does not mean we will be able to obtain such designation for another indication.

If a product that has orphan drug designation subsequently receives the first FDA approval for a particular active ingredient for the disease for which it has such designation, the product is entitled to orphan drug exclusivity, which means that the FDA may not approve any other applications, including a NDA, to market the same drug for the same indication for seven years, except in limited circumstances such as if the FDA finds that the holder of the orphan drug exclusivity has not shown that it can assure the availability of sufficient quantities of the orphan drug to meet the needs of patients with the disease or condition for which the drug was designated. Similarly, the FDA can subsequently approve a drug with the same active moiety for the same condition during the exclusivity period if the FDA concludes that the later drug is clinically superior, meaning the later drug is safer, more effective, or makes a major contribution to patient care. Even if we were to obtain orphan drug designation for RA101495, we may not be the first to obtain marketing approval for any particular orphan indication due to the uncertainties associated with developing pharmaceutical products, and thus approval of RA101495 could be blocked for seven years if another company previously obtained approval and orphan drug exclusivity for the same drug and same condition. If we do obtain exclusive marketing rights in the United States, they may be limited if we seek approval for an indication broader than the orphan designated indication and may be lost if the FDA later determines that the request for designation was materially defective or if we are unable to assure sufficient quantities of the product to meet the needs of the relevant patients. Further, exclusivity may not effectively protect the product from competition because different drugs with different active moieties can be approved for the same condition, the same drugs can be approved for different indications and might then be used off-label in our approved indication, and different drugs for the same condition may already be approved and commercially available.

In Europe, the period of orphan drug exclusivity is ten years, although it may be reduced to six years if, at the end of the fifth year, it is established that the criteria for orphan drug designation are no longer met, in other words, when it is shown on the basis of available evidence that the product is sufficiently profitable not to justify maintenance of market exclusivity. In September 2016, the EMA's Committee for Orphan Medicinal Products adopted a positive opinion recommending RA101495 for the treatment of PNH for designation as an orphan medicinal product to the European Commission, which granted us orphan designation for RA101495 for the treatment of PNH in October 2016.

Laws and regulations governing any international operations we may have in the future may preclude us from developing, manufacturing and selling certain products outside of the United States and require us to develop and implement costly compliance programs.

If we further expand our operations outside of the United States, we must dedicate additional resources to comply with numerous laws and regulations in each jurisdiction in which we plan to operate. The Foreign Corrupt Practices Act, or FCPA, prohibits any U.S. individual or business from paying, offering, authorizing payment or offering of anything of value, directly or indirectly, to any foreign official, political party or candidate for the purpose of influencing any act or decision of the foreign entity in order to assist the individual or business in obtaining or retaining business. The FCPA also obligates companies whose securities are listed in the United States to comply with certain accounting provisions requiring the company to maintain books and records that accurately and fairly reflect all transactions of the corporation, including international subsidiaries, and to devise and maintain an adequate system of internal accounting controls for international operations.

Compliance with the FCPA is expensive and difficult, particularly in countries in which corruption is a recognized problem. In addition, the FCPA presents particular challenges in the pharmaceutical industry, because, in many countries, hospitals are operated by the government, and doctors and other hospital employees are considered foreign officials. Certain payments to hospitals in connection with clinical trials and other work have been deemed to be improper payments to government officials and have led to FCPA enforcement actions.

Various laws, regulations and executive orders also restrict the use and dissemination outside of the United States, or the sharing with certain non-U.S. nationals, of information classified for national security purposes, as well as certain products and technical data relating to those products. If we expand our presence outside of the

[Table of Contents](#)

United States, it will require us to dedicate additional resources to comply with these laws, and these laws may preclude us from developing, manufacturing, or selling certain products and product candidates outside of the United States, which could limit our growth potential and increase our development costs.

The failure to comply with laws governing international business practices may result in substantial civil and criminal penalties and suspension or debarment from government contracting. The Securities and Exchange Commission, or SEC, also may suspend or bar issuers from trading securities on U.S. exchanges for violations of the FCPA's accounting provisions.

Governments outside the United States tend to impose strict price controls, which may adversely affect our revenues, if any.

In some countries, such as the countries of the European Union, the pricing of prescription pharmaceuticals is subject to governmental control. In these countries, pricing negotiations with governmental authorities can take considerable time after the receipt of marketing approval for a product. To obtain reimbursement or pricing approval in some countries, we, or any future collaborators, may be required to conduct a clinical trial that compares the cost-effectiveness of our product to other available therapies. If reimbursement of our products is unavailable or limited in scope or amount, or if pricing is set at unsatisfactory levels, our business could be harmed.

We are subject to extensive government regulation and the failure to comply with these regulations may have a material adverse effect on our operations and business.

Both before and after approval of any product, we and our suppliers, contract manufacturers and clinical investigators are subject to extensive regulation by governmental authorities in the United States and other countries, covering, among other things, testing, manufacturing, quality control, clinical trials, post-marketing studies, labeling, advertising, promotion, distribution, import and export, governmental pricing, price reporting and rebate requirements. Failure to comply with applicable requirements could result in one or more of the following actions: warning letters; unanticipated expenditures; delays in approval or refusal to approve a product candidate; product recall or seizure; interruption of manufacturing or clinical trials; operating or marketing restrictions; injunctions; criminal prosecution and civil or criminal penalties including fines and other monetary penalties; adverse publicity; and disruptions to our business. Further, government investigations into potential violations of these laws would require us to expend considerable resources and face adverse publicity and the potential disruption of our business even if we are ultimately found not to have committed a violation.

Obtaining FDA approval of our product candidates requires substantial time, effort and financial resources and may be subject to both expected and unforeseen delays, and there can be no assurance that any approval will be granted on any of our product candidates on a timely basis, if at all. The FDA may decide that our data are insufficient for approval of our product candidates and require additional preclinical, clinical or other studies or additional work related to chemistry, manufacturing and controls. In addition, we, the FDA, IRBs or independent ethics committees may suspend or terminate human clinical trials at any time on various grounds, including a finding that the patients are or would be exposed to an unacceptable health risk or because of the way in which the investigators on which we rely carry out the trials. If we are required to conduct additional trials or to conduct other testing of our product candidates beyond that which we currently contemplate for regulatory approval, if we are unable to complete successfully our clinical trials or other testing, or if the results of these and other trials or tests fail to demonstrate efficacy or raise safety concerns, we may face substantial additional expenses, be delayed in obtaining marketing approval for our product candidates or may never obtain marketing approval.

We are also required to comply with extensive governmental regulatory requirements after a product has received marketing authorization. Governing regulatory authorities may require post-marketing studies that may negatively impact the commercial viability of a product. Once on the market, a product may become associated with previously undetected adverse effects and/or may develop manufacturing difficulties. As a result of any of these or other problems, a product's regulatory approval could be withdrawn, which could harm our business and operating results.

Even if we obtain FDA approval of RA101495 or any of our other product candidates, we or our partners may never obtain approval or commercialize our products outside of the United States.

[Table of Contents](#)

In order to market any products outside of the United States, we must establish and comply with numerous and varying regulatory requirements of other countries regarding clinical trial design, safety and efficacy. Clinical trials conducted in one country may not be accepted by regulatory authorities in other countries, and regulatory approval in one country does not mean that regulatory approval will be obtained in any other country. Approval procedures vary among countries and can involve additional product testing and validation and additional administrative review periods. Seeking foreign regulatory approvals could result in significant delays, difficulties and costs for us and may require additional preclinical studies or clinical trials which would be costly and time consuming and could delay or prevent introduction of RA101495 or any of our other product candidates in those countries. We do not have experience in obtaining regulatory approval in international markets. If we or our partners fail to comply with regulatory requirements or to obtain and maintain required approvals, our target market will be reduced and our ability to realize the full market potential of our product candidates will be harmed.

Current and future legislation may increase the difficulty and cost for us and any future collaborators to obtain marketing approval of and commercialize our product candidates and affect the prices we, or they, may obtain.

In the United States and some foreign jurisdictions, there have been a number of legislative and regulatory changes and proposed changes regarding the healthcare system that could, among other things, prevent or delay marketing approval of our product candidates, restrict or regulate post-approval activities and affect our ability, or the ability of any future collaborators, to profitably sell any products for which we, or they, obtain marketing approval. We expect that current laws, as well as other healthcare reform measures that may be adopted in the future, may result in more rigorous coverage criteria and in additional downward pressure on the price that we, or any future collaborators, may receive for any approved products.

In March 2010 for example, President Obama signed into law the Patient Protection and Affordable Care Act, as amended by the Health Care and Education Reconciliation Act, or collectively the Affordable Care Act, or ACA. Among the provisions of the ACA of potential importance to our business and our product candidates are the following:

- an annual, non-deductible fee on any entity that manufactures or imports specified branded prescription drugs and biologic products;
- an increase in the statutory minimum rebates a manufacturer must pay under the Medicaid Drug Rebate Program;
- expansion of healthcare fraud and abuse laws, including the civil False Claims Act and the federal Anti-Kickback Statute, new government investigative powers and enhanced penalties for noncompliance;
- a new Medicare Part D coverage gap discount program, in which manufacturers must agree to offer 50% point-of-sale discounts off negotiated prices;
- extension of manufacturers' Medicaid rebate liability;
- expansion of eligibility criteria for Medicaid programs;
- expansion of the entities eligible for discounts under the Public Health Service pharmaceutical pricing program;
- new requirements to report certain financial arrangements with physicians and teaching hospitals;
- a new requirement to annually report drug samples that manufacturers and distributors provide to physicians; and
- a new Patient-Centered Outcomes Research Institute to oversee, identify priorities in, and conduct comparative clinical effectiveness research, along with funding for such research.

In addition, other legislative changes have been proposed and adopted since the ACA was enacted. These changes include the Budget Control Act of 2011, which, among other things, led to aggregate reductions to Medicare payments to providers of 2% per fiscal year, which went into effect on April 1, 2013 and will remain in effect through 2025 unless additional Congressional action is taken. On January 2, 2013, the American Taxpayer Relief Act of 2012 was signed into law, which, among other things, further reduced Medicare payments to several providers and increased the statute of limitations period for the government to recover overpayments to providers from three to five years. These new laws may result in additional reductions in Medicare and other healthcare funding. We expect that additional state and federal healthcare reform measures will be adopted in the future, any of which could limit the amounts that federal and state governments will pay for healthcare products and services, which could result in reduced demand for our product candidates or additional pricing pressures.

[Table of Contents](#)

Legislative and regulatory proposals have been made to expand post-approval requirements and restrict sales and promotional activities for pharmaceutical products. We cannot be sure whether additional legislative changes will be enacted, or whether the FDA regulations, guidance or interpretations will be changed, or what the impact of such changes on the marketing approvals of our product candidates, if any, may be. In addition, increased scrutiny by the United States Congress of the FDA's approval process may significantly delay or prevent marketing approval, as well as subject us and any future collaborators to more stringent product labeling and post-marketing testing and other requirements.

Our relationships with customers and third-party payors, among others, will be subject to applicable anti-kickback, fraud and abuse and other healthcare laws and regulations, which could expose us to penalties, including criminal sanctions, civil penalties, contractual damages, reputational harm, fines, disgorgement, exclusion from participation in government healthcare programs, curtailment or restricting of our operations, and diminished profits and future earnings.

Healthcare providers, physicians and third party-payors will play a primary role in the recommendation and prescription of any products for which we obtain marketing approval. Our future arrangements with third-party payors and customers, if any, will subject us to broadly applicable fraud and abuse and other healthcare laws and regulations. The laws and regulations may constrain the business or financial arrangements and relationships through which we market, sell and distribute any products for which we obtain marketing approval. These include the following:

- *Anti-kickback statute.* The federal Anti-Kickback Statute prohibits, among other things, persons and entities from knowingly and willfully soliciting, offering, receiving or providing remuneration, directly or indirectly, in cash or in kind, to induce or reward, or in return for, either the referral of an individual for, or the purchase, order or recommendation or arranging of, any good or service, for which payment may be made under a federal healthcare program such as Medicare and Medicaid. This statute has been interpreted to apply to arrangements between pharmaceutical manufacturers on the one hand and prescribers, purchasers and formulary managers on the other. Although there are several statutory exceptions and regulatory safe harbors protecting certain common activities from prosecution, they are drawn narrowly, and practices that involve remuneration intended to induce prescribing, purchasing or recommending may be subject to scrutiny if they do not qualify for an exception or safe harbor. A person or entity can be found guilty of violating the federal Anti-Kickback Statute without actual knowledge of the statute or specific intent to violate it. In addition, the government may assert that a claim including items or services resulting from a violation of the federal Anti-Kickback Statute constitutes a false or fraudulent claim for purposes of the federal False Claims Act or federal civil money penalties statute;
- *False claims laws.* The federal false claims and civil monetary penalties laws, including the federal civil False Claims Act, impose criminal and civil penalties, including through civil whistleblower or *qui tam* actions against individuals or entities for, among other things, knowingly presenting, or causing to be presented false or fraudulent claims for payment by a federal healthcare program or making a false statement or record material to payment of a false claim or avoiding, decreasing or concealing an obligation to pay money to the federal government, with potential liability including mandatory treble damages and significant per-claim penalties, currently set at \$10,781 to \$21,563 per false claim;
- *HIPAA.* The federal Health Insurance Portability and Accountability Act of 1996, or HIPAA, imposes criminal and civil liability for, among other things, executing a scheme to defraud any healthcare benefit program or making false statements relating to healthcare matters. Similar to the federal Anti-Kickback Statute, a person or entity does not need to have actual knowledge of the statute or specific intent to violate it in order to have committed a violation. Additionally, HIPAA as amended by the Health Information Technology for Economic and Clinical Health Act and its implementing regulations, also imposes obligations on covered entities and their business associates, including mandatory contractual terms and technical safeguards, with respect to maintaining the privacy, security and transmission of individually identifiable health information;
- *Transparency requirements.* The provision within the ACA commonly referred to as the federal Physician Payments Sunshine Act requires certain manufacturers of drugs, devices, biologics, and medical supplies for which payment is available under Medicare, Medicaid, or the Children's Health Insurance Program, with specific exceptions, to report annually to the Centers for Medicare & Medicaid Services, or CMS, information related to payments or transfers of value made to physicians and teaching hospitals, as

[Table of Contents](#)

well as information regarding ownership and investment interests held by physicians and their immediate family members; and

- *Analogous state and foreign laws.* Analogous state and foreign fraud and abuse laws and regulations, such as state anti-kickback and false claims laws, can apply to sales or marketing arrangements, and claims involving healthcare items or services reimbursed by non-governmental third party payors, and are generally broad and are enforced by many different foreign and state agencies as well as through private actions. Some state laws require pharmaceutical companies to comply with the pharmaceutical industry's voluntary compliance guidelines and the relevant compliance guidance promulgated by the federal government and require drug manufacturers to report information related to payments and other transfers of value to physicians and other healthcare providers or marketing expenditures. State and foreign laws also govern the privacy and security of health information in some circumstances, many of which differ from each other in significant ways and often are not pre-empted by HIPAA, thus complicating compliance efforts.

Efforts to ensure that our business arrangements with third parties, and our business generally, will comply with applicable healthcare laws and regulations will involve substantial costs. It is possible that governmental authorities will conclude that our business practices may not comply with current or future statutes, regulations or case law involving applicable fraud and abuse or other healthcare laws and regulations. If our operations are found to be in violation of any of these laws or any other governmental regulations that may apply to us, we may be subject to significant civil, criminal and administrative penalties, damages, fines, imprisonment, exclusion of products from government funded healthcare programs, such as Medicare and Medicaid, disgorgement, contractual damages, reputational harm, and the curtailment or restructuring of our operations. Defending against any such actions can be costly, time-consuming and may require significant financial and personnel resources. Therefore, even if we are successful in defending against any such actions that may be brought against us, our business may be impaired. Further, if any of the physicians or other healthcare providers or entities with whom we expect to do business is found to be not in compliance with applicable laws, they may be subject to criminal, civil or administrative sanctions, including exclusions from government funded healthcare programs.

Even if we, or any future collaborators, obtain marketing approvals for our product candidates, the terms of approvals and ongoing regulation of our products may limit how we manufacture and market our products, which could impair our ability to generate revenue.

Once marketing approval has been granted, an approved product and its manufacturer and marketer are subject to ongoing review and extensive regulation. We, and any future collaborators, must therefore comply with requirements concerning advertising and promotion for any of our product candidates for which we or they obtain marketing approval. Promotional communications with respect to prescription drugs are subject to a variety of legal and regulatory restrictions and must be consistent with the information in the product's approved labeling. Thus, we and any future collaborators will not be able to promote any products we develop for indications or uses for which they are not approved.

In addition, manufacturers of approved products and those manufacturers' facilities are required to comply with extensive FDA requirements, including ensuring that quality control and manufacturing procedures conform to current Good Manufacturing Practices, or cGMPs, which include requirements relating to quality control and quality assurance as well as the corresponding maintenance of records and documentation and reporting requirements. We, our contract manufacturers, any future collaborators and their contract manufacturers could be subject to periodic unannounced inspections by the FDA to monitor and ensure compliance with cGMPs.

Accordingly, assuming we, or any future collaborators, receive marketing approval for one or more of our product candidates, we, and any future collaborators, and our and their contract manufacturers will continue to expend time, money and effort in all areas of regulatory compliance, including manufacturing, production, product surveillance and quality control.

If we, and any future collaborators, are not able to comply with post-approval regulatory requirements, we, and any future collaborators, could have the marketing approvals for our products withdrawn by regulatory authorities and our, or any future collaborators', ability to market any future products could be limited, which could

[Table of Contents](#)

adversely affect our ability to achieve or sustain profitability. Further, the cost of compliance with post-approval regulations may have a negative effect on our operating results and financial condition.

Any of our product candidates for which we, or any future collaborators, obtain marketing approval in the future could be subject to post-marketing restrictions or withdrawal from the market and we, or any future collaborators, may be subject to substantial penalties if we, or they, fail to comply with regulatory requirements or if we, or they, experience unanticipated problems with our products following approval.

Any of our product candidates for which we, or any future collaborators, obtain marketing approval, as well as the manufacturing processes, post-approval studies and measures, labeling, advertising and promotional activities for such product, among other things, will be subject to ongoing requirements of and review by the FDA, the EMA and other regulatory authorities. These requirements include submissions of safety and other post-marketing information and reports, registration and listing requirements, requirements relating to manufacturing, quality control, quality assurance and corresponding maintenance of records and documents, requirements regarding the distribution of samples to physicians and recordkeeping. Even if marketing approval of a product candidate is granted, the approval may be subject to limitations on the indicated uses for which the product may be marketed or to the conditions of approval, including the requirement to implement a Risk Evaluation and Mitigation Strategy.

The FDA, the EMA and other regulatory authorities may also impose requirements for costly post-marketing studies or clinical trials and surveillance to monitor the safety or efficacy of a product. The FDA and other agencies, including the Department of Justice, closely regulate and monitor the post-approval marketing and promotion of products to ensure that they are manufactured, marketed and distributed only for the approved indications and in accordance with the provisions of the approved labeling. The FDA imposes stringent restrictions on manufacturers' communications regarding off-label use and if we, or any future collaborators, do not market any of our product candidates for which we, or they, receive marketing approval for only their approved indications, we, or they, may be subject to warnings or enforcement action for off-label marketing. Violation of the FDCA and other statutes relating to the promotion and advertising of prescription drugs may lead to investigations or allegations of violations of federal and state health care fraud and abuse laws and state consumer protection laws, including the False Claims Act.

In addition, later discovery of previously unknown adverse events or other problems with our products or their manufacturers or manufacturing processes, or failure to comply with regulatory requirements, may yield various results, including:

- restrictions on the manufacturing of such products;
- restrictions on the labeling or marketing of such products;
- restrictions on product distribution or use;
- requirements to conduct post-marketing studies or clinical trials;
- warning letters or untitled letters;
- withdrawal of the products from the market;
- refusal to approve pending applications or supplements to approved applications that we submit;
- recall of products;
- restrictions on coverage by third-party payors;
- fines, restitution or disgorgement of profits or revenues;
- suspension or withdrawal of marketing approvals;
- refusal to permit the import or export of products;
- product seizure; or
- injunctions or the imposition of civil or criminal penalties.

Additional time may be required to obtain regulatory approval for our product candidates because they are combination products.

Because certain of our product candidates are designed to be self-administered SC by patients and may be packaged as pre-filled cartridges or pens, they may be regulated as drug/device combination products that require coordination within the FDA and similar foreign regulatory agencies for review of their device and drug components. Although the FDA and similar foreign regulatory agencies have systems in place for the review and

[Table of Contents](#)

approval of combination products such as ours, we may experience delays in the development and commercialization of our product candidates due to regulatory timing constraints and uncertainties in the product development and approval process.

If we fail to comply with environmental, health and safety laws and regulations, we could become subject to fines or penalties or incur costs that could harm our business.

We are subject to numerous environmental, health and safety laws and regulations, including those governing laboratory procedures and the handling, use, storage, treatment and disposal of hazardous materials and wastes. From time to time and in the future, our operations may involve the use of hazardous and flammable materials, including chemicals and biological materials, and may also produce hazardous waste products. Even if we contract with third parties for the disposal of these materials and waste products, we cannot completely eliminate the risk of contamination or injury resulting from these materials. In the event of contamination or injury resulting from the use or disposal of our hazardous materials, we could be held liable for any resulting damages, and any liability could exceed our resources. We also could incur significant costs associated with civil or criminal fines and penalties for failure to comply with such laws and regulations.

We maintain workers' compensation insurance to cover us for costs and expenses we may incur due to injuries to our employees resulting from the use of hazardous materials, but this insurance may not provide adequate coverage against potential liabilities. However, we do not maintain insurance for environmental liability or toxic tort claims that may be asserted against us.

In addition, we may incur substantial costs in order to comply with current or future environmental, health and safety laws and regulations. Current or future environmental laws and regulations may impair our research, development or production efforts. In addition, failure to comply with these laws and regulations may result in substantial fines, penalties or other sanctions.

Risks Related to Our Dependence on Third Parties

We rely on third parties to conduct our clinical trials. If they do not perform satisfactorily, our business could be harmed.

We do not independently conduct clinical trials of any of our product candidates. We rely on third parties, such as contract research organizations, or CROs, clinical data management organizations, medical institutions and clinical investigators, to conduct these clinical trials and expect to rely on these third parties to conduct clinical trials of any other product candidate that we develop. Any of these third parties may terminate their engagements with us under certain circumstances. We may not be able to enter into alternative arrangements or do so on commercially reasonable terms. In addition, there is a natural transition period when a new contract research organization begins work. As a result, delays would likely occur, which could negatively impact our ability to meet our expected clinical development timelines and harm our business, financial condition and prospects.

Further, although our reliance on these third parties for clinical development activities limits our control over these activities, we remain responsible for ensuring that each of our trials is conducted in accordance with the applicable protocol, legal and regulatory requirements and scientific standards. For example, notwithstanding the obligations of a CRO for a trial of one of our product candidates, we remain responsible for ensuring that each of our clinical trials is conducted in accordance with the general investigational plan and protocols for the trial. Moreover, the FDA requires us to comply with requirements, commonly referred to as Good Clinical Practices, or GCPs, for conducting, recording and reporting the results of clinical trials to assure that data and reported results are credible and accurate and that the rights, integrity and confidentiality of trial participants are protected. The FDA enforces these GCPs through periodic inspections of trial sponsors, principal investigators, clinical trial sites and IRBs. If we or our third-party contractors fail to comply with applicable GCPs, the clinical data generated in our clinical trials may be deemed unreliable and the FDA may require us to perform additional clinical trials before approving our product candidates, which would delay the marketing approval process. We cannot be certain that, upon inspection, the FDA will determine that any of our clinical trials comply with GCPs. We are also required to register clinical trials and post the results of completed clinical trials on a government-sponsored database,

[Table of Contents](#)

ClinicalTrials.gov, within certain timeframes. Failure to do so can result in fines, adverse publicity and civil and criminal sanctions.

Furthermore, the third parties conducting clinical trials on our behalf are not our employees, and except for remedies available to us under our agreements with such contractors, we cannot control whether or not they devote sufficient time, skill and resources to our ongoing development programs. These contractors may also have relationships with other commercial entities, including our competitors, for whom they may also be conducting clinical trials or other drug development activities, which could impede their ability to devote appropriate time to our clinical programs. If these third parties, including clinical investigators, do not successfully carry out their contractual duties, meet expected deadlines or conduct our clinical trials in accordance with regulatory requirements or our stated protocols, we may not be able to obtain, or may be delayed in obtaining, marketing approvals for our product candidates. If that occurs, we will not be able to, or may be delayed in our efforts to, successfully commercialize our product candidates. In such an event, our financial results and the commercial prospects for any product candidates that we seek to develop could be harmed, our costs could increase and our ability to generate revenues could be delayed, impaired or foreclosed.

Use of third parties to manufacture our product candidates may increase the risk that we will not have sufficient quantities of our product candidates, products, or necessary quantities at an acceptable cost.

We do not own or operate manufacturing facilities for the production of clinical or commercial quantities of our product candidates, and we lack the resources and the capabilities to do so. As a result, we currently rely on third parties for supply of the active pharmaceutical ingredients, or API, in our product candidates. Our current strategy is to outsource all manufacturing of our product candidates and products to third parties.

We currently engage third-party manufacturers to provide the API, and other third parties to provide services for the final drug product formulation of RA101495 that is being used in our clinical trials. Although we believe that there are several potential alternative manufacturers who could manufacture RA101495, we may incur added costs and delays in identifying and qualifying any such replacement. In addition, we have not yet concluded a commercial supply contract with any commercial manufacturer. There is no assurance that we will be able to timely secure needed supply arrangements on satisfactory terms, or at all. Our failure to secure these arrangements as needed could have a material adverse effect on our ability to complete the development of our product candidates or, to commercialize them, if approved. We may be unable to conclude agreements for commercial supply with third-party manufacturers, or may be unable to do so on acceptable terms. There may be difficulties in scaling up to commercial quantities and formulation of RA101495 and the costs of manufacturing could be prohibitive.

Even if we are able to establish and maintain arrangements with third-party manufacturers, reliance on third-party manufacturers entails additional risks, including:

- reliance on third-parties for manufacturing process development, regulatory compliance and quality assurance;
- limitations on supply availability resulting from capacity and scheduling constraints of third-parties;
- the possible breach of manufacturing agreements by third-parties because of factors beyond our control; and
- the possible termination or non-renewal of the manufacturing agreements by the third-party, at a time that is costly or inconvenient to us.

If we do not maintain our key manufacturing relationships, we may fail to find replacement manufacturers or develop our own manufacturing capabilities, which could delay or impair our ability to obtain regulatory approval for our products. If we do find replacement manufacturers, we may not be able to enter into agreements with them on terms and conditions favorable to us and there could be a substantial delay before new facilities could be qualified and registered with the FDA and other foreign regulatory authorities.

Our lead product candidate may ultimately be regulated as a drug/device combination product. Third-party manufacturers may not be able to comply with the cGMP regulatory requirements applicable to drugs and drug/device combination products, including applicable provisions of the FDA's drug cGMP regulations, device cGMP requirements embodied in the Quality System Regulation, or QSR, or similar regulatory requirements outside

[Table of Contents](#)

the United States. Our failure, or the failure of our third-party manufacturers, to comply with applicable regulations could result in sanctions being imposed on us, including clinical holds, fines, injunctions, civil penalties, delays, suspension or withdrawal of approvals, seizures or voluntary recalls of product candidates, operating restrictions and criminal prosecutions, any of which could significantly affect supplies of our product candidates. The facilities used by our contract manufacturers to manufacture our product candidates must be evaluated by the FDA pursuant to inspections that will be conducted after we submit our NDA to the FDA. We do not control the manufacturing process of, and are completely dependent on, our contract manufacturing partners for compliance with cGMPs. If our contract manufacturers cannot successfully manufacture material that conforms to our specifications and the strict regulatory requirements of the FDA or others, we may not be able to secure and/or maintain regulatory approval for our product manufactured at these facilities. In addition, we have no control over the ability of our contract manufacturers to maintain adequate quality control, quality assurance and qualified personnel. If the FDA finds deficiencies or a comparable foreign regulatory authority does not approve these facilities for the manufacture of our product candidates or if it withdraws any such approval in the future, we may need to find alternative manufacturing facilities, which would significantly impact our ability to develop, obtain regulatory approval for or market our product candidates, if approved. Contract manufacturers may face manufacturing or quality control problems causing drug substance production and shipment delays or a situation where the contractor may not be able to maintain compliance with the applicable cGMP requirements. Any failure to comply with cGMP requirements or other FDA, EMA and comparable foreign regulatory requirements could adversely affect our clinical research activities and our ability to develop our product candidates and market our products following approval.

The FDA and other foreign regulatory authorities require manufacturers to register manufacturing facilities. The FDA and corresponding foreign regulators also inspect these facilities to confirm compliance with cGMPs. Contract manufacturers may face manufacturing or quality control problems causing drug substance production and shipment delays or a situation where the contractor may not be able to maintain compliance with the applicable cGMP requirements. Any failure to comply with cGMP requirements or other FDA, EMA and comparable foreign regulatory requirements could adversely affect our clinical research activities and our ability to develop our product candidates and market our products following approval.

If our third-party manufacturer of our product candidates is unable to increase the scale of its production of our product candidates, and/or increase the product yield of its manufacturing, then our costs to manufacture the product may increase and commercialization may be delayed.

In order to produce sufficient quantities to meet the demand for clinical trials and, if approved, subsequent commercialization of RA101495 or any of our other product candidates in our pipeline or that we may develop, our third party manufacturer will be required to increase its production and optimize its manufacturing processes while maintaining the quality of the product. The transition to larger scale production could prove difficult. In addition, if our third party manufacturer is not able to optimize its manufacturing process to increase the product yield for our product candidates, or if it is unable to produce increased amounts of our product candidates while maintaining the quality of the product, then we may not be able to meet the demands of clinical trials or market demands, which could decrease our ability to generate profits and have a material adverse impact on our business and results of operation.

We may need to maintain licenses for active ingredients from third parties to develop and commercialize some of our product candidates, which could increase our development costs and delay our ability to commercialize those product candidates.

Should we decide to use active pharmaceutical ingredients in any of our product candidates that are proprietary to one or more third parties, we would need to maintain licenses to those active ingredients from those third parties. If we are unable to gain or continue to access rights to these active ingredients prior to conducting preclinical toxicology studies intended to support clinical trials, we may need to develop alternate product candidates from these programs by either accessing or developing alternate active ingredients, resulting in increased development costs and delays in commercialization of these product candidates. If we are unable to gain or maintain continued access rights to the desired active ingredients on commercially reasonable terms or develop suitable alternate active ingredients, we may not be able to commercialize product candidates from these programs.

[Table of Contents](#)

We enter into various contracts in the normal course of our business in which we indemnify the other party to the contract. In the event we have to perform under these indemnification provisions, it could have a material adverse effect on our business, financial condition and results of operations.

In the normal course of business, we periodically enter into academic, commercial, service, collaboration, licensing, consulting and other agreements that contain indemnification provisions. With respect to our academic and other research agreements, we typically indemnify the institution and related parties from losses arising from claims relating to the products, processes or services made, used, sold or performed pursuant to the agreements for which we have secured licenses, and from claims arising from our or our sublicensees' exercise of rights under the agreement. With respect to our commercial agreements, we indemnify our vendors from any third-party product liability claims that could result from the production, use or consumption of the product, as well as for alleged infringements of any patent or other intellectual property right by a third party.

Should our obligation under an indemnification provision exceed applicable insurance coverage or if we were denied insurance coverage, our business, financial condition and results of operations could be adversely affected. Similarly, if we are relying on a collaborator to indemnify us and the collaborator is denied insurance coverage or the indemnification obligation exceeds the applicable insurance coverage and does not have other assets available to indemnify us, our business, financial condition and results of operations could be adversely affected.

We expect to seek to establish collaborations and, if we are not able to establish them on commercially reasonable terms, we may have to alter our development and commercialization plans.

We expect to seek one or more collaborators for the development and commercialization of one or more of our product candidates. For example, we started collaborating with Merck in 2013. Likely collaborators may include large and mid-size pharmaceutical companies, regional and national pharmaceutical companies and biotechnology companies. In addition, if we are able to obtain marketing approval for product candidates from foreign regulatory authorities, we intend to enter into strategic relationships with international biotechnology or pharmaceutical companies for the commercialization of such product candidates outside of the United States.

We face significant competition in seeking appropriate collaborators. Whether we reach a definitive agreement for a collaboration will depend, among other things, upon our assessment of the collaborator's resources and expertise, the terms and conditions of the proposed collaboration and the proposed collaborator's evaluation of a number of factors. Those factors may include the potential differentiation of our product candidate from competing product candidates, design or results of clinical trials, the likelihood of approval by the FDA, the EMA or comparable foreign regulatory authorities and the regulatory pathway for any such approval, the potential market for the product candidate, the costs and complexities of manufacturing and delivering the product to patients and the potential of competing products. The collaborator may also consider alternative product candidates or technologies for similar indications that may be available for collaboration and whether such a collaboration could be more attractive than the one with us for our product candidate. If we elect to increase our expenditures to fund development or commercialization activities on our own, we may need to obtain additional capital, which may not be available to us on acceptable terms or at all. If we do not have sufficient funds, we may not be able to further develop our product candidates or bring them to market and generate product revenue.

Collaborations are complex and time-consuming to negotiate and document. Further, there have been a significant number of recent business combinations among large pharmaceutical companies that have resulted in a reduced number of potential future collaborators. Any collaboration agreements that we enter into in the future may contain restrictions on our ability to enter into potential collaborations or to otherwise develop specified product candidates. We may not be able to negotiate collaborations on a timely basis, on acceptable terms, or at all. If we are unable to do so, we may have to curtail the development of the product candidate for which we are seeking to collaborate, reduce or delay its development program or one or more of our other development programs, delay its potential commercialization or reduce the scope of any sales or marketing activities, or increase our expenditures and undertake development or commercialization activities at our own expense.

If we enter into collaborations with third parties for the development and commercialization of our product candidates, our prospects with respect to those product candidates will depend in significant part on the success of those collaborations.

[Table of Contents](#)

We expect to enter into additional collaborations for the development and commercialization of certain of our product candidates. If we enter into such collaborations, we will have limited control over the amount and timing of resources that our collaborators will dedicate to the development or commercialization of our product candidates. Our ability to generate revenues from these arrangements will depend on any future collaborators' abilities to successfully perform the functions assigned to them in these arrangements. In addition, any future collaborators may have the right to abandon research or development projects and terminate applicable agreements, including funding obligations, prior to or upon the expiration of the agreed upon terms.

Collaborations involving our product candidates pose a number of risks, including the following:

- collaborators have significant discretion in determining the efforts and resources that they will apply to these collaborations;
- collaborators may not perform their obligations as expected;
- collaborators may not pursue development and commercialization of our product candidates or may elect not to continue or renew development or commercialization programs, based on clinical trial results, changes in the collaborators' strategic focus or available funding or external factors, such as an acquisition, that divert resources or create competing priorities;
- collaborators may delay clinical trials, provide insufficient funding for a clinical trial program, stop a clinical trial or abandon a product candidate, repeat or conduct new clinical trials or require a new formulation of a product candidate for clinical testing;
- collaborators could independently develop, or develop with third parties, products that compete directly or indirectly with our product candidates;
- a collaborator with marketing and distribution rights to one or more products may not commit sufficient resources to the marketing and distribution of such product or products;
- disagreements with collaborators, including disagreements over proprietary rights, including trade secrets and intellectual property rights, contract interpretation, or the preferred course of development might cause delays or termination of the research, development or commercialization of product candidates, might lead to additional responsibilities for us with respect to product candidates, or might result in litigation or arbitration, any of which would be time-consuming and expensive;
- collaborators may not properly maintain or defend our intellectual property rights or may use our proprietary information in such a way as to invite litigation that could jeopardize or invalidate our intellectual property or proprietary information or expose us to potential litigation;
- collaborators may infringe the intellectual property rights of third parties, which may expose us to litigation and potential liability; and
- collaborations may be terminated and, if terminated, may result in a need for additional capital to pursue further development or commercialization of the applicable product candidates.

Collaboration agreements may not lead to development or commercialization of product candidates in the most efficient manner or at all. If any future collaborator of ours is involved in a business combination, it could decide to delay, diminish or terminate the development or commercialization of any product candidate licensed to it by us.

Risks Related to Our Intellectual Property

Our success depends on our ability to protect our intellectual property and proprietary technology.

Our success depends in large part on our ability to obtain and maintain patent protection and trade secret protection in the United States and other countries with respect to our proprietary product candidates. If we do not adequately protect our intellectual property rights, competitors may be able to erode, negate or preempt any competitive advantage we may have, which could harm our business and ability to achieve profitability. To protect our proprietary position, we file patent applications in the United States and abroad related to our novel product candidates that are important to our business; we also license or purchase patent applications filed by others. The patent application and approval process is expensive and time-consuming. We may not be able to file and prosecute all necessary or desirable patent applications at a reasonable cost or in a timely manner.

[Table of Contents](#)

Agreements through which we license patent rights may not give us control over patent prosecution or maintenance, so that we may not be able to control which claims or arguments are presented and may not be able to secure, maintain, or successfully enforce necessary or desirable patent protection from those patent rights. We have not had and do not have primary control over patent prosecution and maintenance for certain of the patents and patent applications we license, and therefore cannot guarantee that these patents and applications will be prosecuted or maintained in a manner consistent with the best interests of our business. We cannot be certain that patent prosecution and maintenance activities by our licensors have been or will be conducted in compliance with applicable laws and regulations or will result in valid and enforceable patents.

If the scope of the patent protection we or our licensors obtain is not sufficiently broad, we may not be able to prevent others from developing and commercialize technology and products similar or identical to ours. The degree of patent protection we require to successfully compete in the marketplace may be unavailable or severely limited in some cases and may not adequately protect our rights or permit us to gain or keep any competitive advantage. We cannot provide any assurances that any of our licensed patents have, or that any of our pending licensed patent applications that mature into issued patents will include, claims with a scope sufficient to protect our proprietary platform or otherwise provide any competitive advantage, nor can we assure you that our licenses are or will remain in force. In addition, the laws of foreign countries may not protect our rights to the same extent as the laws of the United States. Furthermore, patents have a limited lifespan. In the United States, the natural expiration of a patent is generally twenty years after it is filed. Various extensions may be available; however, the life of a patent, and the protection it affords, is limited. Given the amount of time required for the development, testing and regulatory review of new product candidates, patents protecting such candidates might expire before or shortly after such candidates are commercialized. As a result, our licensed patent portfolio may not provide us with adequate and continuing patent protection sufficient to exclude others from commercializing products similar to our product candidates. In addition, the patent portfolio licensed to us is, or may be, licensed to third parties, such as outside our field, and such third parties may have certain enforcement rights. Thus, patents licensed to us could be put at risk of being invalidated or interpreted narrowly in litigation filed by or against another licensee or in administrative proceedings brought by or against another licensee in response to such litigation or for other reasons.

Even if they are unchallenged, our licensed patents and pending patent applications, if issued, may not provide us with any meaningful protection or prevent competitors from designing around our patent claims to circumvent our licensed patents by developing similar or alternative technologies or therapeutics in a non-infringing manner. For example, a third party may develop a competitive therapy that provides benefits similar to one or more of our product candidates but that uses a vector or an expression construct that falls outside the scope of our patent protection or license rights. If the patent protection provided by the patents and patent applications we hold or pursue with respect to our product candidates is not sufficiently broad to impede such competition, our ability to successfully commercialize our product candidates could be negatively affected, which would harm our business. Although currently all of our patents and patent applications are in-licensed, similar risks would apply to any patents or patent applications that we may own or in-license in the future.

We, or any future partners, collaborators, or licensees, may fail to identify patentable aspects of inventions made in the course of development and commercialization activities before it is too late to obtain patent protection on them. Therefore, we may miss potential opportunities to strengthen our patent position.

It is possible that defects of form in the preparation or filing of our patents or patent applications may exist, or may arise in the future, for example with respect to proper priority claims, inventorship, claim scope, or requests for patent term adjustments. If we or our partners, collaborators, licensees, or licensors, whether current or future, fail to establish, maintain or protect such patents and other intellectual property rights, such rights may be reduced or eliminated. If our partners, collaborators, licensees, or licensors, are not fully cooperative or disagree with us as to the prosecution, maintenance or enforcement of any patent rights, such patent rights could be compromised. If there are material defects in the form, preparation, prosecution, or enforcement of our patents or patent applications, such patents may be invalid and/or unenforceable, and such applications may never result in valid, enforceable patents. Any of these outcomes could impair our ability to prevent competition from third parties, which may have an adverse impact on our business.

The patent position of biotechnology and pharmaceutical companies generally is highly uncertain. No consistent policy regarding the breadth of claims allowed in biotechnology and pharmaceutical patents has emerged

[Table of Contents](#)

to date in the United States or in many foreign jurisdictions. In addition, the determination of patent rights with respect to pharmaceutical compounds commonly involves complex legal and factual questions, which has in recent years been the subject of much litigation. As a result, the issuance, scope, validity, enforceability and commercial value of our patent rights are highly uncertain.

Pending patent applications cannot be enforced against third parties practicing the technology claimed in such applications unless and until a patent issues from such applications. Assuming the other requirements for patentability are met, currently, the first to file a patent application is generally entitled to the patent. However, prior to March 16, 2013, in the United States, the first to invent was entitled to the patent. Publications of discoveries in the scientific literature often lag behind the actual discoveries, and patent applications in the United States and other jurisdictions are not published until 18 months after filing, or in some cases not at all. Therefore, we cannot be certain that we were the first to make the inventions claimed in our patents or pending patent applications, or that we were the first to file for patent protection of such inventions. Similarly, we cannot be certain that parties from whom we do or may license or purchase patent rights were the first to make relevant claimed inventions, or were the first to file for patent protection for them. If third parties have filed patent applications on inventions claimed in our patents or applications on or before March 15, 2013, an interference proceeding in the United States can be initiated by such third parties to determine who was the first to invent any of the subject matter covered by the patent claims of our applications. If third parties have filed such applications after March 15, 2013, a derivation proceeding in the United States can be initiated by such third parties to determine whether our invention was derived from theirs.

Moreover, because the issuance of a patent is not conclusive as to its inventorship, scope, validity or enforceability, our patents or pending patent applications may be challenged in the courts or patent offices in the United States and abroad. There is no assurance that all of the potentially relevant prior art relating to our patents and patent applications has been found. If such prior art exists, it may be used to invalidate a patent, or may prevent a patent from issuing from a pending patent application. For example, such patent filings may be subject to a third-party preissuance submission of prior art to the U.S. Patent and Trademark Office, or USPTO, to other patent offices around the world. Alternately or additionally, we may become involved in post-grant review procedures, oppositions, derivations, proceedings, reexaminations, *inter partes* review or interference proceedings, in the United States or elsewhere, challenging patents or patent applications in which we have rights, including patents on which we rely to protect our business. An adverse determination in any such challenges may result in loss of exclusivity or in patent claims being narrowed, invalidated or held unenforceable, in whole or in part, which could limit our ability to stop others from using or commercializing similar or identical technology and products, or limit the duration of the patent protection of our technology and products. In addition, given the amount of time required for the development, testing and regulatory review of new product candidates, patents protecting such candidates might expire before or shortly after such candidates are commercialized.

Pending and future patent applications may not result in patents being issued that protect our business, in whole or in part, or which effectively prevent others from commercializing competitive products. Changes in either the patent laws or interpretation of the patent laws in the United States and other countries may diminish the value of our patents or narrow the scope of our patent protection. In addition, the laws of foreign countries may not protect our rights to the same extent or in the same manner as the laws of the United States. For example, patent laws in various jurisdictions, including significant commercial markets such as Europe, restrict the patentability of methods of treatment of the human body more than United States law does.

The patent application process is subject to numerous risks and uncertainties, and there can be no assurance that we or any of our future development partners will be successful in protecting our product candidates by obtaining and defending patents. These risks and uncertainties include the following:

- the USPTO and various foreign governmental patent agencies require compliance with a number of procedural, documentary, fee payment and other provisions during the patent process. There are situations in which noncompliance can result in abandonment or lapse of a patent or patent application, resulting in partial or complete loss of patent rights in the relevant jurisdiction. In such an event, competitors might be able to enter the market earlier than would otherwise have been the case;
- patent applications may not result in any patents being issued;
- patents that may be issued or in-licensed may be challenged, invalidated, modified, revoked, circumvented, found to be unenforceable or otherwise may not provide any competitive advantage;

[Table of Contents](#)

- our competitors, many of whom have substantially greater resources and many of whom have made significant investments in competing technologies, may seek or may have already obtained patents that will limit, interfere with or eliminate our ability to make, use, and sell our potential product candidates;
- there may be significant pressure on the U.S. government and international governmental bodies to limit the scope of patent protection both inside and outside the United States for disease treatments that prove successful, as a matter of public policy regarding worldwide health concerns; and
- countries other than the United States may have patent laws less favorable to patentees than those upheld by U.S. courts, allowing foreign competitors a better opportunity to create, develop and market competing product candidates.

Issued patents that we have or may obtain or license may not provide us with any meaningful protection, prevent competitors from competing with us or otherwise provide us with any competitive advantage. Our competitors may be able to circumvent our patents by developing similar or alternative technologies or products in a non-infringing manner. Our competitors may also seek approval to market their own products similar to or otherwise competitive with our products. Alternatively, our competitors may seek to market generic versions of any approved products by submitting ANDAs to the FDA in which they claim that patents owned or licensed by us are invalid, unenforceable or not infringed. In these circumstances, we may need to defend or assert our patents, or both, including by filing lawsuits alleging patent infringement. In any of these types of proceedings, a court or other agency with jurisdiction may find our patents invalid or unenforceable, or that our competitors are competing in a non-infringing manner. Thus, even if we have valid and enforceable patents, these patents still may not provide protection against competing products or processes sufficient to achieve our business objectives.

Pursuant to the terms of some of our license agreements with third parties, some of our third party licensors have the right, but not the obligation in certain circumstances to control enforcement of our licensed patents or defense of any claims asserting the invalidity of these patents. Even if we are permitted to pursue such enforcement or defense, we will require the cooperation of our licensors, and cannot guarantee that we would receive it and on what terms. We cannot be certain that our licensors will allocate sufficient resources or prioritize their or our enforcement of such patents or defense of such claims to protect our interests in the licensed patents. If we cannot obtain patent protection, or enforce existing or future patents against third parties, our competitive position and our financial condition could suffer.

In addition, we rely on the protection of our trade secrets and proprietary know-how. Although we have taken steps to protect our trade secrets and unpatented know-how, including entering into confidentiality agreements with third parties, and confidential information and inventions agreements with employees, consultants and advisors, we cannot provide any assurances that all such agreements have been duly executed, and third parties may still obtain this information or may come upon this or similar information independently. Additionally, if the steps taken to maintain our trade secrets are deemed inadequate, we may have insufficient recourse against third parties for misappropriating its trade secrets. If any of these events occurs or if we otherwise lose protection for our trade secrets or proprietary know-how, our business may be harmed.

It is difficult and costly to protect our intellectual property and our proprietary technologies, and we may not be able to ensure their protection.

Our commercial success will depend in part on obtaining and maintaining patent protection and trade secret protection for the use, formulation and structure of our products and product candidates, the methods used to manufacture them, the related therapeutic targets and associated methods of treatment as well as on successfully defending these patents against potential third-party challenges. Our ability to protect our products and product candidates from unauthorized making, using, selling, offering to sell or importing by third parties is dependent on the extent to which we have rights under valid and enforceable patents that cover these activities.

The patent positions of pharmaceutical, biotechnology and other life sciences companies can be highly uncertain and involve complex legal and factual questions for which important legal principles remain unresolved. Changes in either the patent laws or in interpretations of patent laws in the United States and other countries may diminish the value of our intellectual property. Further, the determination that a patent application or patent claim meets all of the requirements for patentability is a subjective determination based on the application of law and jurisprudence. The ultimate determination by the USPTO or by a court or other trier of fact in the United States, or

[Table of Contents](#)

corresponding foreign national patent offices or courts, on whether a claim meets all requirements of patentability cannot be assured. For example, our C5 inhibitor portfolio consists of three families of patent applications that we own directed to C5 inhibitors and related methods of use. Although we have conducted searches for third-party publications, patents and other information that may affect the patentability of claims in our various patent applications and patents, we cannot be certain that all relevant information has been identified. Accordingly, we cannot predict the breadth of claims that may be allowed or enforced in our patents or patent applications, in our licensed patents or patent applications or in third-party patents.

We cannot provide assurances that any of our patent applications will be found to be patentable, including over our own prior art patents, or will issue as patents. Neither can we make assurances as to the scope of any claims that may issue from our pending and future patent applications nor to the outcome of any proceedings by any potential third parties that could challenge the patentability, validity or enforceability of our patents and patent applications in the United States or foreign jurisdictions. Any such challenge, if successful, could limit patent protection for our products and product candidates and/or materially harm our business.

The degree of future protection for our proprietary rights is uncertain because legal means afford only limited protection and may not adequately protect our rights or permit us to gain or keep our competitive advantage. For example:

- we may not be able to generate sufficient data to support full patent applications that protect the entire breadth of developments in one or more of our programs, including our PNH program;
- it is possible that one or more of our pending patent applications will not become an issued patent or, if issued, that the patent(s) will be sufficient to protect our technology, provide us with a basis for commercially viable products or provide us with any competitive advantages;
- if our pending applications issue as patents, they may be challenged by third parties as not infringed, invalid or unenforceable under United States or foreign laws; or
- if issued, the patents under which we hold rights may not be valid or enforceable.

In addition, to the extent that we are unable to obtain and maintain patent protection for one of our products or product candidates or in the event that such patent protection expires, it may no longer be cost-effective to extend our portfolio by pursuing additional development of a product or product candidate for follow-on indications.

We also may rely on trade secrets to protect our technologies or products, especially where we do not believe patent protection is appropriate or obtainable. For example, the patents underlying our proprietary peptide chemistry technology, which we license from third parties on a non-exclusive basis in some cases, expire by 2022. As a result, we anticipate that trade secrets will serve as the primary protection for the know-how behind our proprietary platform. Also, we cannot provide any assurances that any of our licensed patents have claims with a scope sufficient to protect our proprietary platform or otherwise provide any competitive advantage, nor can we assure you that our licenses are or will remain in full force or effect, in which case we would similarly rely on trade secrets. However, trade secrets are difficult to protect. Although we use reasonable efforts to protect our trade secrets, our employees, consultants, contractors, outside scientific collaborators and other advisers may unintentionally or willfully disclose our information to competitors. Enforcing a claim that a third-party entity illegally obtained and is using any of our trade secrets is expensive and time-consuming, and the outcome is unpredictable. In addition, courts outside the United States are sometimes less willing to protect trade secrets. Moreover, our competitors may independently develop equivalent knowledge, methods and know-how. Notably, proprietary technology protected by a trade secret does not preempt the patent of independently developed equivalent technology, even if such equivalent technology is invented subsequent to the technology protected by a trade secret.

Obtaining and maintaining patent protection depends on compliance with various procedural, document submission, fee payment and other requirements imposed by governmental patent agencies, and our patent protection could be reduced or eliminated for non-compliance with these requirements.

Periodic maintenance fees, renewal fees, annuity fees and various other governmental fees on patents and applications are required to be paid to the USPTO and various governmental patent agencies outside of the United States in several stages over the lifetime of the patents and applications. The USPTO and various non-U.S.

[Table of Contents](#)

governmental patent agencies require compliance with a number of procedural, documentary, fee payment and other similar provisions during the patent application process and after a patent has issued. There are situations in which non-compliance can result in abandonment or lapse of the patent or patent application, resulting in partial or complete loss of patent rights in the relevant jurisdiction. Under the terms of some of our licenses, we do not have the ability to maintain or prosecute patents in the portfolio, and must therefore rely on third parties to comply with these requirements.

Patent terms may be inadequate to protect our competitive position on our products for an adequate amount of time.

Given the amount of time required for the development, testing and regulatory review of new product candidates, patents protecting such candidates might expire before or shortly after such candidates are commercialized. We expect to seek extensions of patent terms in the United States and, if available, in other countries where we are prosecuting patents. In the United States, the Drug Price Competition and Patent Term Restoration Act of 1984 permits a patent term extension of up to five years beyond the normal expiration of the patent, which is limited to the approved indication (or any additional indications approved during the period of extension). However, the applicable authorities, including the FDA and the USPTO in the United States, and any equivalent regulatory authority in other countries, may not agree with our assessment of whether such extensions are available, and may refuse to grant extensions to our patents, or may grant more limited extensions than we request. If this occurs, our competitors may be able to take advantage of our investment in development and clinical trials by referencing our clinical and preclinical data and launch their product earlier than might otherwise be the case.

Changes to the patent law in the United States and other jurisdictions could diminish the value of patents in general, thereby impairing our ability to protect our products.

As is the case with other biopharmaceutical companies, our success is heavily dependent on intellectual property, particularly patents. Obtaining and enforcing patents in the biopharmaceutical industry involves both technological and legal complexity and is therefore costly, time consuming and inherently uncertain. Recent patent reform legislation in the United States, including the Leahy-Smith America Invents Act, or the America Invents Act, could increase those uncertainties and costs. The America Invents Act was signed into law on September 16, 2011, and many of the substantive changes became effective on March 16, 2013. The America Invents Act reforms United States patent law in part by changing the U.S. patent system from a “first to invent” system to a “first inventor to file” system, expanding the definition of prior art, and developing a post-grant review system. This legislation changes United States patent law in a way that may weaken our ability to obtain patent protection in the United States for those applications filed after March 16, 2013.

Further, the America Invents Act created new procedures to challenge the validity of issued patents in the United States, including post-grant review and *inter partes* review proceedings, which some third parties have been using to cause the cancellation of selected or all claims of issued patents of competitors. For a patent with an effective filing date of March 16, 2013 or later, a petition for post-grant review can be filed by a third party in a nine month window from issuance of the patent. A petition for *inter partes* review can be filed immediately following the issuance of a patent if the patent has an effective filing date prior to March 16, 2013. A petition for *inter partes* review can be filed after the nine month period for filing a post-grant review petition has expired for a patent with an effective filing date of March 16, 2013 or later. Post-grant review proceedings can be brought on any ground of invalidity, whereas *inter partes* review proceedings can only raise an invalidity challenge based on published prior art and patents. These adversarial actions at the USPTO review patent claims without the presumption of validity afforded to U.S. patents in lawsuits in U.S. federal courts, and use a lower burden of proof than used in litigation in U.S. federal courts. Therefore, it is generally considered easier for a competitor or third party to have a U.S. patent invalidated in a USPTO post-grant review or *inter partes* review proceeding than invalidated in a litigation in a U.S. federal court. If any of our patents are challenged by a third party in such a USPTO proceeding, there is no guarantee that we or our licensors or collaborators will be successful in defending the patent, which would result in a loss of the challenged patent right to us.

In addition, recent court rulings in cases such as *Association for Molecular Pathology v. Myriad Genetics, Inc., BRCA1- & BRCA2-Based Hereditary Cancer Test Patent Litigation, and Promega Corp. v. Life Technologies Corp.* have narrowed the scope of patent protection available in certain circumstances and weakened

[Table of Contents](#)

the rights of patent owners in certain situations. In addition to increasing uncertainty with regard to our ability to obtain patents in the future, this combination of events has created uncertainty with respect to the value of patents once obtained. Depending on future actions by the U.S. Congress, the U.S. courts, the USPTO and the relevant law-making bodies in other countries, the laws and regulations governing patents could change in unpredictable ways that would weaken our ability to obtain new patents or to enforce our existing patents and patents that we might obtain in the future.

We may not be able to enforce our intellectual property rights throughout the world.

Filing, prosecuting, enforcing and defending patents on our product candidates in all countries throughout the world would be prohibitively expensive, and our intellectual property rights in some countries outside the United States can be less extensive than those in the United States. The requirements for patentability may differ in certain countries, particularly in developing countries; thus, even in countries where we do pursue patent protection, there can be no assurance that any patents will issue with claims that cover our products.

Moreover, our ability to protect and enforce our intellectual property rights may be adversely affected by unforeseen changes in foreign intellectual property laws. Additionally, laws of some countries outside of the United States and Europe do not afford intellectual property protection to the same extent as the laws of the United States and Europe. Many companies have encountered significant problems in protecting and defending intellectual property rights in certain foreign jurisdictions. The legal systems of some countries, including India, China and other developing countries, do not favor the enforcement of patents and other intellectual property rights. This could make it difficult for us to stop the infringement of our patents or the misappropriation of our other intellectual property rights. For example, many foreign countries have compulsory licensing laws under which a patent owner must grant licenses to third parties. Consequently, we may not be able to prevent third parties from practicing our inventions in certain countries outside the United States and Europe. Competitors may use our technologies in jurisdictions where we have not obtained patent protection to develop and market their own products and, further, may export otherwise infringing products to territories where we have patent protection, if our ability to enforce our patents to stop infringing activities is inadequate. These products may compete with our products, and our patents or other intellectual property rights may not be effective or sufficient to prevent them from competing.

Agreements through which we license patent rights may not give us sufficient rights to permit us to pursue enforcement of our licensed patents or defense of any claims asserting the invalidity of these patents (or control of enforcement or defense) of such patent rights in all relevant jurisdictions as requirements may vary.

Proceedings to enforce our patent rights in foreign jurisdictions, whether or not successful, could result in substantial costs and divert our efforts and resources from other aspects of our business. Moreover, such proceedings could put our patents at risk of being invalidated or interpreted narrowly and our patent applications at risk of not issuing and could provoke third parties to assert claims against us. We may not prevail in any lawsuits that we initiate and the damages or other remedies awarded, if any, may not be commercially meaningful. Furthermore, while we intend to protect our intellectual property rights in major markets for our products, we cannot ensure that we will be able to initiate or maintain similar efforts in all jurisdictions in which we may wish to market our products. Accordingly, our efforts to protect our intellectual property rights in such countries may be inadequate.

Others may claim an ownership interest in our intellectual property which could expose it to litigation and have a significant adverse effect on its prospects.

A third party may claim an ownership interest in one or more of our or our licensors' patents or other proprietary or intellectual property rights. A third party could bring legal actions against us and seek monetary damages and/or enjoin clinical testing, manufacturing and marketing of the affected product or products. While we are presently unaware of any claims or assertions by third-parties with respect to our patents or other intellectual property, we cannot guarantee that a third-party will not assert a claim or an interest in any of such patents or intellectual property. If we become involved in any litigation, it could consume a substantial portion of our resources, and cause a significant diversion of effort by our technical and management personnel. If any of these actions are successful, in addition to any potential liability for damages, we could be required to obtain a license to continue to manufacture or market the affected product, in which case we may be required to pay substantial royalties or grant cross-licenses to our patents. We cannot, however, assure you that any such license will be

[Table of Contents](#)

available on acceptable terms, if at all. Ultimately, we could be prevented from commercializing a product, or be forced to cease some aspect of our business operations as a result of claims of patent infringement or violation of other intellectual property rights. Further, the outcome of intellectual property litigation is subject to uncertainties that cannot be adequately quantified in advance, including the demeanor and credibility of witnesses and the identity of any adverse party. This is especially true in intellectual property cases that may turn on the testimony of experts as to technical facts upon which experts may reasonably disagree.

If we are sued for infringing intellectual property rights of third parties, such litigation could be costly and time consuming and could prevent or delay us from developing or commercializing our product candidates.

Our commercial success depends, in part, on our ability to develop, manufacture, market and sell our product candidates without infringing the intellectual property and other proprietary rights of third parties. Third parties may have U.S. and non-U.S. issued patents and pending patent applications relating to compounds, methods of manufacturing compounds and/or methods of use for the treatment of the disease indications for which we are developing our product candidates or relating to the use of complement inhibition that may cover our product candidates or approach to complement inhibition. If any third-party patents or patent applications are found to cover our product candidates or their methods of use or manufacture or our approach to complement inhibition, we may not be free to manufacture or market our product candidates as planned without obtaining a license, which may not be available on commercially reasonable terms, or at all.

There is a substantial amount of intellectual property litigation in the biotechnology and pharmaceutical industries, and we may become party to, or threatened with, litigation or other adversarial proceedings regarding intellectual property rights with respect to our products candidates, including interference and post-grant proceedings before the USPTO. There may be third-party patents or patent applications with claims to materials, formulations, methods of manufacture or methods for treatment related to the composition, use or manufacture of our product candidates. We cannot guarantee that any of our patent searches or analyses including, but not limited to, the identification of relevant patents, the scope of patent claims or the expiration of relevant patents are complete or thorough, nor can we be certain that we have identified each and every patent and pending application in the United States and abroad that is relevant to or necessary for the commercialization of our product candidates in any jurisdiction. Because patent applications can take many years to issue, there may be currently pending patent applications which may later result in issued patents that our product candidates may be accused of infringing. In addition, third parties may obtain patents in the future and claim that use of our technologies infringes upon these patents. Accordingly, third parties may assert infringement claims against us based intellectual property rights that exist now or arise in the future. The outcome of intellectual property litigation is subject to uncertainties that cannot be adequately quantified in advance. The pharmaceutical and biotechnology industries have produced a significant number of patents, and it may not always be clear to industry participants, including us, which patents cover various types of products or methods of use or manufacture. The scope of protection afforded by a patent is subject to interpretation by the courts, and the interpretation is not always uniform. If we were sued for patent infringement, we would need to demonstrate that our product candidates, products or methods either do not infringe the patent claims of the relevant patent or that the patent claims are invalid or unenforceable, and we may not be able to do this. Proving invalidity is difficult. For example, in the United States, proving invalidity requires a showing of clear and convincing evidence to overcome the presumption of validity enjoyed by issued patents. Even if we are successful in these proceedings, we may incur substantial costs and the time and attention of our management and scientific personnel could be diverted in pursuing these proceedings, which could significantly harm our business and operating results. In addition, we may not have sufficient resources to bring these actions to a successful conclusion.

If we are found to infringe a third party's intellectual property rights, we could be forced, including by court order, to cease developing, manufacturing or commercializing the infringing product candidate or product. Alternatively, we may be required to obtain a license from such third party in order to use the infringing technology and continue developing, manufacturing or marketing the infringing product candidate or product. However, we may not be able to obtain any required license on commercially reasonable terms or at all. Even if we were able to obtain a license, it could be non-exclusive, thereby giving our competitors access to the same technologies licensed to us; alternatively or additionally it could include terms that impede or destroy our ability to compete successfully in the commercial marketplace. In addition, we could be found liable for monetary damages, including treble damages and attorneys' fees if we are found to have willfully infringed a patent. A finding of infringement could

[Table of Contents](#)

prevent us from commercializing our product candidates or force us to cease some of our business operations, which could harm our business. Claims that we have misappropriated the confidential information or trade secrets of third parties could have a similar negative impact on our business.

We may be subject to claims by third parties asserting that our employees or we have misappropriated their intellectual property, or claiming ownership of what we regard as our own intellectual property.

Many of our current and former employees and our licensors' current and former employees, including our senior management, were previously employed at universities or at other biotechnology or pharmaceutical companies, including some which may be competitors or potential competitors. Some of these employees, including each member of our senior management, executed proprietary rights, non-disclosure and non-competition agreements, or similar agreements, in connection with such previous employment. Although we try to ensure that our employees do not use the proprietary information or know-how of others in their work for us, we may be subject to claims that we or these employees have used or disclosed intellectual property, including trade secrets or other proprietary information, of any such third party. Litigation may be necessary to defend against such claims. If we fail in defending any such claims, in addition to paying monetary damages, we may lose valuable intellectual property rights or personnel or sustain damages. Such intellectual property rights could be awarded to a third party, and we could be required to obtain a license from such third party to commercialize our technology or products. Such a license may not be available on commercially reasonable terms or at all. Even if we are successful in defending against such claims, litigation could result in substantial costs and be a distraction to management.

In addition, while we typically require our employees, consultants and contractors who may be involved in the development of intellectual property to execute agreements assigning such intellectual property to us, we may be unsuccessful in executing such an agreement with each party who in fact develops intellectual property that we regard as our own, which may result in claims by or against us related to the ownership of such intellectual property. If we fail in prosecuting or defending any such claims, in addition to paying monetary damages, we may lose valuable intellectual property rights. Even if we are successful in prosecuting or defending against such claims, litigation could result in substantial costs and be a distraction to our senior management and scientific personnel.

We may become involved in lawsuits to protect or enforce our patents or other intellectual property, which could be expensive, time consuming and unsuccessful.

Competitors may infringe our patents, trademarks, copyrights or other intellectual property. To counter infringement or unauthorized use, we may be required to file infringement claims, which can be expensive and time consuming and divert the time and attention of our management and scientific personnel. Any claims we assert against perceived infringers could provoke these parties to assert counterclaims against us alleging that we infringe their patents, in addition to counterclaims asserting that our patents are invalid or unenforceable, or both. In any patent infringement proceeding, there is a risk that a court will decide that a patent of ours is invalid or unenforceable, in whole or in part, and that we do not have the right to stop the other party from using the invention at issue. There is also a risk that, even if the validity of such patents is upheld, the court will construe the patent's claims narrowly or decide that we do not have the right to stop the other party from using the invention at issue on the grounds that our patent claims do not cover the invention. An adverse outcome in a litigation or proceeding involving one or more of our patents could limit our ability to assert those patents against those parties or other competitors, and may curtail or preclude our ability to exclude third parties from making and selling similar or competitive products. Similarly, if we assert trademark infringement claims, a court may determine that the marks we have asserted are invalid or unenforceable, or that the party against whom we have asserted trademark infringement has superior rights to the marks in question. In this case, we could ultimately be forced to cease use of such trademarks.

Even if we establish infringement, the court may decide not to grant an injunction against further infringing activity and instead award only monetary damages, which may or may not be an adequate remedy. Furthermore, because of the substantial amount of discovery required in connection with intellectual property litigation, there is a risk that some of our confidential information could be compromised by disclosure during litigation. There could also be public announcements of the results of hearings, motions or other interim proceedings or developments. If securities analysts or investors perceive these results to be negative, it could adversely affect the price of shares of our common stock. Moreover, there can be no assurance that we will have sufficient financial or other resources to

[Table of Contents](#)

file and pursue such infringement claims, which typically last for years before they are concluded. Even if we ultimately prevail in such claims, the monetary cost of such litigation and the diversion of the attention of our management and scientific personnel could outweigh any benefit we receive as a result of the proceedings.

Additionally, for certain of our in-licensed patent rights, we do not have the right to bring suit for infringement and must rely on third parties to enforce these rights for us. If we cannot or choose not to take action against those we believe infringe our intellectual property rights, we may have difficulty competing in certain markets where such potential infringers conduct their business, and our commercialization efforts may suffer as a result.

If we fail to comply with our obligations under our existing and any future intellectual property licenses with third parties, we could lose license rights that are important to our business.

We are a party to a collaboration and license agreement with Merck Sharp & Dohme Corp., under which we license patent rights relating to peptides that modulate the activity of a Merck-designated non-complement cardiovascular product candidate. We are party to several other license agreements, under which we license patent rights related to our proprietary technology and other product candidates. We may enter into additional license agreements in the future. Our license agreement with Merck imposes, and we expect that future license agreements will impose, various diligence, milestone payment, royalty, insurance and other obligations on us. If we fail to comply with our obligations under these licenses, our licensors may have the right to terminate these license agreements, in which event we might not be able to market any product that is covered by these agreements, or our licensors may convert the license to a non-exclusive license, which could negatively impact the value of the product candidate being developed under the license agreement. Termination of these license agreements or reduction or elimination of our licensed rights may also result in our having to negotiate new or reinstated licenses with less favorable terms.

If we are unable to protect the confidentiality of our trade secrets, the value of our technology could be negatively impacted and our business would be harmed.

In addition to the protection afforded by patents, we also rely on trade secret protection for certain aspects of our intellectual property. For example, the patents underlying our proprietary peptide chemistry technology expire by 2022. As a result, we anticipate that we will rely on trade secrets as the primary protection for the know-how behind our proprietary platform. We seek to protect these trade secrets, in part, by entering into non-disclosure and confidentiality agreements with parties who have access to them, such as our employees, consultants, independent contractors, advisors, contract manufacturers, suppliers and other third parties. We also enter into confidentiality and invention or patent assignment agreements with employees and certain consultants. Any party with whom we have executed such an agreement may breach that agreement and disclose our proprietary information, including our trade secrets, and we may not be able to obtain adequate remedies for such breaches. Enforcing a claim that a party illegally disclosed or misappropriated a trade secret is difficult, expensive and time-consuming, and the outcome is unpredictable. Additionally, if the steps taken to maintain our trade secrets are deemed inadequate, we may have insufficient recourse against third parties for misappropriating the trade secret. Further, if any of our trade secrets were to be lawfully obtained or independently developed by a competitor, we would have no right to prevent such third party, or those to whom they communicate such technology or information, from using that technology or information to compete with us. If any of our trade secrets were to be disclosed to or independently developed by a competitor, our business and competitive position could be harmed.

If our trademarks and trade names are not adequately protected, then we may not be able to build name recognition in our marks of interest and our business may be adversely affected.

Our trademarks or trade names may be challenged, infringed, circumvented or declared generic or determined to be infringing on other marks. We rely on both registration and common law protection for our trademarks. We may not be able to protect our rights to these trademarks and trade names or may be forced to stop using these names, which we need for name recognition by potential partners or customers in our markets of interest. During trademark registration proceedings, we may receive rejections. Although we would be given an opportunity to respond to those rejections, we may be unable to overcome such rejections. In addition, in the USPTO and in comparable agencies in many foreign jurisdictions, third parties are given an opportunity to oppose

[Table of Contents](#)

pending trademark applications and to seek to cancel registered trademarks. Opposition or cancellation proceedings may be filed against our trademarks, and our trademarks may not survive such proceedings. If we are unable to establish name recognition based on our trademarks and trade names, we may not be able to compete effectively and our business may be adversely affected.

Risks Related to Employee Matters and Managing Growth

We only have a limited number of employees to manage and operate our business.

As of September 30, 2016, we had 44 full-time or part-time employees. Our focus on the development of RA101495 requires us to optimize cash utilization and to manage and operate our business in a highly efficient manner. We cannot assure you that we will be able to hire and/or retain adequate staffing levels to develop RA101495 or run our operations and/or to accomplish all of the objectives that we otherwise would seek to accomplish.

Cyber-attacks or other failures in telecommunications or information technology systems could result in information theft, data corruption and significant disruption of our business operations.

We utilize information technology, or IT, systems and networks to process, transmit and store electronic information in connection with our business activities. As use of digital technologies has increased, cyber incidents, including deliberate attacks and attempts to gain unauthorized access to computer systems and networks, have increased in frequency and sophistication. These threats pose a risk to the security of our systems and networks, the confidentiality and the availability and integrity of our data. There can be no assurance that we will be successful in preventing cyber-attacks or successfully mitigating their effects. Similarly, there can be no assurance that our collaborators, CROs, third-party logistics providers, distributors and other contractors and consultants will be successful in protecting our clinical and other data that is stored on their systems. Any cyber-attack or destruction or loss of data could have a material adverse effect on our business and prospects. In addition, we may suffer reputational harm or face litigation or adverse regulatory action as a result of cyber-attacks or other data security breaches and may incur significant additional expense to implement further data protection measures.

We depend heavily on our executive officers, directors, and principal consultants and the loss of their services would materially harm our business.

Our success depends, and will likely continue to depend, upon our ability to hire, retain the services of our current executive officers, directors, principal consultants and others. In addition, we have established relationships with universities and research institutions which have historically provided, and continue to provide, us with access to research laboratories, clinical trials, facilities and patients. Our ability to compete in the biotechnology and pharmaceuticals industries depends upon our ability to attract and retain highly qualified managerial, scientific and medical personnel.

Our industry has experienced a high rate of turnover of management personnel in recent years. Any of our personnel may terminate their employment at will. If we lose one or more of our executive officers or other key employees, our ability to implement our business strategy successfully could be seriously harmed. Furthermore, replacing executive officers or other key employees may be difficult and may take an extended period of time because of the limited number of individuals in our industry with the breadth of skills and experience required to develop, gain marketing approval of and commercialize products successfully.

Competition to hire from this limited pool is intense, and we may be unable to hire, train, retain or motivate these additional key employees on acceptable terms given the competition among numerous pharmaceutical and biotechnology companies for similar personnel. We also experience competition for the hiring of scientific and clinical personnel from universities and research institutions.

We rely on consultants and advisors, including scientific and clinical advisors, to assist us in formulating our research and development and commercialization strategy. Our consultants and advisors may be employed by other entities and may have commitments under consulting or advisory contracts with those entities that may limit

[Table of Contents](#)

their availability to us. If we are unable to continue to attract and retain highly qualified personnel, our ability to develop and commercialize our product candidates will be limited.

Our employees, independent contractors, consultants, collaborators and contract research organizations may engage in misconduct or other improper activities, including non-compliance with regulatory standards and requirements, which could cause significant liability for us and harm our reputation.

We are exposed to the risk that our employees, independent contractors, consultants, collaborators and contract research organizations may engage in fraudulent conduct or other illegal activity. Misconduct by those parties could include intentional, reckless and/or negligent conduct or disclosure of unauthorized activities to us that violates: (1) FDA regulations or similar regulations of comparable non-U.S. regulatory authorities, including those laws requiring the reporting of true, complete and accurate information to such authorities, (2) manufacturing standards, (3) federal and state healthcare fraud and abuse laws and regulations and similar laws and regulations established and enforced by comparable non-U.S. regulatory authorities, and (4) laws that require the reporting of financial information or data accurately. Activities subject to these laws also involve the improper use of information obtained in the course of clinical trials, which could result in regulatory sanctions and serious harm to our reputation. It is not always possible to identify and deter misconduct, and the precautions we take to detect and prevent this activity may not be effective in controlling unknown or unmanaged risks or losses or in protecting us from governmental investigations or other actions or lawsuits stemming from a failure to be in compliance with such laws, standards or regulations. If any such actions are instituted against us, and we are not successful in defending ourselves or asserting our rights, those actions could have a significant impact on our business and results of operations, including the imposition of civil, criminal and administrative penalties, damages, monetary fines, possible exclusion from participation in Medicare, Medicaid and other federal healthcare programs, contractual damages, reputational harm, diminished profits and future earnings, and curtailment of our operations, any of which could have a material adverse effect on our ability to operate our business and our results of operations.

We expect to expand our organization, and as a result, we may encounter difficulties in managing our growth, which could disrupt our operations.

We expect to experience significant growth in the number of our employees and the scope of our operations, particularly in the areas of drug manufacturing, regulatory affairs and sales, marketing and distribution, as well as to support our public company operations. To manage these growth activities, we must continue to implement and improve our managerial, operational and financial systems, expand our facilities and continue to recruit and train additional qualified personnel. Our management may need to devote a significant amount of its attention to managing these growth activities. Moreover, our expected growth could require us to relocate to a different geographic area of the country. Due to our limited financial resources and the limited experience of our management team in managing a company with such anticipated growth, we may not be able to effectively manage the expansion or relocation of our operations, retain key employees, or identify, recruit and train additional qualified personnel. Our inability to manage the expansion or relocation of our operations effectively may result in weaknesses in our infrastructure, give rise to operational mistakes, loss of business opportunities, loss of employees and reduced productivity among remaining employees. Our expected growth could also require significant capital expenditures and may divert financial resources from other projects, such as the development of additional product candidates. If we are unable to effectively manage our expected growth, our expenses may increase more than expected, our ability to generate revenues could be reduced and we may not be able to implement our business strategy, including the successful commercialization of our product candidates.

Risks Related to Our Common Stock

An active trading market for our common stock may not be sustained.

In October 2016, we closed our initial public offering. Prior to this offering, there was no public market for our common stock. Although we have completed our initial public offering and shares of our common stock are listed and trading on The NASDAQ Global Market, an active trading market for our shares may not be sustained. If an active market for our common stock does not continue, it may be difficult for our stockholders to sell their shares without depressing the market price for the shares or sell their shares at or above the prices at which they acquired their shares or sell their shares at the time they would like to sell. Any inactive trading market for our common stock

[Table of Contents](#)

may also impair our ability to raise capital to continue to fund our operations by selling shares and may impair our ability to acquire other companies or technologies by using our shares as consideration.

The price of our common stock may be volatile and fluctuate substantially.

Our stock price is likely to be highly volatile. The stock market in general and the market for smaller pharmaceutical and biotechnology companies in particular have experienced extreme volatility that has often been unrelated to the operating performance of particular companies. The market price for our common stock may be influenced by many factors, including:

- the success of existing or new competitive products or technologies;
- regulatory actions with respect to our product candidates or our competitors' products and product candidates;
- announcements by us or our competitors of significant acquisitions, strategic partnerships, joint ventures, collaborations or capital commitments;
- the timing and results of clinical trials of RA101495 and any other product candidates;
- commencement or termination of collaborations for our development programs;
- failure or discontinuation of any of our development programs;
- results of clinical trials of product candidates of our competitors;
- regulatory or legal developments in the United States and other countries;
- developments or disputes concerning patent applications, issued patents or other proprietary rights;
- the recruitment or departure of key personnel;
- the level of expenses related to any of our product candidates or clinical development programs;
- the results of our efforts to develop additional product candidates or products;
- actual or anticipated changes in estimates as to financial results or development timelines;
- announcement or expectation of additional financing efforts;
- sales of our common stock by us, our insiders or other stockholders;
- variations in our financial results or those of companies that are perceived to be similar to us;
- changes in estimates or recommendations by securities analysts, if any, that cover our stock;
- changes in the structure of healthcare payment systems;
- market conditions in the pharmaceutical and biotechnology sectors;
- general economic, industry and market conditions; and
- the other factors described in this "Risk Factors" section.

In the past, securities class action litigation has often been brought against a company following a decline in the market price of its securities. This risk is especially relevant for biopharmaceutical companies, which have experienced significant stock price volatility in recent years. The results of any legal proceedings are inherently uncertain and, regardless of the ultimate outcome or the merits, require substantial time and other resources to defend. Accordingly, any litigation that we could face may result in substantial costs to us, divert management's attention and resources from our company as well as harm our reputation with analysts and investors, which could substantially harm our business, financial condition and results of operations.

We have broad discretion in the use of our cash and cash equivalents and may not use them effectively.

Our management has broad discretion to use our cash and cash equivalents to fund our operations and could spend these funds in ways that do not improve our results of operations or enhance the value of our common stock. The failure by our management to apply these funds effectively could result in financial losses that could have a material adverse effect on our business, cause the price of our common stock to decline and delay the development of our product candidates. Pending our use to fund operations, we may invest our cash and cash equivalents in a manner that does not produce income or that loses value.

We are an "emerging growth company," and the reduced disclosure requirements applicable to emerging growth companies may make our common stock less attractive to investors.

We are an "emerging growth company," as defined in the JOBS Act, and may remain an emerging growth company for up to five years following our initial public offering. For so long as we remain an emerging growth

[Table of Contents](#)

company, we are permitted and plan to rely on exemptions from certain disclosure requirements that are applicable to other public companies that are not emerging growth companies. These exemptions include not being required to comply with the auditor attestation requirements of Section 404 of the Sarbanes-Oxley Act of 2002, or SOX Section 404, not being required to comply with any requirement that may be adopted by the Public Company Accounting Oversight Board regarding mandatory audit firm rotation or a supplement to the auditor's report providing additional information about the audit and the financial statements, reduced disclosure obligations regarding executive compensation and exemptions from the requirements of holding a nonbinding advisory vote on executive compensation and stockholder approval of any golden parachute payments not previously approved. We cannot predict whether investors will find our common stock less attractive if we rely on these exemptions. If some investors find our common stock less attractive as a result, there may be a less active trading market for our common stock and our stock price may be more volatile.

In addition, the JOBS Act provides that an emerging growth company can take advantage of an extended transition period for complying with new or revised accounting standards. This allows an emerging growth company to delay the adoption of certain accounting standards until those standards would otherwise apply to private companies. We have irrevocably elected not to avail ourselves of this exemption from new or revised accounting standards and, therefore, we will be subject to the same new or revised accounting standards as other public companies that are not emerging growth companies.

We will incur increased costs as a result of operating as a public company, and our management will be required to devote substantial time to new compliance initiatives and corporate governance practices.

As a public company, and particularly after we are no longer an "emerging growth company," we will incur significant legal, accounting and other expenses that we did not incur as a private company, which we anticipate could amount to between \$1.0 million and \$2.0 million annually. The Sarbanes-Oxley Act of 2002, the Dodd-Frank Wall Street Reform and Consumer Protection Act, the listing requirements of the NASDAQ Global Market and other applicable securities rules and regulations impose various requirements on public companies, including establishment and maintenance of effective disclosure and financial controls and corporate governance practices. We expect that we will need to hire additional accounting, finance and other personnel in connection with our becoming, and our efforts to comply with the requirements of being, a public company and our management and other personnel will need to devote a substantial amount of time towards maintaining compliance with these requirements. These requirements will increase our legal and financial compliance costs and will make some activities more time-consuming and costly. For example, we expect that the rules and regulations applicable to us as a public company may make it more difficult and more expensive for us to obtain director and officer liability insurance, which could make it more difficult for us to attract and retain qualified members of our board of directors. We are currently evaluating these rules and regulations, and cannot predict or estimate the amount of additional costs we may incur or the timing of such costs. These rules and regulations are often subject to varying interpretations, in many cases due to their lack of specificity, and, as a result, their application in practice may evolve over time as new guidance is provided by regulatory and governing bodies. This could result in continuing uncertainty regarding compliance matters and higher costs necessitated by ongoing revisions to disclosure and governance practices.

We are evaluating these rules and regulations, and cannot predict or estimate the amount of additional costs we may incur or the timing of such costs. These rules and regulations are often subject to varying interpretations, in many cases due to their lack of specificity, and, as a result, their application in practice may evolve over time as new guidance is provided by regulatory and governing bodies which could result in continuing uncertainty regarding compliance matters and higher costs necessitated by ongoing revisions to disclosure and governance practices.

Pursuant to SOX Section 404 we will be required to furnish a report by our management on our internal control over financial reporting beginning with our second filing of an Annual Report on Form 10-K with the SEC after we become a public company. However, while we remain an emerging growth company, we will not be required to include an attestation report on internal control over financial reporting issued by our independent registered public accounting firm. To achieve compliance with SOX Section 404 within the prescribed period, we will be engaged in a process to document and evaluate our internal control over financial reporting, which is both costly and challenging. In this regard, we will need to continue to dedicate internal resources, potentially engage outside consultants and adopt a detailed work plan to assess and document the adequacy of internal control over

[Table of Contents](#)

financial reporting, continue steps to improve control processes as appropriate, validate through testing that controls are functioning as documented and implement a continuous reporting and improvement process for internal control over financial reporting. Despite our efforts, there is a risk that we will not be able to conclude, within the prescribed timeframe or at all, that our internal control over financial reporting is effective. If we identify one or more material weaknesses, it could result in an adverse reaction in the financial markets due to a loss of confidence in the reliability of our financial statements.

A significant portion of our total outstanding shares is restricted from immediate resale but may be sold into the market in the near future, which could cause the market price of our common stock to decline significantly, even if our business is doing well.

Sales of a substantial number of shares of our common stock in the public market could occur at any time. These sales, or the perception in the market that the holders of a large number of shares of common stock intend to sell shares, could reduce the market price of our common stock. Following our initial public offering in October 2016, including the closing of the underwriters' option to purchase additional shares in November 2016, we have outstanding 22,546,165 of which 14,439,550 shares are subject to restrictions on transfer under 180-day lock-up arrangements with the underwriters of our initial public offering. These restrictions are due to expire on April 23, 2016, resulting in the majority of these shares becoming eligible for public sale on April 24, 2016 if they are registered under the Securities Act or if they qualify for an exemption from registration under the Securities Act including under Rules 144 or 701.

Moreover, holders of an aggregate of 13,852,369 shares of our common stock have rights, subject to conditions, to require us to file registration statements covering their shares or to include their shares in registration statements that we may file for ourselves or other stockholders. We also plan to register all shares of common stock that we may issue under our equity compensation plans. Once we register these shares, they can be freely sold in the public market upon issuance and once vested, subject to volume limitations applicable to affiliates and the lock-up agreements. If these additional shares are sold, or if it is perceived that they will be sold, in the public market, the trading price of our common stock could decline.

We might not be able to utilize a significant portion of our net operating loss carryforwards and research and development tax credit carryforwards.

As of December 31, 2015, we had federal and state net operating loss carryforwards of \$22.8 million and \$20.5 million, respectively, and federal research and development tax credit carryforwards of \$1.5 million and state research and development tax credit carryforwards of \$1.0 million. If not utilized, the net operating loss carryforwards will begin to expire in 2029. If not utilized, the research and development credits will begin to expire in 2030. These net operating loss and tax credit carryforwards could expire unused and be unavailable to offset future income tax liabilities. In addition, under Section 382 of the Internal Revenue Code of 1986, as amended, and corresponding provisions of state law, if a corporation undergoes an "ownership change," which is generally defined as a greater than 50% change, by value, in its equity ownership over a three-year period, the corporation's ability to use its pre-change net operating loss carryforwards and other pre-change tax attributes to offset its post-change income may be limited. We have not determined if we have experienced Section 382 ownership changes in the past and if a portion of our net operating loss and tax credit carryforwards are subject to an annual limitation under Section 382. In addition, we may experience ownership changes in the future as a result of subsequent shifts in our stock ownership, some of which may be outside of our control. We have not conducted a detailed study to document whether our historical activities qualify to support the research and development credit carryforwards. A detailed study could result in adjustment to our research and development credit carryforwards. If we determine that an ownership change has occurred and our ability to use our historical net operating loss and tax credit carryforwards is materially limited, or if our research and development carryforwards are adjusted, it would harm our future operating results by effectively increasing our future tax obligations.

We do not anticipate paying any cash dividends on our capital stock in the foreseeable future. Accordingly, stockholders must rely on capital appreciation, if any, for any return on their investment.

We have never declared nor paid cash dividends on our capital stock. We currently plan to retain all of our future earnings, if any, to finance the operation, development and growth of our business. In addition, the terms of

[Table of Contents](#)

any future debt or credit agreements may preclude us from paying dividends. As a result, capital appreciation, if any, of our common stock will be your sole source of gain for the foreseeable future.

Concentration of ownership of our common stock among our existing executive officers, directors and principal stockholders may prevent new investors from influencing significant corporate decisions.

Immediately following our initial public offering, our executive officers and directors, combined with our stockholders who owned more than 5% of our outstanding common stock and their affiliates in the aggregate, beneficially owned shares representing approximately 59.8% of our common stock. As a result, if these stockholders were to choose to act together, they would be able to control all matters submitted to our stockholders for approval, as well as our management and affairs. For example, these persons, if they choose to act together, would control the election of directors and approval of any merger, consolidation or sale of all or substantially all of our assets. This concentration of ownership control may:

- delay, defer or prevent a change in control;
- entrench our management or the board of directors; or
- impede a merger, consolidation, takeover or other business combination involving us that other stockholders may desire.

Provisions in our corporate charter documents and under Delaware law may prevent or frustrate attempts by our stockholders to change our management or hinder efforts to acquire a controlling interest in us.

Provisions in our corporate charter and our bylaws may discourage, delay or prevent a merger, acquisition or other change in control of us that stockholders may consider favorable, including transactions in which you might otherwise receive a premium for your shares. These provisions could also limit the price that investors might be willing to pay in the future for shares of our common stock, thereby depressing the market price of our common stock. In addition, because our board of directors is responsible for appointing the members of our management team, these provisions may frustrate or prevent any attempts by our stockholders to replace or remove our current management by making it more difficult for stockholders to replace members of our board of directors. Among other things, these provisions:

- establish a classified board of directors such that all members of the board are not elected at one time;
- allow the authorized number of our directors to be changed only by resolution of our board of directors;
- limit the manner in which stockholders can remove directors from the board;
- establish advance notice requirements for nominations for election to the board of directors or for proposing matters that can be acted on at stockholder meetings;
- require that stockholder actions must be effected at a duly called stockholder meeting and prohibit actions by our stockholders by written consent;
- limit who may call a special meeting of stockholders;
- authorize our board of directors to issue preferred stock without stockholder approval, which could be used to institute a “poison pill” that would work to dilute the stock ownership of a potential hostile acquirer, effectively preventing acquisitions that have not been approved by our board of directors; and
- require the approval of the holders of at least 75% of the votes that all our stockholders would be entitled to cast to amend or repeal certain provisions of our charter or bylaws.

Moreover, because we are incorporated in Delaware, we are governed by the provisions of Section 203 of the General Corporation Law of the State of Delaware, which prohibits a person who owns in excess of 15% of our outstanding voting stock from merging or combining with us for a period of three years after the date of the transaction in which the person acquired in excess of 15% of our outstanding voting stock, unless the merger or combination is approved in a prescribed manner. This could discourage, delay or prevent someone from acquiring us or merging with us, whether or not it is desired by, or beneficial to, our stockholders. This could also have the effect of discouraging others from making tender offers for our common stock, including transactions that may be in your best interests. These provisions may also prevent changes in our management or limit the price that investors are willing to pay for our stock.

[Table of Contents](#)

If securities or industry analysts do not publish research or publish inaccurate or unfavorable research about our business, our share price and trading volume could decline.

The trading market for our common stock may be influenced, in part, by the research and reports that industry or securities analysts publish about us or our business. We do not currently have and may never obtain research coverage by securities and industry analysts. If no or few securities or industry analysts commence coverage of us, or one or more of the analysts who cover us issues an adverse opinion about our company, our stock price would likely decline. If one or more of these analysts ceases research coverage of us or fails to regularly publish reports on us, we could lose visibility in the financial markets, which in turn could cause our stock price or trading volume to decline.

[Table of Contents](#)

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

Recent Sales of Unregistered Equity Securities

In the three months ending September 30, 2016, we have granted stock options to purchase an aggregate of 882,835 shares of our common stock, at a weighted-average exercise price of \$5.60 per share, to employees.

The issuances of the securities described above were deemed to be exempt from registration pursuant to Rule 701 promulgated under the Securities Act as transactions pursuant to compensatory benefit plans. The shares of common stock issued upon the exercise of options are deemed to be restricted securities for purposes of the Securities Act.

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. SunTrust Robinson Humphrey, Inc. acted as manager for the offering. The offering commenced on May 5, 2016 and did not terminate until the sale of all of the shares offered.

The estimated net proceeds to us, after deducting underwriting discounts of \$6.4 million and estimated offering expenses of \$2.3 million, were approximately \$82.9 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, 2016, no net offering proceeds had been used.

Item 6. Exhibits

3.1	Amended and Restated Certificate of Incorporation of the Registrant
3.2	Amended and Restated By-laws of the Registrant
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	XBRL Instance Document.
101.SCH	XBRL Schema Document.

[Table of Contents](#)

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 29, 2016

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)

Delaware

The First State

I, JEFFREY W. BULLOCK, SECRETARY OF STATE OF THE STATE OF DELAWARE, DO HEREBY CERTIFY THE ATTACHED IS A TRUE AND CORRECT COPY OF THE RESTATED CERTIFICATE OF "RA PHARMACEUTICALS, INC.", FILED IN THIS OFFICE ON THE THIRTY-FIRST DAY OF OCTOBER, A.D. 2016, AT 12:22 O'CLOCK P.M.

A FILED COPY OF THIS CERTIFICATE HAS BEEN FORWARDED TO THE NEW CASTLE COUNTY RECORDER OF DEEDS.



A handwritten signature in black ink, appearing to read "JB", is written over a horizontal line. Below the line, the text "Jeffrey W. Bullock, Secretary of State" is printed in a small font.

4539789 8100
SR# 20166422384

Authentication: 203251355
Date: 10-31-16

You may verify this certificate online at corp.delaware.gov/authver.shtml

THIRD AMENDED AND RESTATED
CERTIFICATE OF INCORPORATION

State of Delaware
Secretary of State
Division of Corporations
Delivered 12:22 PM 10/31/2016
FILED 12:22 PM 10/31/2016
SR 20166422384 - File Number 4539789

OF

RA PHARMACEUTICALS, INC.

Ra Pharmaceuticals, Inc., a corporation organized and existing under the laws of the State of Delaware (the "Corporation"), hereby certifies as follows:

1. The name of the Corporation is Ra Pharmaceuticals, Inc. The date of the filing of its original Certificate of Incorporation with the Secretary of State of the State of Delaware was June 27, 2008 (the "Original Certificate").
2. This Third Amended and Restated Certificate of Incorporation (this "Certificate") amends, restates and integrates the provisions of the Second Amended and Restated Certificate of Incorporation that was filed with the Secretary of State of the State of Delaware on July 9, 2015, and as amended from time to time (the "Amended and Restated Certificate"), and was duly adopted in accordance with the provisions of Sections 228, 242 and 245 of the General Corporation Law of the State of Delaware (the "DGCL").
3. The text of the Amended and Restated Certificate is hereby amended and restated in its entirety to provide as herein set forth in full.

ARTICLE I

The name of the Corporation is Ra Pharmaceuticals, Inc.

ARTICLE II

The address of the Corporation's registered office in the State of Delaware is c/o The Corporation Trust Company, 1209 Orange Street in the City of Wilmington, County of New Castle, 19801. The name of its registered agent at such address is The Corporation Trust Company.

ARTICLE III

The purpose of the Corporation is to engage in any lawful act or activity for which corporations may be organized under the DGCL.

ARTICLE IV

CAPITAL STOCK

The total number of shares of capital stock which the Corporation shall have authority to issue is one hundred fifty-five million (155,000,000), of which (i) one hundred fifty million (150,000,000) shares shall be a class designated as common stock, par value \$0.001 per share (the "Common Stock"), and (ii) five million (5,000,000) shares shall be a class designated as undesignated preferred stock, par value \$0.001 per share (the "Undesignated Preferred Stock").

Except as otherwise provided in any certificate of designations of any series of Undesignated Preferred Stock, the number of authorized shares of the class of Common Stock or Undesignated Preferred Stock may from time to time be increased or decreased (but not below the number of shares of such class outstanding) by the affirmative vote of the holders of a majority in voting power of the outstanding shares of capital stock of the Corporation irrespective of the provisions of Section 242(b)(2) of the DGCL.

The powers, preferences and rights of, and the qualifications, limitations and restrictions upon, each class or series of stock shall be determined in accordance with, or as set forth below in, this Article IV.

A. COMMON STOCK

Subject to all the rights, powers and preferences of the Undesignated Preferred Stock and except as provided by law or in this Certificate (or in any certificate of designations of any series of Undesignated Preferred Stock):

(a) the holders of the Common Stock shall have the exclusive right to vote for the election of directors of the Corporation (the "Directors") and on all other matters requiring stockholder action, each outstanding share entitling the holder thereof to one vote on each matter properly submitted to the stockholders of the Corporation for their vote; provided, however, that, except as otherwise required by law, holders of Common Stock, as such, shall not be entitled to vote on any amendment to this Certificate (or on any amendment to a certificate of designations of any series of Undesignated Preferred Stock) that alters or changes the powers, preferences, rights or other terms of one or more outstanding series of Undesignated Preferred Stock if the holders of such affected series of Undesignated Preferred Stock are entitled to vote, either separately or together with the holders of one or more other such series, on such amendment pursuant to this Certificate (or pursuant to a certificate of designations of any series of Undesignated Preferred Stock) or pursuant to the DGCL;

(b) dividends may be declared and paid or set apart for payment upon the Common Stock out of any assets or funds of the Corporation legally available for the payment of dividends, but only when and as declared by the Board of Directors or any authorized committee thereof; and

(c) upon the voluntary or involuntary liquidation, dissolution or winding up of the Corporation, the net assets of the Corporation shall be distributed pro rata to the holders of the Common Stock.

B. UNDESIGNATED PREFERRED STOCK

The Board of Directors or any authorized committee thereof is expressly authorized, to the fullest extent permitted by law, to provide by resolution or resolutions for, out of the unissued shares of Undesignated Preferred Stock, the issuance of the shares of Undesignated Preferred Stock in one or more series of such stock, and by filing a certificate of designations pursuant to applicable law of the State of Delaware, to establish or change from time to time the number of shares of each such series, and to fix the designations, powers, including voting powers, full or limited, or no voting powers, preferences and the relative, participating, optional or other special rights of the shares of each series and any qualifications, limitations and restrictions thereof.

ARTICLE V

STOCKHOLDER ACTION

1. **Action without Meeting.** Any action required or permitted to be taken by the stockholders of the Corporation at any annual or special meeting of stockholders of the Corporation must be effected at a duly called annual or special meeting of stockholders and may not be taken or effected by a written consent of stockholders in lieu thereof.

2. **Special Meetings.** Except as otherwise required by statute and subject to the rights, if any, of the holders of any series of Undesignated Preferred Stock, special meetings of the stockholders of the Corporation may be called only by the Board of Directors acting pursuant to a resolution approved by the affirmative vote of a majority of the Directors then in office, and special meetings of stockholders may not be called by any other person or persons. Only those matters set forth in the notice of the special meeting may be considered or acted upon at a special meeting of stockholders of the Corporation.

ARTICLE VI

DIRECTORS

1. **General.** The business and affairs of the Corporation shall be managed by or under the direction of the Board of Directors except as otherwise provided herein or required by law.

2. **Election of Directors.** Election of Directors need not be by written ballot unless the By-laws of the Corporation (the "By-laws") shall so provide.

3. Number of Directors; Term of Office. The number of Directors of the Corporation shall be fixed solely and exclusively by resolution duly adopted from time to time by the Board of Directors. The Directors, other than those who may be elected by the holders of any series of Undesignated Preferred Stock, shall be classified, with respect to the term for which they severally hold office, into three classes. The initial Class I Directors of the Corporation shall be Douglas A. Treco and Edward T. Mathers; the initial Class II Directors of the Corporation shall be Robert Heft, Jason Lettmann and Rajeev Shah; and the initial Class III Directors of the Corporation shall be Timothy Pearson and Peter Tuxen Bisgaard. The initial Class I Directors shall serve for a term expiring at the annual meeting of stockholders to be held in 2017, the initial Class II Directors shall serve for a term expiring at the annual meeting of stockholders to be held in 2018, and the initial Class III Directors shall serve for a term expiring at the annual meeting of stockholders to be held in 2019. At each annual meeting of stockholders, Directors elected to succeed those Directors whose terms expire shall be elected for a term of office to expire at the third succeeding annual meeting of stockholders after their election. Notwithstanding the foregoing, the Directors elected to each class shall hold office until their successors are duly elected and qualified or until their earlier resignation, death or removal.

Notwithstanding the foregoing, whenever, pursuant to the provisions of Article IV of this Certificate, the holders of any one or more series of Undesignated Preferred Stock shall have the right, voting separately as a series or together with holders of other such series, to elect Directors at an annual or special meeting of stockholders, the election, term of office, filling of vacancies and other features of such directorships shall be governed by the terms of this Certificate and any certificate of designations applicable to such series.

4. Vacancies. Subject to the rights, if any, of the holders of any series of Undesignated Preferred Stock to elect Directors and to fill vacancies in the Board of Directors relating thereto, any and all vacancies and newly created directorships in the Board of Directors, however occurring, including, without limitation, by reason of an increase in the size of the Board of Directors, or the death, resignation, disqualification or removal of a Director, shall be filled solely and exclusively by the affirmative vote of a majority of the remaining Directors then in office, even if less than a quorum of the Board of Directors, and not by the stockholders. Any Director appointed in accordance with the preceding sentence shall hold office for the remainder of the full term of the class of Directors in which the new directorship was created or the vacancy occurred and until such Director's successor shall have been duly elected and qualified or until his or her earlier resignation, death or removal. Subject to the rights, if any, of the holders of any series of Undesignated Preferred Stock to elect Directors, when the number of Directors is increased or decreased, the Board of Directors shall, subject to Article VI.3 hereof, determine the class or classes to which the increased or decreased number of Directors shall be apportioned; provided, however, that no decrease in the number of Directors shall shorten the term of any incumbent Director.

5. Removal. Subject to the rights, if any, of any series of Undesignated Preferred Stock to elect Directors and to remove any Director whom the holders of any such series have the right to elect, any Director (including persons elected by Directors to fill vacancies in the

Board of Directors) may be removed from office (i) only with cause and (ii) only by the affirmative vote of the holders of 75% or more of the voting power of the outstanding shares of capital stock then entitled to vote at an election of Directors. At least forty-five (45) days prior to any annual or special meeting of stockholders at which it is proposed that any Director be removed from office, written notice of such proposed removal and the alleged grounds thereof shall be sent to the Director whose removal will be considered at the meeting.

ARTICLE VII

LIMITATION OF LIABILITY

A Director of the Corporation shall not be personally liable to the Corporation or its stockholders for monetary damages for breach of fiduciary duty as a Director, except for liability (a) for any breach of the Director's duty of loyalty to the Corporation or its stockholders, (b) for acts or omissions not in good faith or which involve intentional misconduct or a knowing violation of law, (c) under Section 174 of the DGCL or (d) for any transaction from which the Director derived an improper personal benefit. If the DGCL is amended after the effective date of this Certificate to authorize corporate action further eliminating or limiting the personal liability of Directors, then the liability of a Director of the Corporation shall be eliminated or limited to the fullest extent permitted by the DGCL, as so amended.

Any amendment, repeal or modification of this Article VII by either of (i) the stockholders of the Corporation or (ii) an amendment to the DGCL, shall not adversely affect any right or protection existing at the time of such amendment, repeal or modification with respect to any acts or omissions occurring before such amendment, repeal or modification of a person serving as a Director at the time of such amendment, repeal or modification.

ARTICLE VIII

AMENDMENT OF BY-LAWS

1. Amendment by Directors. Except as otherwise provided by law, the By-laws of the Corporation may be amended or repealed by the Board of Directors by the affirmative vote of a majority of the Directors then in office.

2. Amendment by Stockholders. The By-laws of the Corporation may be amended or repealed at any annual meeting of stockholders, or special meeting of stockholders called for such purpose, by the affirmative vote of at least 75% of the voting power of the outstanding shares of capital stock entitled to vote on such amendment or repeal, voting together as a single class; provided, however, that if the Board of Directors recommends that stockholders approve such amendment or repeal at such meeting of stockholders, such amendment or repeal shall only require the affirmative vote of the majority of the voting power of the outstanding shares of capital stock entitled to vote on such amendment or repeal, voting together as a single class.

ARTICLE IX

AMENDMENT OF CERTIFICATE OF INCORPORATION

The Corporation reserves the right to amend or repeal this Certificate in the manner now or hereafter prescribed by statute and this Certificate, and all rights conferred upon stockholders herein are granted subject to this reservation. Whenever any vote of the holders of capital stock of the Corporation is required to amend or repeal any provision of this Certificate, and in addition to any other vote of holders of capital stock that is required by this Certificate or by law, such amendment or repeal shall require the affirmative vote of the majority of the voting power of the outstanding shares of capital stock entitled to vote on such amendment or repeal, and the affirmative vote of the majority of the voting power of the outstanding shares of each class entitled to vote thereon as a class, at a duly constituted meeting of stockholders called expressly for such purpose; provided, however, that the affirmative vote of not less than 75% of the voting power of the outstanding shares of capital stock entitled to vote on such amendment or repeal, and the affirmative vote of not less than 75% of the voting power of the outstanding shares of each class entitled to vote thereon as a class, shall be required to amend or repeal any provision of Article V, Article VI, Article WI, Article VIII or Article IX of this Certificate.

[End of Text]

THIS THIRD AMENDED AND RESTATED CERTIFICATE OF INCORPORATION is executed as of this 31st day of October, 2016.

RA PHARMACEUTICALS, INC.

By: /s/Douglas A. Treco
Name: Douglas A. Treco, Ph.D.
Title: President and Chief Executive Officer

AMENDED AND RESTATED
BY-LAWS
OF
RA PHARMACEUTICALS, INC.

(the "Corporation")

ARTICLE I

Stockholders

SECTION 1. Annual Meeting. The annual meeting of stockholders (any such meeting being referred to in these By-laws as an "Annual Meeting") shall be held at the hour, date and place within or without the United States which is fixed by the Board of Directors, which time, date and place may subsequently be changed at any time by vote of the Board of Directors.

SECTION 2. Notice of Stockholder Business and Nominations.

(a) Annual Meetings of Stockholders.

(1) Nominations of persons for election to the Board of Directors of the Corporation and the proposal of other business to be considered by the stockholders may be brought before an Annual Meeting (i) by or at the direction of the Board of Directors or (ii) by any stockholder of the Corporation who was a stockholder of record at the time of giving of notice provided for in this By-law, who is entitled to vote at the meeting, who is present (in person or by proxy) at the meeting and who complies with the notice procedures set forth in this By-law as to such nomination or business. For the avoidance of doubt, the foregoing clause (ii) shall be the exclusive means for a stockholder to bring nominations or business properly before an Annual Meeting (other than matters properly brought under Rule 14a-8 (or any successor rule) under the Securities Exchange Act of 1934, as amended (the "Exchange Act")), and such stockholder must comply with the notice and other procedures set forth in Article I, Section 2(a)(2) and (3) of this By-law to bring such nominations or business properly before an Annual Meeting. In addition to the other requirements set forth in this By-law, for any proposal of business to be considered at an Annual Meeting, it must be a proper subject for action by stockholders of the Corporation under Delaware law.

(2) For nominations or other business to be properly brought before an Annual Meeting by a stockholder pursuant to clause (ii) of Article I, Section 2(a)(1) of this By-law, the stockholder must (i) have given Timely Notice (as defined below) thereof in writing to the Secretary of the Corporation, (ii) have provided any updates or supplements to such notice at the times and in the forms required by this By-law and (iii) together with the beneficial owner(s), if any, on whose behalf the nomination or

business proposal is made, have acted in accordance with the representations set forth in the Solicitation Statement (as defined below) required by this By-law. To be timely, a stockholder's written notice shall be received by the Secretary at the principal executive offices of the Corporation not later than the close of business on the ninetieth (90th) day nor earlier than the close of business on the one hundred twentieth (120th) day prior to the one-year anniversary of the preceding year's Annual Meeting; provided, however, that in the event the Annual Meeting is first convened more than thirty (30) days before or more than sixty (60) days after such anniversary date, or if no Annual Meeting were held in the preceding year, notice by the stockholder to be timely must be received by the Secretary of the Corporation not later than the close of business on the later of the ninetieth (90th) day prior to the scheduled date of such Annual Meeting or the tenth (10th) day following the day on which public announcement of the date of such meeting is first made (such notice within such time periods shall be referred to as "Timely Notice"). Notwithstanding anything to the contrary provided herein, for the first Annual Meeting following the initial public offering of common stock of the Corporation, a stockholder's notice shall be timely if received by the Secretary at the principal executive offices of the Corporation not later than the close of business on the later of the ninetieth (90th) day prior to the scheduled date of such Annual Meeting or the tenth (10th) day following the day on which public announcement of the date of such Annual Meeting is first made or sent by the Corporation. Such stockholder's Timely Notice shall set forth:

(A) as to each person whom the stockholder proposes to nominate for election or reelection as a director, (i) the name, age, business address and residence address of the nominee, (ii) the principal occupation or employment of the nominee, (iii) the class and number of shares of the corporation that are held of record or are beneficially owned by the nominee and any derivative positions held or beneficially held by the nominee, (iv) whether and the extent to which any hedging or other transaction or series of transactions has been entered into by or on behalf of the nominee with respect to any securities of the corporation, and a description of any other agreement, arrangement or understanding (including any short position or any borrowing or lending of shares), the effect or intent of which is to mitigate loss to, or to manage the risk or benefit of share price changes for, or to increase or decrease the voting power of the nominee, (v) a description of all arrangements or understandings between or among the stockholder and each nominee and any other person or persons (naming such person or persons) pursuant to which the nominations are to be made by the stockholder or concerning the nominee's potential service on the Board of Directors, (vi) a written statement executed by the nominee acknowledging that as a director of the corporation, the nominee will owe fiduciary duties under Delaware law with respect to the corporation and its stockholders, and (vii) all information relating to such person that is required to be disclosed in solicitations of proxies for election of directors in an election contest, or is otherwise required, in each case pursuant to Regulation 14A under the Exchange Act (including such person's written consent to being named in the proxy statement as a nominee and to serving as a director if elected);

(B) as to any other business that the stockholder proposes to bring before the meeting, a brief description of the business desired to be brought before the meeting, the reasons for conducting such business at the meeting, the text, if any, of any resolutions or By-law amendment proposed for adoption, and any material interest in such business of each Proposing Person (as defined below);

(C) (i) the name and address of the stockholder giving the notice, as they appear on the Corporation's books, and the names and addresses of the other Proposing Persons (if any) and (ii) as to each Proposing Person, the following information: (a) the class or series and number of all shares of capital stock of the Corporation which are, directly or indirectly, owned beneficially or of record by such Proposing Person or any of its affiliates or associates (as such terms are defined in Rule 12b-2 promulgated under the Exchange Act), including any shares of any class or series of capital stock of the Corporation as to which such Proposing Person or any of its affiliates or associates has a right to acquire beneficial ownership at any time in the future, (b) all Synthetic Equity Interests (as defined below) in which such Proposing Person or any of its affiliates or associates, directly or indirectly, holds an interest including a description of the material terms of each such Synthetic Equity Interest, including without limitation, identification of the counterparty to each such Synthetic Equity Interest and disclosure, for each such Synthetic Equity Interest, as to (x) whether or not such Synthetic Equity Interest conveys any voting rights, directly or indirectly, in such shares to such Proposing Person, (y) whether or not such Synthetic Equity Interest is required to be, or is capable of being, settled through delivery of such shares and (z) whether or not such Proposing Person and/or, to the extent known, the counterparty to such Synthetic Equity Interest has entered into other transactions that hedge or mitigate the economic effect of such Synthetic Equity Interest, (c) any proxy (other than a revocable proxy given in response to a public proxy solicitation made pursuant to, and in accordance with, the Exchange Act), agreement, arrangement, understanding or relationship pursuant to which such Proposing Person has or shares a right to, directly or indirectly, vote any shares of any class or series of capital stock of the Corporation, (d) any rights to dividends or other distributions on the shares of any class or series of capital stock of the Corporation, directly or indirectly, owned beneficially by such Proposing Person that are separated or separable from the underlying shares of the Corporation, and (e) any performance-related fees (other than an asset based fee) that such Proposing Person, directly or indirectly, is entitled to based on any increase or decrease in the value of shares of any class or series of capital stock of the Corporation or any Synthetic Equity Interests (the disclosures to be made pursuant to the foregoing clauses (a) through (e) are referred to, collectively, as "Material Ownership Interests") and (iii) a description of the material terms of all agreements, arrangements or understandings (whether or not in writing) entered into by any Proposing Person or any of its affiliates or associates with any other person for the purpose of acquiring, holding, disposing or voting of any shares of any class or series of capital stock of the Corporation;

(D) (i) a description of all agreements, arrangements or understandings by and among any of the Proposing Persons, or by and among any Proposing Persons and any other person (including with any proposed nominee(s)), pertaining to the nomination(s), the nominee's service on the Board of Directors, or other business proposed to be brought before the meeting of stockholders (which description shall identify the name of each other person who is party to such an agreement, arrangement or understanding), and (ii) identification of the names and addresses of other stockholders (including beneficial owners) known by any of the Proposing Persons to support such nominations or other business proposal(s), and to the extent known the class and number of all shares of the Corporation's capital stock owned beneficially or of record by such other stockholder(s) or other beneficial owner(s); and

(E) a statement whether or not the stockholder giving the notice and/or the other Proposing Person(s), if any, will deliver a proxy statement and form of proxy to holders of, in the case of a business proposal, at least the percentage of voting power of all of the shares of capital stock of the Corporation required under applicable law to approve the proposal or, in the case of a nomination or nominations, at least the percentage of voting power of all of the shares of capital stock of the Corporation reasonably believed by such Proposing Person to be sufficient to elect the nominee or nominees proposed to be nominated by such stockholder (such statement, the "Solicitation Statement").

For purposes of this Article I of these By-laws, the term "Proposing Person" shall mean the following persons: (i) the stockholder of record providing the notice of nominations or business proposed to be brought before a stockholders' meeting, and (ii) the beneficial owner(s), if different, on whose behalf the nominations or business proposed to be brought before a stockholders' meeting is made. For purposes of this Section 2 of Article I of these By-laws, the term "Synthetic Equity Interest" shall mean any transaction, agreement or arrangement (or series of transactions, agreements or arrangements), including, without limitation, any derivative, swap, hedge, repurchase or so-called "stock borrowing" agreement or arrangement, the purpose or effect of which is to, directly or indirectly: (a) give a person or entity economic benefit and/or risk similar to ownership of shares of any class or series of capital stock of the Corporation, in whole or in part, including due to the fact that such transaction, agreement or arrangement provides, directly or indirectly, the opportunity to profit or avoid a loss from any increase or decrease in the value of any shares of any class or series of capital stock of the Corporation, (b) mitigate loss to, reduce the economic risk of or manage the risk of share price changes for, any person or entity with respect to any shares of any class or series of capital stock of the Corporation, (c) otherwise provide in any manner the opportunity to profit or avoid a loss from any decrease in the value of any shares of any class or series of capital stock of the Corporation, or (d) increase or decrease the voting power of any person or entity with respect to any shares of any class or series of capital stock of the Corporation.

(3) A stockholder providing Timely Notice of nominations or business proposed to be brought before an Annual Meeting shall further update and supplement

such notice, if necessary, so that the information (including, without limitation, the Material Ownership Interests information) provided or required to be provided in such notice pursuant to this By-law shall be true and correct as of the record date for the meeting and as of the date that is ten (10) business days prior to such Annual Meeting, and such update and supplement shall be received by the Secretary at the principal executive offices of the Corporation not later than the close of business on the fifth (5th) business day after the record date for the Annual Meeting (in the case of the update and supplement required to be made as of the record date), and not later than the close of business on the eighth (8th) business day prior to the date of the Annual Meeting (in the case of the update and supplement required to be made as of ten (10) business days prior to the meeting).

(4) Notwithstanding anything in the second sentence of Article I, Section 2(a)(2) of this By-law to the contrary, in the event that the number of directors to be elected to the Board of Directors of the Corporation is increased and there is no public announcement naming all of the nominees for director or specifying the size of the increased Board of Directors made by the Corporation at least ten (10) days before the last day a stockholder may deliver a notice of nomination in accordance with the second sentence of Article I, Section 2(a)(2), a stockholder's notice required by this By-law shall also be considered timely, but only with respect to nominees for any new positions created by such increase, if it shall be received by the Secretary of the Corporation not later than the close of business on the tenth (10th) day following the day on which such public announcement is first made by the Corporation.

(b) General.

(1) Only such persons who are nominated in accordance with the provisions of this By-law shall be eligible for election and to serve as directors and only such business shall be conducted at an Annual Meeting as shall have been brought before the meeting in accordance with the provisions of this By-law or in accordance with Rule 14a-8 under the Exchange Act. The Board of Directors or a designated committee thereof shall have the power to determine whether a nomination or any business proposed to be brought before the meeting was made in accordance with the provisions of this By-law. If neither the Board of Directors nor such designated committee makes a determination as to whether any stockholder proposal or nomination was made in accordance with the provisions of this By-law, the presiding officer of the Annual Meeting shall have the power and duty to determine whether the stockholder proposal or nomination was made in accordance with the provisions of this By-law. If the Board of Directors or a designated committee thereof or the presiding officer, as applicable, determines that any stockholder proposal or nomination was not made in accordance with the provisions of this By-law, such proposal or nomination shall be disregarded and shall not be presented for action at the Annual Meeting.

(2) Except as otherwise required by law, nothing in this Article I, Section 2 shall obligate the Corporation or the Board of Directors to include in any proxy statement or other stockholder communication distributed on behalf of the Corporation or the Board

of Directors information with respect to any nominee for director or any other matter of business submitted by a stockholder.

(3) Notwithstanding the foregoing provisions of this Article I, Section 2, if the nominating or proposing stockholder (or a qualified representative of the stockholder) does not appear at the Annual Meeting to present a nomination or any business, such nomination or business shall be disregarded, notwithstanding that proxies in respect of such vote may have been received by the Corporation. For purposes of this Article I, Section 2, to be considered a qualified representative of the proposing stockholder, a person must be authorized by a written instrument executed by such stockholder or an electronic transmission delivered by such stockholder to act for such stockholder as proxy at the meeting of stockholders and such person must produce such written instrument or electronic transmission, or a reliable reproduction of the written instrument or electronic transmission, to the presiding officer at the meeting of stockholders.

(4) For purposes of this By-law, "public announcement" shall mean disclosure in a press release reported by the Dow Jones News Service, Associated Press or comparable national news service or in a document publicly filed by the Corporation with the Securities and Exchange Commission pursuant to Section 13, 14 or 15(d) of the Exchange Act.

(5) Notwithstanding the foregoing provisions of this By-law, a stockholder shall also comply with all applicable requirements of the Exchange Act and the rules and regulations thereunder with respect to the matters set forth in this By-law. Nothing in this By-law shall be deemed to affect any rights of (i) stockholders to have proposals included in the Corporation's proxy statement pursuant to Rule 14a-8 (or any successor rule), as applicable, under the Exchange Act and, to the extent required by such rule, have such proposals considered and voted on at an Annual Meeting or (ii) the holders of any series of Undesignated Preferred Stock to elect directors under specified circumstances.

SECTION 3. Special Meetings. Except as otherwise required by statute and subject to the rights, if any, of the holders of any series of Undesignated Preferred Stock, special meetings of the stockholders of the Corporation may be called only by the Board of Directors acting pursuant to a resolution approved by the affirmative vote of a majority of the Directors then in office. The Board of Directors may postpone or reschedule any previously scheduled special meeting of stockholders. Only those matters set forth in the notice of the special meeting may be considered or acted upon at a special meeting of stockholders of the Corporation. Nominations of persons for election to the Board of Directors of the Corporation and stockholder proposals of other business shall not be brought before a special meeting of stockholders to be considered by the stockholders.

SECTION 4. Notice of Meetings; Adjournments.

(a) A notice of each Annual Meeting stating the hour, date and place, if any, of such Annual Meeting and the means of remote communication, if any, by which stockholders and proxyholders may be deemed to be present in person and vote at such meeting, shall be given not

less than ten (10) days nor more than sixty (60) days before the Annual Meeting, to each stockholder entitled to vote thereat by delivering such notice to such stockholder or by mailing it, postage prepaid, addressed to such stockholder at the address of such stockholder as it appears on the Corporation's stock transfer books. Without limiting the manner by which notice may otherwise be given to stockholders, any notice to stockholders may be given by electronic transmission in the manner provided in Section 232 of the Delaware General Corporation Law ("DGCL").

(b) Unless otherwise required by the DGCL, notice of all special meetings of stockholders shall be given in the same manner as provided for Annual Meetings, except that the notice of all special meetings shall state the purpose or purposes for which the meeting has been called.

(c) Notice of an Annual Meeting or special meeting of stockholders need not be given to a stockholder if a waiver of notice is executed, or waiver of notice by electronic transmission is provided, before or after such meeting by such stockholder or if such stockholder attends such meeting, unless such attendance is for the express purpose of objecting at the beginning of the meeting to the transaction of any business because the meeting was not lawfully called or convened.

(d) The Board of Directors may postpone and reschedule any previously scheduled Annual Meeting or special meeting of stockholders and any record date with respect thereto, regardless of whether any notice or public disclosure with respect to any such meeting has been sent or made pursuant to Section 2 of this Article I of these By-laws or otherwise. In no event shall the public announcement of an adjournment, postponement or rescheduling of any previously scheduled meeting of stockholders commence a new time period for the giving of a stockholder's notice under this Article I of these By-laws.

(e) The presiding officer may adjourn any meeting of stockholders whether or not a quorum is present. When any Annual Meeting or special meeting of stockholders is adjourned to another hour, date or place, notice need not be given of the adjourned meeting other than an announcement at the meeting at which the adjournment is taken of the hour, date and place, if any, to which the meeting is adjourned and the means of remote communications, if any, by which stockholders and proxyholders may be deemed to be present in person and vote at such adjourned meeting; provided, however, that if the adjournment is for more than thirty (30) days from the meeting date, or if after the adjournment a new record date is fixed for the adjourned meeting, notice of the adjourned meeting and the means of remote communications, if any, by which stockholders and proxyholders may be deemed to be present in person and vote at such adjourned meeting shall be given to each stockholder of record entitled to vote thereat and each stockholder who, by law or under the Certificate of Incorporation of the Corporation (as the same may hereafter be amended and/or restated, the "Certificate") or these By-laws, is entitled to such notice.

SECTION 5. Quorum. A majority of the voting power of the outstanding shares entitled to vote, present in person or represented by proxy, shall constitute a quorum at any meeting of stockholders. If less than a quorum is present at a meeting, the holders of voting stock representing a majority of the voting power present at the meeting or the presiding officer

may adjourn the meeting from time to time, and the meeting may be held as adjourned without further notice, except as provided in Section 4 of this Article I. At such adjourned meeting at which a quorum is present, any business may be transacted which might have been transacted at the original meeting. The stockholders present at a duly constituted meeting may continue to transact business until adjournment, notwithstanding the withdrawal of enough stockholders to leave less than a quorum.

SECTION 6. Voting and Proxies. Stockholders shall have one vote for each share of stock entitled to vote owned by them of record according to the stock ledger of the Corporation as of the record date, unless otherwise provided by law or by the Certificate. Stockholders may vote either (i) in person, (ii) by written proxy or (iii) by a transmission permitted by Section 212(c) of the DGCL. Any copy, facsimile telecommunication or other reliable reproduction of the writing or transmission permitted by Section 212(c) of the DGCL may be substituted for or used in lieu of the original writing or transmission for any and all purposes for which the original writing or transmission could be used, provided that such copy, facsimile telecommunication or other reproduction shall be a complete reproduction of the entire original writing or transmission. Proxies shall be filed in accordance with the procedures established for the meeting of stockholders. A proxy with respect to stock held in the name of two or more persons shall be valid if executed by or on behalf of any one of them unless at or prior to the exercise of the proxy the Corporation receives a specific written notice to the contrary from any one of them.

SECTION 7. Action at Meeting. When a quorum is present at any meeting of stockholders, any matter before any such meeting (other than an election of a director or directors) shall be decided by a majority of the votes properly cast for and against such matter, except where a larger vote is required by law, by the Certificate or by these By-laws. Any election of directors by stockholders shall be determined by a plurality of the votes properly cast on the election of directors.

SECTION 8. Stockholder Lists. The Secretary or an Assistant Secretary (or the Corporation's transfer agent or other person authorized by these By-laws or by law) shall prepare and make, at least ten (10) days before every Annual Meeting or special meeting of stockholders, a complete list of the stockholders entitled to vote at the meeting, arranged in alphabetical order, and showing the address of each stockholder and the number of shares registered in the name of each stockholder. Such list shall be open to the examination of any stockholder, for a period of at least ten (10) days prior to the meeting as provided in the manner, and subject to the terms, set forth in Section 219 of the DGCL (or any successor provision). The list shall also be open to the examination of any stockholder.

SECTION 9. Presiding Officer. The Board of Directors shall designate a representative to preside over all Annual Meetings or special meetings of stockholders, provided that if the Board of Directors does not so designate such a presiding officer, then the Chairman of the Board, if one is elected, shall preside over such meetings. If the Board of Directors does not so designate such a presiding officer and there is no Chairman of the Board or the Chairman of the Board is unable to so preside or is absent, then the Chief Executive Officer, if one is elected,

shall preside over such meetings, provided further that if there is no Chief Executive Officer or the Chief Executive Officer is unable to so preside or is absent, then the President shall preside over such meetings. The presiding officer at any Annual Meeting or special meeting of stockholders shall have the power, among other things, to adjourn such meeting at any time and from time to time, subject to Sections 4 and 5 of this Article I. The order of business and all other matters of procedure at any meeting of the stockholders shall be determined by the presiding officer.

SECTION 10. Inspectors of Elections. The Corporation shall, in advance of any meeting of stockholders, appoint one or more inspectors to act at the meeting and make a written report thereof. The Corporation may designate one or more persons as alternate inspectors to replace any inspector who fails to act. If no inspector or alternate is able to act at a meeting of stockholders, the presiding officer shall appoint one or more inspectors to act at the meeting. Any inspector may, but need not, be an officer, employee or agent of the Corporation. Each inspector, before entering upon the discharge of his or her duties, shall take and sign an oath faithfully to execute the duties of inspector with strict impartiality and according to the best of his or her ability. The inspectors shall perform such duties as are required by the DGCL, including the counting of all votes and ballots. The inspectors may appoint or retain other persons or entities to assist the inspectors in the performance of the duties of the inspectors. The presiding officer may review all determinations made by the inspectors, and in so doing the presiding officer shall be entitled to exercise his or her sole judgment and discretion and he or she shall not be bound by any determinations made by the inspectors. All determinations by the inspectors and, if applicable, the presiding officer, shall be subject to further review by any court of competent jurisdiction.

ARTICLE II

Directors

SECTION 1. Powers. The business and affairs of the Corporation shall be managed by or under the direction of the Board of Directors except as otherwise provided by the Certificate or required by law.

SECTION 2. Number and Terms. The number of directors of the Corporation shall be fixed solely and exclusively by resolution duly adopted from time to time by the Board of Directors. The directors shall hold office in the manner provided in the Certificate.

SECTION 3. Qualification. No director need be a stockholder of the Corporation.

SECTION 4. Vacancies. Vacancies in the Board of Directors shall be filled in the manner provided in the Certificate.

SECTION 5. Removal. Directors may be removed from office only in the manner provided in the Certificate.

SECTION 6. Resignation. A director may resign at any time by electronic transmission or by giving written notice to the Chairman of the Board, if one is elected, the President or the Secretary. A resignation shall be effective upon receipt, unless the resignation otherwise provides.

SECTION 7. Regular Meetings. The regular annual meeting of the Board of Directors shall be held, without notice other than this Section 7, on the same date and at the same place as the Annual Meeting following the close of such meeting of stockholders. Other regular meetings of the Board of Directors may be held at such hour, date and place as the Board of Directors may by resolution from time to time determine.

SECTION 8. Special Meetings. Special meetings of the Board of Directors may be called, orally or in writing, by or at the request of a majority of the directors, the Chairman of the Board, if one is elected, or the President. The person calling any such special meeting of the Board of Directors may fix the hour, date and place thereof.

SECTION 9. Notice of Meetings. Notice of the hour, date and place of all special meetings of the Board of Directors shall be given to each director by the Secretary or an Assistant Secretary, or in case of the death, absence, incapacity or refusal of such persons, by the Chairman of the Board, if one is elected, or the President or such other officer designated by the Chairman of the Board, if one is elected, or the President. Notice of any special meeting of the Board of Directors shall be given to each director in person, by telephone, or by facsimile, electronic mail or other form of electronic communication, sent to his or her business or home address, at least twenty-four (24) hours in advance of the meeting, or by written notice mailed to his or her business or home address, at least forty-eight (48) hours in advance of the meeting. Such notice shall be deemed to be delivered when hand-delivered to such address, read to such director by telephone, deposited in the mail so addressed, with postage thereon prepaid if mailed, dispatched or transmitted if sent by facsimile transmission or by electronic mail or other form of electronic communications. A written waiver of notice signed or electronically transmitted before or after a meeting by a director and filed with the records of the meeting shall be deemed to be equivalent to notice of the meeting. The attendance of a director at a meeting shall constitute a waiver of notice of such meeting, except where a director attends a meeting for the express purpose of objecting at the beginning of the meeting to the transaction of any business because such meeting is not lawfully called or convened. Except as otherwise required by law, by the Certificate or by these By-laws, neither the business to be transacted at, nor the purpose of, any meeting of the Board of Directors need be specified in the notice or waiver of notice of such meeting.

SECTION 10. Quorum. At any meeting of the Board of Directors, a majority of the total number of directors shall constitute a quorum for the transaction of business, but if less than a quorum is present at a meeting, a majority of the directors present may adjourn the meeting from time to time, and the meeting may be held as adjourned without further notice. Any business which might have been transacted at the meeting as originally noticed may be transacted at such adjourned meeting at which a quorum is present. For purposes of this section, the total number

of directors includes any unfilled vacancies or newly created directorships on the Board of Directors.

SECTION 11. Action at Meeting. At any meeting of the Board of Directors at which a quorum is present, the vote of a majority of the directors present shall constitute action by the Board of Directors, unless otherwise required by law, by the Certificate or by these By-laws.

SECTION 12. Action by Consent. Any action required or permitted to be taken at any meeting of the Board of Directors may be taken without a meeting if all members of the Board of Directors consent thereto in writing or by electronic transmission and the writing or writings or electronic transmission or transmissions are filed with the records of the meetings of the Board of Directors. Such filing shall be in paper form if the minutes are maintained in paper form and shall be in electronic form if the minutes are maintained in electronic form. Such consent shall be treated as a resolution of the Board of Directors for all purposes.

SECTION 13. Manner of Participation. Directors may participate in meetings of the Board of Directors by means of conference telephone or other communications equipment by means of which all directors participating in the meeting can hear each other, and participation in a meeting in accordance herewith shall constitute presence in person at such meeting for purposes of these By-laws.

SECTION 14. Presiding Director. The Board of Directors shall designate a representative to preside over all meetings of the Board of Directors, provided that if the Board of Directors does not so designate such a presiding director or such designated presiding director is unable to so preside or is absent, then the Chairman of the Board, if one is elected, shall preside over all meetings of the Board of Directors. If both the designated presiding director, if one is so designated, and the Chairman of the Board, if one is elected, are unable to preside or are absent, the Board of Directors shall designate an alternate representative to preside over a meeting of the Board of Directors.

SECTION 15. Committees. The Board of Directors, by vote of a majority of the directors then in office, may elect one or more committees, including, without limitation, a Compensation Committee, a Nominating & Corporate Governance Committee and an Audit Committee, and may delegate thereto some or all of its powers except those which by law, by the Certificate or by these By-laws may not be delegated. Except as the Board of Directors may otherwise determine, any such committee may make rules for the conduct of its business, but unless otherwise provided by the Board of Directors or in such rules, its business shall be conducted so far as possible in the same manner as is provided by these By-laws for the Board of Directors. All members of such committees shall hold such offices at the pleasure of the Board of Directors. The Board of Directors may abolish any such committee at any time. Any committee to which the Board of Directors delegates any of its powers or duties shall keep records of its meetings and shall report its action to the Board of Directors.

ARTICLE III

Officers

SECTION 1. Enumeration. The officers of the Corporation shall consist of a President, a Treasurer, a Secretary and such other officers, including, without limitation, a Chairman of the Board of Directors, a Chief Executive Officer and one or more Vice Presidents (including Executive Vice Presidents or Senior Vice Presidents), Assistant Vice Presidents, Assistant Treasurers and Assistant Secretaries, as the Board of Directors may determine.

SECTION 2. Election. At the regular annual meeting of the Board of Directors following the Annual Meeting, the Board of Directors shall elect the President, the Treasurer and the Secretary. Other officers may be elected by the Board of Directors at such regular annual meeting of the Board of Directors or at any other regular or special meeting.

SECTION 3. Qualification. No officer need be a stockholder or a director. Any person may occupy more than one office of the Corporation at any time.

SECTION 4. Tenure. Except as otherwise provided by the Certificate or by these By-laws, each of the officers of the Corporation shall hold office until the regular annual meeting of the Board of Directors following the next Annual Meeting and until his or her successor is elected and qualified or until his or her earlier resignation or removal.

SECTION 5. Resignation. Any officer may resign by delivering his or her written or electronically transmitted resignation to the Corporation addressed to the President or the Secretary, and such resignation shall be effective upon receipt, unless the resignation otherwise provides.

SECTION 6. Removal. Except as otherwise provided by law or by resolution of the Board of Directors, the Board of Directors may remove any officer with or without cause by the affirmative vote of a majority of the directors then in office.

SECTION 7. Absence or Disability. In the event of the absence or disability of any officer, the Board of Directors may designate another officer to act temporarily in place of such absent or disabled officer.

SECTION 8. Vacancies. Any vacancy in any office may be filled for the unexpired portion of the term by the Board of Directors.

SECTION 9. President. The President shall, subject to the direction of the Board of Directors, have such powers and shall perform such duties as the Board of Directors may from time to time designate.

SECTION 10. Chairman of the Board. The Chairman of the Board, if one is elected, shall have such powers and shall perform such duties as the Board of Directors may from time to time designate.

SECTION 11. Chief Executive Officer. The Chief Executive Officer, if one is elected, shall have such powers and shall perform such duties as the Board of Directors may from time to time designate.

SECTION 12. Vice Presidents and Assistant Vice Presidents. Any Vice President (including any Executive Vice President or Senior Vice President) and any Assistant Vice President shall have such powers and shall perform such duties as the Board of Directors or the Chief Executive Officer may from time to time designate.

SECTION 13. Treasurer and Assistant Treasurers. The Treasurer shall, subject to the direction of the Board of Directors and except as the Board of Directors or the Chief Executive Officer may otherwise provide, have general charge of the financial affairs of the Corporation and shall cause to be kept accurate books of account. The Treasurer shall have custody of all funds, securities, and valuable documents of the Corporation. He or she shall have such other duties and powers as may be designated from time to time by the Board of Directors or the Chief Executive Officer. Any Assistant Treasurer shall have such powers and perform such duties as the Board of Directors or the Chief Executive Officer may from time to time designate.

SECTION 14. Secretary and Assistant Secretaries. The Secretary shall record all the proceedings of the meetings of the stockholders and the Board of Directors (including committees of the Board of Directors) in books kept for that purpose. In his or her absence from any such meeting, a temporary secretary chosen at the meeting shall record the proceedings thereof. The Secretary shall have charge of the stock ledger (which may, however, be kept by any transfer or other agent of the Corporation). The Secretary shall have custody of the seal of the Corporation, and the Secretary, or an Assistant Secretary shall have authority to affix it to any instrument requiring it, and, when so affixed, the seal may be attested by his or her signature or that of an Assistant Secretary. The Secretary shall have such other duties and powers as may be designated from time to time by the Board of Directors or the Chief Executive Officer. In the absence of the Secretary, any Assistant Secretary may perform his or her duties and responsibilities. Any Assistant Secretary shall have such powers and perform such duties as the Board of Directors or the Chief Executive Officer may from time to time designate.

SECTION 15. Other Powers and Duties. Subject to these By-laws and to such limitations as the Board of Directors may from time to time prescribe, the officers of the Corporation shall each have such powers and duties as generally pertain to their respective offices, as well as such powers and duties as from time to time may be conferred by the Board of Directors or the Chief Executive Officer.

ARTICLE IV

Capital Stock

SECTION 1. Certificates of Stock. Each stockholder shall be entitled to a certificate of the capital stock of the Corporation in such form as may from time to time be prescribed by the Board of Directors. Such certificate shall be signed by any two authorized officers of the Corporation. The Corporation seal and the signatures by the Corporation's officers, the transfer agent or the registrar may be facsimiles. In case any officer, transfer agent or registrar who has signed or whose facsimile signature has been placed on such certificate shall have ceased to be such officer, transfer agent or registrar before such certificate is issued, it may be issued by the Corporation with the same effect as if he or she were such officer, transfer agent or registrar at the time of its issue. Every certificate for shares of stock which are subject to any restriction on transfer and every certificate issued when the Corporation is authorized to issue more than one class or series of stock shall contain such legend with respect thereto as is required by law. Notwithstanding anything to the contrary provided in these Bylaws, the Board of Directors of the Corporation may provide by resolution or resolutions that some or all of any or all classes or series of its stock shall be uncertificated shares (except that the foregoing shall not apply to shares represented by a certificate until such certificate is surrendered to the Corporation), and by the approval and adoption of these Bylaws the Board of Directors has determined that all classes or series of the Corporation's stock may be uncertificated, whether upon original issuance, re-issuance, or subsequent transfer.

SECTION 2. Transfers. Subject to any restrictions on transfer and unless otherwise provided by the Board of Directors, shares of stock that are represented by a certificate may be transferred on the books of the Corporation by the surrender to the Corporation or its transfer agent of the certificate theretofore properly endorsed or accompanied by a written assignment or power of attorney properly executed, with transfer stamps (if necessary) affixed, and with such proof of the authenticity of signature as the Corporation or its transfer agent may reasonably require. Shares of stock that are not represented by a certificate may be transferred on the books of the Corporation by submitting to the Corporation or its transfer agent such evidence of transfer and following such other procedures as the Corporation or its transfer agent may require.

SECTION 3. Record Holders. Except as may otherwise be required by law, by the Certificate or by these By-laws, the Corporation shall be entitled to treat the record holder of stock as shown on its books as the owner of such stock for all purposes, including the payment of dividends and the right to vote with respect thereto, regardless of any transfer, pledge or other disposition of such stock, until the shares have been transferred on the books of the Corporation in accordance with the requirements of these By-laws.

SECTION 4. Record Date. In order that the Corporation may determine the stockholders entitled to notice of or to vote at any meeting of stockholders or any adjournment thereof or entitled to receive payment of any dividend or other distribution or allotment of any rights, or entitled to exercise any rights in respect of any change, conversion or exchange of stock or for the purpose of any other lawful action, the Board of Directors may fix a record date,

which record date shall not precede the date upon which the resolution fixing the record date is adopted by the Board of Directors, and which record date: (a) in the case of determination of stockholders entitled to vote at any meeting of stockholders, shall, unless otherwise required by law, not be more than sixty (60) nor less than ten (10) days before the date of such meeting and (b) in the case of any other action, shall not be more than sixty (60) days prior to such other action. If no record date is fixed: (i) the record date for determining stockholders entitled to notice of or to vote at a meeting of stockholders shall be at the close of business on the day next preceding the day on which notice is given, or, if notice is waived, at the close of business on the day next preceding the day on which the meeting is held; and (ii) the record date for determining stockholders for any other purpose shall be at the close of business on the day on which the Board of Directors adopts the resolution relating thereto.

SECTION 5. Replacement of Certificates. In case of the alleged loss, destruction or mutilation of a certificate of stock of the Corporation, a duplicate certificate may be issued in place thereof, upon such terms as the Board of Directors may prescribe.

ARTICLE V

Indemnification

SECTION 1. Definitions. For purposes of this Article:

(a) "Corporate Status" describes the status of a person who is serving or has served (i) as a Director of the Corporation, (ii) as an Officer of the Corporation, (iii) as a Non-Officer Employee of the Corporation, or (iv) as a director, partner, trustee, officer, employee or agent of any other corporation, partnership, limited liability company, joint venture, trust, employee benefit plan, foundation, association, organization or other legal entity which such person is or was serving at the request of the Corporation. For purposes of this Section 1(a), a Director, Officer or Non-Officer Employee of the Corporation who is serving or has served as a director, partner, trustee, officer, employee or agent of a Subsidiary shall be deemed to be serving at the request of the Corporation. Notwithstanding the foregoing, "Corporate Status" shall not include the status of a person who is serving or has served as a director, officer, employee or agent of a constituent corporation absorbed in a merger or consolidation transaction with the Corporation with respect to such person's activities prior to said transaction, unless specifically authorized by the Board of Directors or the stockholders of the Corporation;

(b) "Director" means any person who serves or has served the Corporation as a director on the Board of Directors of the Corporation;

(c) "Disinterested Director" means, with respect to each Proceeding in respect of which indemnification is sought hereunder, a Director of the Corporation who is not and was not a party to such Proceeding;

(d) "Expenses" means all attorneys' fees, retainers, court costs, transcript costs, fees of expert witnesses, private investigators and professional advisors (including, without limitation, accountants and investment bankers), travel expenses, duplicating costs, printing and

binding costs, costs of preparation of demonstrative evidence and other courtroom presentation aids and devices, costs incurred in connection with document review, organization, imaging and computerization, telephone charges, postage, delivery service fees, and all other disbursements, costs or expenses of the type customarily incurred in connection with prosecuting, defending, preparing to prosecute or defend, investigating, being or preparing to be a witness in, settling or otherwise participating in, a Proceeding;

(e) "Liabilities" means judgments, damages, liabilities, losses, penalties, excise taxes, fines and amounts paid in settlement;

(f) "Non-Officer Employee" means any person who serves or has served as an employee or agent of the Corporation, but who is not or was not a Director or Officer;

(g) "Officer" means any person who serves or has served the Corporation as an officer of the Corporation appointed by the Board of Directors of the Corporation;

(h) "Proceeding" means any threatened, pending or completed action, suit, arbitration, alternate dispute resolution mechanism, inquiry, investigation, administrative hearing or other proceeding, whether civil, criminal, administrative, arbitrate or investigative; and

(i) "Subsidiary" shall mean any corporation, partnership, limited liability company, joint venture, trust or other entity of which the Corporation owns (either directly or through or together with another Subsidiary of the Corporation) either (i) a general partner, managing member or other similar interest or (ii) (A) fifty percent (50%) or more of the voting power of the voting capital equity interests of such corporation, partnership, limited liability company, joint venture or other entity, or (B) fifty percent (50%) or more of the outstanding voting capital stock or other voting equity interests of such corporation, partnership, limited liability company, joint venture or other entity.

SECTION 2. Indemnification of Directors and Officers.

(a) Subject to the operation of Section 4 of this Article V of these By-laws, each Director and Officer shall be indemnified and held harmless by the Corporation to the fullest extent authorized by the DGCL, as the same exists or may hereafter be amended (but, in the case of any such amendment, only to the extent that such amendment permits the Corporation to provide broader indemnification rights than such law permitted the Corporation to provide prior to such amendment), and to the extent authorized in this Section 2.

(1) Actions, Suits and Proceedings Other than By or In the Right of the Corporation. Each Director and Officer shall be indemnified and held harmless by the Corporation against any and all Expenses and Liabilities that are incurred or paid by such Director or Officer or on such Director's or Officer's behalf in connection with any Proceeding or any claim, issue or matter therein (other than an action by or in the right of the Corporation), which such Director or Officer is, or is threatened to be made, a party to or participant in by reason of such Director's or Officer's Corporate Status, if such Director or Officer acted in good faith and in a manner such Director or Officer reasonably believed to be in or not opposed to the best interests of the Corporation and,

with respect to any criminal proceeding, had no reasonable cause to believe his or her conduct was unlawful.

(2) Actions, Suits and Proceedings By or In the Right of the Corporation. Each Director and Officer shall be indemnified and held harmless by the Corporation against any and all Expenses that are incurred by such Director or Officer or on such Director's or Officer's behalf in connection with any Proceeding or any claim, issue or matter therein by or in the right of the Corporation, which such Director or Officer is, or is threatened to be made, a party to or participant in by reason of such Director's or Officer's Corporate Status, if such Director or Officer acted in good faith and in a manner such Director or Officer reasonably believed to be in or not opposed to the best interests of the Corporation; provided, however, that no indemnification shall be made under this Section 2(a)(2) in respect of any claim, issue or matter as to which such Director or Officer shall have been finally adjudged by a court of competent jurisdiction to be liable to the Corporation, unless, and only to the extent that, the Court of Chancery or another court in which such Proceeding was brought shall determine upon application that, despite adjudication of liability, but in view of all the circumstances of the case, such Director or Officer is fairly and reasonably entitled to indemnification for such Expenses that such court deems proper.

(3) Survival of Rights. The rights of indemnification provided by this Section 2 shall continue as to a Director or Officer after he or she has ceased to be a Director or Officer and shall inure to the benefit of his or her heirs, executors, administrators and personal representatives.

(4) Actions by Directors or Officers. Notwithstanding the foregoing, the Corporation shall indemnify any Director or Officer seeking indemnification in connection with a Proceeding initiated by such Director or Officer only if such Proceeding (including any parts of such Proceeding not initiated by such Director or Officer) was authorized in advance by the Board of Directors of the Corporation, unless such Proceeding was brought to enforce such Officer's or Director's rights to indemnification or, in the case of Directors, advancement of Expenses under these By-laws in accordance with the provisions set forth herein.

SECTION 3. Indemnification of Non-Officer Employees. Subject to the operation of Section 4 of this Article V of these By-laws, each Non-Officer Employee may, in the discretion of the Board of Directors of the Corporation, be indemnified by the Corporation to the fullest extent authorized by the DGCL, as the same exists or may hereafter be amended, against any or all Expenses and Liabilities that are incurred by such Non-Officer Employee or on such Non-Officer Employee's behalf in connection with any threatened, pending or completed Proceeding, or any claim, issue or matter therein, which such Non-Officer Employee is, or is threatened to be made, a party to or participant in by reason of such Non-Officer Employee's Corporate Status, if such Non-Officer Employee acted in good faith and in a manner such Non-Officer Employee reasonably believed to be in or not opposed to the best interests of the Corporation and, with respect to any criminal proceeding, had no reasonable cause to believe his or her conduct was unlawful. The rights of indemnification provided by this Section 3 shall exist as to a Non-

Officer Employee after he or she has ceased to be a Non-Officer Employee and shall inure to the benefit of his or her heirs, personal representatives, executors and administrators. Notwithstanding the foregoing, the Corporation may indemnify any Non-Officer Employee seeking indemnification in connection with a Proceeding initiated by such Non-Officer Employee only if such Proceeding was authorized in advance by the Board of Directors of the Corporation.

SECTION 4. Determination. Unless ordered by a court, no indemnification shall be provided pursuant to this Article V to a Director, to an Officer or to a Non-Officer Employee unless a determination shall have been made that such person acted in good faith and in a manner such person reasonably believed to be in or not opposed to the best interests of the Corporation and, with respect to any criminal Proceeding, such person had no reasonable cause to believe his or her conduct was unlawful. Such determination shall be made by (a) a majority vote of the Disinterested Directors, even though less than a quorum of the Board of Directors, (b) a committee comprised of Disinterested Directors, such committee having been designated by a majority vote of the Disinterested Directors (even though less than a quorum), (c) if there are no such Disinterested Directors, or if a majority of Disinterested Directors so directs, by independent legal counsel in a written opinion, or (d) by the stockholders of the Corporation.

SECTION 5. Advancement of Expenses to Directors Prior to Final Disposition.

(a) The Corporation shall advance all Expenses incurred by or on behalf of any Director in connection with any Proceeding in which such Director is involved by reason of such Director's Corporate Status within thirty (30) days after the receipt by the Corporation of a written statement from such Director requesting such advance or advances from time to time, whether prior to or after final disposition of such Proceeding. Such statement or statements shall reasonably evidence the Expenses incurred by such Director and shall be preceded or accompanied by an undertaking by or on behalf of such Director to repay any Expenses so advanced if it shall ultimately be determined that such Director is not entitled to be indemnified against such Expenses. Notwithstanding the foregoing, the Corporation shall advance all Expenses incurred by or on behalf of any Director seeking advancement of expenses hereunder in connection with a Proceeding initiated by such Director only if such Proceeding (including any parts of such Proceeding not initiated by such Director) was (i) authorized by the Board of Directors of the Corporation, or (ii) brought to enforce such Director's rights to indemnification or advancement of Expenses under these By-laws.

(b) If a claim for advancement of Expenses hereunder by a Director is not paid in full by the Corporation within thirty (30) days after receipt by the Corporation of documentation of Expenses and the required undertaking, such Director may at any time thereafter bring suit against the Corporation to recover the unpaid amount of the claim and if successful in whole or in part, such Director shall also be entitled to be paid the expenses of prosecuting such claim. The failure of the Corporation (including its Board of Directors or any committee thereof, independent legal counsel, or stockholders) to make a determination concerning the permissibility of such advancement of Expenses under this Article V shall not be a defense to an action brought by a Director for recovery of the unpaid amount of an advancement claim and

shall not create a presumption that such advancement is not permissible. The burden of proving that a Director is not entitled to an advancement of expenses shall be on the Corporation.

(c) In any suit brought by the Corporation to recover an advancement of expenses pursuant to the terms of an undertaking, the Corporation shall be entitled to recover such expenses upon a final adjudication that the Director has not met any applicable standard for indemnification set forth in the DGCL.

SECTION 6. Advancement of Expenses to Officers and Non-Officer Employees Prior to Final Disposition.

(a) The Corporation may, at the discretion of the Board of Directors of the Corporation, advance any or all Expenses incurred by or on behalf of any Officer or any Non-Officer Employee in connection with any Proceeding in which such person is involved by reason of his or her Corporate Status as an Officer or Non-Officer Employee upon the receipt by the Corporation of a statement or statements from such Officer or Non-Officer Employee requesting such advance or advances from time to time, whether prior to or after final disposition of such Proceeding. Such statement or statements shall reasonably evidence the Expenses incurred by such Officer or Non-Officer Employee and shall be preceded or accompanied by an undertaking by or on behalf of such person to repay any Expenses so advanced if it shall ultimately be determined that such Officer or Non-Officer Employee is not entitled to be indemnified against such Expenses.

(b) In any suit brought by the Corporation to recover an advancement of expenses pursuant to the terms of an undertaking, the Corporation shall be entitled to recover such expenses upon a final adjudication that the Officer or Non-Officer Employee has not met any applicable standard for indemnification set forth in the DGCL.

SECTION 7. Contractual Nature of Rights.

(a) The provisions of this Article V shall be deemed to be a contract between the Corporation and each Director and Officer entitled to the benefits hereof at any time while this Article V is in effect, in consideration of such person's past or current and any future performance of services for the Corporation. Neither amendment, repeal or modification of any provision of this Article V nor the adoption of any provision of the Certificate of Incorporation inconsistent with this Article V shall eliminate or reduce any right conferred by this Article V in respect of any act or omission occurring, or any cause of action or claim that accrues or arises or any state of facts existing, at the time of or before such amendment, repeal, modification or adoption of an inconsistent provision (even in the case of a proceeding based on such a state of facts that is commenced after such time), and all rights to indemnification and advancement of Expenses granted herein or arising out of any act or omission shall vest at the time of the act or omission in question, regardless of when or if any proceeding with respect to such act or omission is commenced. The rights to indemnification and to advancement of expenses provided by, or granted pursuant to, this Article V shall continue notwithstanding that the person has ceased to be a director or officer of the Corporation and shall inure to the benefit of the estate, heirs, executors, administrators, legatees and distributees of such person.

(b) If a claim for indemnification hereunder by a Director or Officer is not paid in full by the Corporation within sixty (60) days after receipt by the Corporation of a written claim for indemnification, such Director or Officer may at any time thereafter bring suit against the Corporation to recover the unpaid amount of the claim, and if successful in whole or in part, such Director or Officer shall also be entitled to be paid the expenses of prosecuting such claim. The failure of the Corporation (including its Board of Directors or any committee thereof, independent legal counsel, or stockholders) to make a determination concerning the permissibility of such indemnification under this Article V shall not be a defense to an action brought by a Director or Officer for recovery of the unpaid amount of an indemnification claim and shall not create a presumption that such indemnification is not permissible. The burden of proving that a Director or Officer is not entitled to indemnification shall be on the Corporation.

(c) In any suit brought by a Director or Officer to enforce a right to indemnification hereunder, it shall be a defense that such Director or Officer has not met any applicable standard for indemnification set forth in the DGCL.

SECTION 8. Non-Exclusivity of Rights. The rights to indemnification and to advancement of Expenses set forth in this Article V shall not be exclusive of any other right which any Director, Officer, or Non-Officer Employee may have or hereafter acquire under any statute, provision of the Certificate or these By-laws, agreement, vote of stockholders or Disinterested Directors or otherwise.

SECTION 9. Insurance. The Corporation may maintain insurance, at its expense, to protect itself and any Director, Officer or Non-Officer Employee against any liability of any character asserted against or incurred by the Corporation or any such Director, Officer or Non-Officer Employee, or arising out of any such person's Corporate Status, whether or not the Corporation would have the power to indemnify such person against such liability under the DGCL or the provisions of this Article V.

SECTION 10. Other Indemnification. The Corporation's obligation, if any, to indemnify or provide advancement of Expenses to any person under this Article V as a result of such person serving, at the request of the Corporation, as a director, partner, trustee, officer, employee or agent of another corporation, partnership, joint venture, trust, employee benefit plan or other enterprise shall be reduced by any amount such person may collect as indemnification or advancement of Expenses from such other corporation, partnership, joint venture, trust, employee benefit plan or enterprise (the "Primary Indemnitor"). Any indemnification or advancement of Expenses under this Article V owed by the Corporation as a result of a person serving, at the request of the Corporation, as a director, partner, trustee, officer, employee or agent of another corporation, partnership, joint venture, trust, employee benefit plan or other enterprise shall only be in excess of, and shall be secondary to, the indemnification or advancement of Expenses available from the applicable Primary Indemnitor(s) and any applicable insurance policies.

ARTICLE VI

Miscellaneous Provisions

SECTION 1. Fiscal Year. The fiscal year of the Corporation shall be determined by the Board of Directors.

SECTION 2. Seal. The Board of Directors shall have power to adopt and alter the seal of the Corporation.

SECTION 3. Execution of Instruments. All deeds, leases, transfers, contracts, bonds, notes and other obligations to be entered into by the Corporation in the ordinary course of its business without director action may be executed on behalf of the Corporation by the Chairman of the Board, if one is elected, the President or the Treasurer or any other officer, employee or agent of the Corporation as the Board of Directors or the executive committee of the Board may authorize.

SECTION 4. Voting of Securities. Unless the Board of Directors otherwise provides, the Chairman of the Board, if one is elected, the President or the Treasurer may waive notice of and act on behalf of the Corporation (including with regard to voting and actions by written consent), or appoint another person or persons to act as proxy or attorney in fact for the Corporation with or without discretionary power and/or power of substitution, at any meeting of stockholders or shareholders of any other corporation or organization, any of whose securities are held by the Corporation.

SECTION 5. Resident Agent. The Board of Directors may appoint a resident agent upon whom legal process may be served in any action or proceeding against the Corporation.

SECTION 6. Corporate Records. The original or attested copies of the Certificate, By-laws and records of all meetings of the incorporators, stockholders and the Board of Directors and the stock transfer books, which shall contain the names of all stockholders, their record addresses and the amount of stock held by each, may be kept outside the State of Delaware and shall be kept at the principal office of the Corporation, at an office of its counsel, at an office of its transfer agent or at such other place or places as may be designated from time to time by the Board of Directors.

SECTION 7. Certificate. All references in these By-laws to the Certificate shall be deemed to refer to the Amended and Restated Certificate of Incorporation of the Corporation, as amended and/or restated and in effect from time to time.

SECTION 8. Exclusive Jurisdiction of Delaware Courts. Unless the Corporation consents in writing to the selection of an alternative forum, the Court of Chancery of the State of Delaware shall be the sole and exclusive forum for (i) any derivative action or proceeding brought on behalf of the Corporation, (ii) any action asserting a claim of breach of a fiduciary

duty owed by any director, officer, stockholder or other employee of the Corporation to the Corporation or the Corporation's stockholders, (iii) any action asserting a claim arising pursuant to any provision of the Delaware General Corporation Law or the Certificate or By-laws, or (iv) any action asserting a claim against the Corporation governed by the internal affairs doctrine. Any person or entity purchasing or otherwise acquiring any interest in shares of capital stock of the Corporation shall be deemed to have notice of and consented to the provisions of this Section 8.

SECTION 9. Amendment of By-laws.

(a) Amendment by Directors. Except as provided otherwise by law, these By-laws may be amended or repealed by the Board of Directors by the affirmative vote of a majority of the directors then in office.

(b) Amendment by Stockholders. These By-laws may be amended or repealed at any Annual Meeting, or special meeting of stockholders called for such purpose in accordance with these By-Laws, by the affirmative vote of at least seventy-five percent (75%) of the voting power of the outstanding shares entitled to vote on such amendment or repeal, voting together as a single class; provided, however, that if the Board of Directors recommends that stockholders approve such amendment or repeal at such meeting of stockholders, such amendment or repeal shall only require the affirmative vote of the majority of the voting power of the outstanding shares entitled to vote on such amendment or repeal, voting together as a single class. Notwithstanding the foregoing, stockholder approval shall not be required unless mandated by the Certificate, these By-laws, or other applicable law.

SECTION 10. Notices. If mailed, notice to stockholders shall be deemed given when deposited in the mail, postage prepaid, directed to the stockholder at such stockholder's address as it appears on the records of the Corporation. Without limiting the manner by which notice otherwise may be given to stockholders, any notice to stockholders may be given by electronic transmission in the manner provided in Section 232 of the DGCL.

SECTION 11. Waivers. A written waiver of any notice, signed by a stockholder or director, or waiver by electronic transmission by such person, whether given before or after the time of the event for which notice is to be given, shall be deemed equivalent to the notice required to be given to such person. Neither the business to be transacted at, nor the purpose of, any meeting need be specified in such a waiver.

Adopted October 5, 2016, subject to and effective upon the closing of the Corporation's initial public offering on its Registration Statement on Form S-1.

**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 29, 2016

/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 29, 2016

/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, 2016, 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 29, 2016

/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
