Akebia Therapeutics, Inc.
Form 10-Q
November 09, 2016

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 001-36352

AKEBIA THERAPEUTICS, INC.

(Exact Name of Registrant as Specified in Its Charter)

Delaware (State or Other Jurisdiction of Incorporation or Organization)	20-8756903 (I.R.S. Employer Identification No.)
245 First Street, Suite 1100, Cambridge, MA (Address of Principal Executive Offices)	02142 (Zip Code)

(617) 871-2098

(Registrant’s Telephone Number, Including Area Code)

(Former Name, Former Address and Former Fiscal Year, if Changed Since Last Report)

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 Date File required to be submitted and posted pursuant to Rule 405 of Regulation S-T (§232.405 of this chapter) during the preceding 12 months (or for such shorter period that the registrant was required to submit and post such files). Yes No

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

Large accelerated filer Accelerated filer

Non-accelerated filer Smaller reporting company

Indicate by check mark whether registrant is a shell company (as defined in Rule 12b-2 of the Exchange Act). Yes No

Indicate the number of shares outstanding of each of the issuer’s classes of common stock, as of the latest practicable date.

Class	Outstanding at October 31, 2016
Common Stock, $0.00001 par value	38,324,472

NOTE REGARDING FORWARD-LOOKING STATEMENTS

This Quarterly Report on Form 10-Q contains forward-looking statements that are being made pursuant to the safe harbor provisions of the Private Securities Litigation Reform Act of 1995, or PSLRA, with the intention of obtaining the benefits of the "safe harbor" provisions of the PSLRA. Forward-looking statements involve risks and uncertainties. All statements other than statements of historical facts contained in this Quarterly Report on Form 10-Q are forward-looking statements. In some cases, you can identify forward-looking statements by words such as "anticipate," "believe," "contemplate," "continue," "could," "estimate," "expect," "intend," "may," "plan," "potential," "predict," "project," "target," "will," "would," or the negative of these words or other comparable terminology. These forward-looking statements include, but are not limited to, statements about:

- the projected timing of (1) our PRO_2TECT and $INNO_2VATE$ clinical programs, (2) submission of a new drug application, or NDA, for vadadustat, and (3) commencement of Phase 1 clinical studies of AKB-6899;
- the pace of enrollment for the PRO_2TECT and $INNO_2VATE$ clinical programs;
- our plans to seek a geographic collaboration for the development and commercialization of vadadustat outside the United States;
- our development plans with respect to vadadustat and AKB-6899;
- the timing or likelihood of regulatory filings and approvals, including any required post-marketing testing or any labeling and other restrictions;
- our plans to commercialize vadadustat, if it is approved;
- the implementation of our business model and strategic plans for our business, product candidates and technology;
- our commercialization, marketing and manufacturing capabilities and strategy;
- our competitive position;
- our intellectual property position;
- developments and projections relating to our competitors and our industry;
- our estimates regarding expenses (including those associated with the PRO_2TECT and $INNO_2VATE$ clinical programs), future revenue, capital requirements and needs for additional financing; and
- other risks and uncertainties, including those listed under Part II, Item 1A. Risk Factors.

All forward-looking statements in this Quarterly Report on Form 10-Q involve known and unknown risks, uncertainties and other factors that may cause our actual results, performance or achievements to be materially different from any future results, performance or achievements expressed or implied by these forward-looking statements. Factors that may cause actual results to differ materially from current expectations include, among other things, those listed under Part II, Item 1A. Risk Factors and elsewhere in this Quarterly Report on Form 10-Q. Given these uncertainties, you should not place undue reliance on these forward-looking statements. Except as required by law, we assume no obligation to update or revise these forward-looking statements for any reason, even if new information becomes available in the future.

This Quarterly Report on Form 10-Q also contains estimates, projections and other information concerning our industry, our business, and the markets for certain diseases, including data regarding the estimated size of those markets, and the incidence and prevalence of certain medical conditions. Information that is based on estimates, forecasts, projections, market research or similar methodologies is inherently subject to uncertainties and actual events or circumstances may differ materially from events and circumstances reflected in this information. Unless otherwise expressly stated, we obtained this industry, business, market and other data from reports, research surveys, studies and similar data prepared by market research firms and other third parties, industry, medical and general publications, government data and similar sources.

Akebia Therapeutics, Inc.

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PART I—FINANCIAL INFORMATION

ITEM 1. FINANCIAL STATEMENTS.

AKEBIA THERAPEUTICS, 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	$ 65,830	$ 49,778
Available for sale securities	95,492	88,676
Prepaid expenses and other current assets	3,825	2,563
Total current assets	165,147	141,017
Property and equipment, net	2,688	540
Deferred offering costs	—	102
Other assets	1,283	1,281
Total assets	$ 169,118	$ 142,940
Liabilities and stockholders' equity		
Current liabilities:		
Accounts payable	$ 8,524	$ 2,313
Accrued expenses	17,615	9,555
Total current liabilities	26,139	11,868
Deferred rent	2,262	69
Deferred revenue	40,000	—
Other non-current liabilities	7	5
Total liabilities	68,408	11,942
Commitments and contingencies (Note 8)		
Stockholders' equity:		
Preferred stock $0.00001 par value, 25,000,000 shares authorized at September 30, 2016 and December 31, 2015; 0 shares issued and outstanding at September 30, 2016 and December 31, 2015	—	—
Common stock: $0.00001 par value; 175,000,000 shares authorized at September 30, 2016 and December 31, 2015; 38,253,190 and 30,662,218 shares issued and outstanding at September 30, 2016 and December 31, 2015, respectively	—	—
Additional paid-in capital	359,991	292,783
Treasury stock, at cost, 0 shares in 2016, 8,643 shares in 2015	—	(162)
Accumulated other comprehensive loss	(7)	(234)
Accumulated deficit	(259,274)	(161,389)
Total stockholders' equity	100,710	130,998
Total liabilities and stockholders' equity	$ 169,118	$ 142,940

See accompanying notes to unaudited condensed consolidated financial statements.

AKEBIA THERAPEUTICS, INC.

Condensed Consolidated Statements of Operations and Comprehensive Loss

(Unaudited)

(in thousands, except share and per share data)

	Three Months Ended September 30,		Nine Months Ended September 30,	
	2016	2015	2016	2015
Operating expenses:				
Research and development	$31,238	$15,604	$82,350	$28,772
General and administrative	4,944	4,074	16,066	12,691
Total operating expenses	36,182	19,678	98,416	41,463
Operating loss	(36,182)	(19,678)	(98,416)	(41,463)
Other income (expense):				
Interest income	219	139	722	350
Other income (expense)	(345)	64	(191)	254
Net loss	$(36,308)	$(19,475)	$(97,885)	$(40,859)
Net loss per share applicable to common stockholders - basic and diluted	$(0.96)	$(0.68)	$(2.61)	$(1.62)
Weighted-average number of common shares used in net loss per share applicable to common stockholders—basic and diluted	37,897,902	28,784,231	37,528,869	25,175,077
Comprehensive loss:				
Net loss	$(36,308)	$(19,475)	$(97,885)	$(40,859)
Other comprehensive income (loss) - unrealized gain (loss) on securities	(44)	20	(7)	(7)
Comprehensive loss	$(36,352)	$(19,455)	$(97,892)	$(40,866)

See accompanying notes to unaudited condensed consolidated financial statements.

AKEBIA THERAPEUTICS, INC.

Condensed Consolidated Statements of Cash Flows

(Unaudited)

(in thousands)

	Nine Months Ended	
	September 30, 2016	September 30, 2015
Operating activities:		
Net loss	$(97,885)	$ (40,859)
Adjustments to reconcile net loss to net cash used in operating activities:		
Depreciation and amortization	172	85
Amortization of premium/discount on investments	399	418
Loss on disposal of property and equipment	306	—
Stock-based compensation	4,101	3,386
Changes in operating assets and liabilities:		
Prepaid expenses and other current assets	(1,263)	224
Accounts payable	6,196	3,252
Accrued expense	8,017	4,024
Deferred revenue	40,000	—
Deferred rent	2,193	(25)
Net cash used in operating activities	(37,764)	(29,495)
Investing activities:		
Purchase of equipment	(2,614)	(342)
Proceeds from the maturities of available for sale securities	125,151	49,547
Purchase of available for sale securities	(132,138)	(64,531)
Net cash used in investing activities	(9,601)	(15,326)
Financing activities:		
Proceeds from the issuance of common stock, net of issuance costs	63,205	78,579
Proceeds from the sale of stock under employee stock purchase plan	106	109
Proceeds from the exercise of stock options	125	70
Payments on capital lease obligations	(19)	(5)
Net cash provided by financing activities	63,417	78,753
Decrease in cash and cash equivalents	16,052	33,932
Cash and cash equivalents at beginning of the period	49,778	32,780
Cash and cash equivalents at end of the period	$65,830	$ 66,712
Non-cash financing activities		
Unpaid follow-on offering costs	$65	$ —
Assets acquired under capital lease	$—	$ 12

See accompanying notes to unaudited condensed consolidated financial statements

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Akebia Therapeutics, Inc.

Notes to Condensed Consolidated Financial Statements

(Unaudited)

September 30, 2016

1. Nature of Organization and Operations

Incorporated in Delaware in 2007, Akebia Therapeutics, Inc. (Akebia, or the Company) is a biopharmaceutical company focused on the development of novel proprietary therapeutics based on hypoxia inducible factor, or HIF, biology and the commercialization of these products for patients with serious unmet medical needs. HIF is the primary regulator of the production of red blood cells, or RBCs, in the body and a potentially novel mechanism for the treatment of anemia secondary to chronic kidney disease, or CKD. Pharmacologic modulation of the HIF pathway may also have broader therapeutic applications in acute renal failure, organ protection, ischemia-reperfusion injury, cancer, ophthalmology, and inflammatory diseases. The Company's lead product candidate, vadadustat, is being developed as a once-daily, oral therapy for the treatment of anemia of CKD. The Company has successfully completed Phase 2 development demonstrating that vadadustat can safely and predictably raise hemoglobin levels in patients with anemia related to CKD. The Company has commenced its vadadustat Phase 3 program, which includes the PRO_2TECT studies for non-dialysis patients with anemia secondary to CKD and $INNO_2VATE$ studies for dialysis-dependent patients.

The Company's operations to date have been limited to organizing and staffing the Company, business planning, raising capital, acquiring and developing its technology, identifying potential product candidates and undertaking preclinical and clinical studies. The Company has not generated any product revenue to date and may never generate any product revenue in the future. The Company's product candidates are subject to long development cycles and the Company may be unsuccessful in its efforts to develop, obtain regulatory approval for or market its product candidates.

The Company is subject to a number of risks including, but not limited to, the need to obtain adequate additional funding, including the resources necessary to fund its recently commenced global Phase 3 development of vadadustat in non-dialysis and dialysis patients with anemia related to CKD. In December 2015, the Company began dosing patients in its Phase 3 vadadustat program in non-dialysis patients, PRO_2TECT. The Company initiated its Phase 3 program of vadadustat in dialysis-dependent patients, $INNO_2VATE$, in August 2016 and anticipates full enrollment by early 2018. The Company has engaged a clinical research organization for the PRO_2TECT and $INNO_2VATE$ programs. The Company expects the cost of the Phase 3 program to be in the range of $80,000 to $85,000 per patient, and it plans to enroll approximately 3,100 patients in PRO_2TECT and approximately 2,600 patients in $INNO_2VATE$.

The Company is also subject to a number of other risks including possible failure of preclinical testing or clinical trials, the need to obtain marketing approval for its product candidates, the development of new technological innovations by competitors, the need to successfully commercialize and gain market acceptance of any of the Company's products that are approved and uncertainty around intellectual property matters. If the Company does not successfully commercialize any of its products, it will be unable to generate product revenue or achieve profitability.

In December 2015, the Company entered into a collaboration agreement with Mitsubishi Tanabe Pharma Corporation, or MTPC, to develop and commercialize vadadustat in Japan and certain other countries in Asia for total milestone payments of up to $350.0 million, including up to $100.0 million in upfront and development payments, of which $40.0 million was received in January 2016. If Japanese patients are not included in either the global Phase 3

PRO$_2$TECT or INNO$_2$VATE programs, $20.0 million of the $40.0 million received would be used to fund further local development of vadadustat in Japan or be refunded to MTPC. In addition, the Company is also eligible to receive tiered double-digit royalty payments on sales of vadadustat.

Through the end of 2015 the Company had raised approximately $187.4 million of net proceeds from three underwritten public offerings, including our initial public offering. In January 2016, the Company completed a follow-on public offering whereby the Company sold 7,250,000 shares of common stock at a price of $9.00 per share. The aggregate net proceeds received by the Company from the offering were approximately $61.0 million, net of underwriting discounts and commissions and estimated offering expenses payable by the Company.

In May 2016, the Company entered into a Sales Agreement with Cantor Fitzgerald & Co. to periodically sell up to $75.0 million of shares of common stock in an at-the-market, or ATM, offering. During the third quarter of 2016, the Company sold 239,906 shares of common stock pursuant to the Sales Agreement. The aggregate net proceeds received by the Company were approximately $2.2 million, net of commissions.

The Company believes that its cash, cash equivalents and available for sale securities of $161.3 million as of September 30, 2016 is sufficient to fund its current operating plan through the second quarter of 2017. There can be no assurance, however, that the current operating plan will be achieved in the timeframe anticipated by the Company, or that its cash resources will fund the Company’s

operating plan for the period anticipated by the Company or that additional funding will be available on terms acceptable to the Company, or at all.

As of September 30, 2016, the Company had cash and cash equivalents of approximately $161.3 million and current accounts payable and accrued expenses of $26.1 million. The Company has incurred substantial losses since inception, primarily due to investments in research and development, and we expect to continue to incur substantial losses over the next four to five years. Without any additional financings or other transactions, the Company anticipates that it will have sufficient cash available to support its development programs and business operations through the second quarter of 2017.

The accompanying financial statements have been prepared assuming that the Company will continue as a going concern, which contemplates the realization of assets and satisfaction of liabilities in the normal course of business. In the future, the Company's ability to continue as a going concern is dependent on its ability to raise additional capital to fund its research and development programs and meet its obligations on a timely basis. As of September 30, 2016, the financial statements do not include any adjustments relating to recoverability and classification of recorded asset amounts or the amounts and classification of liabilities that might be necessary should the Company be unable to continue its existence.

To potentially mitigate the risk that the Company may be unable to continue as a going concern, it plans to pursue all or a combination of potential strategic alliances, collaborations and other strategic transactions. The Company also may seek additional capital through public or private equity offerings (including its ATM offering), which could have a dilutive impact on stockholders and the issuance, or even potential issuance, of shares could have a negative effect on the market price of common stock. Even if the Company is able to secure additional capital, such financings may only be available on unattractive terms, or could result in significant dilution of stockholders' interests. If none of the foregoing alternatives is available or, if available, the Company is unable to raise sufficient capital through such transactions, it may be forced to limit or cease its development activities.

2. Summary of Significant Accounting Policies

Basis of Presentation

The accompanying condensed consolidated financial statements include the accounts of the Company and its wholly-owned subsidiaries, Akebia Therapeutics Securities Corporation and Akebia Europe Limited. All intercompany balances and transactions have been eliminated in consolidation. These condensed consolidated financial statements have been prepared in conformity with accounting principles generally accepted in the United States of America (U.S. GAAP). Any reference in these notes to applicable guidance is meant to refer to the authoritative U.S. GAAP as found in the Accounting Standards Codification (ASC) and Accounting Standards Update (ASU) of the Financial Accounting Standards Board (FASB).

In the quarter ended December 31, 2015, the Company identified and corrected an error in the historical classification of certain operating costs between research and development and general and administrative expenses. The Company

concluded the effect of this classification error was not material to its consolidated financial statements for any prior period. The classification correction had no effect on the Company's current or historical total operating expenses or net loss.

The results of operations for the interim periods are not necessarily indicative of the results of operations to be expected for the full year. These interim condensed consolidated financial statements should be read in conjunction with the audited consolidated financial statements as of and for the year ended December 31, 2015, and the notes thereto, which are included in the Company's Annual Report on Form 10-K (File No. 001-36352), which was filed with the Securities and Exchange Commission (SEC) on March 14, 2016.

Recent Accounting Pronouncements

From time to time, new accounting pronouncements are issued by the FASB or other standard-setting bodies and adopted by the Company as of the specified effective date. Unless otherwise discussed, the Company believes the impact of recently issued standards that are not yet effective will not have a material impact on its financial position or results of operations upon adoption.

In March 2016, the FASB issued ASU 2016-09, Improvements to Employee Share-Based Payment Accounting, as part of its Simplification Initiative. The areas for simplification in this update involve several aspects of the accounting for share-based payment transactions, including the income tax consequences, classification of awards as either equity or liabilities, and classification on the statement of cash flows. Some of the areas for simplification apply only to nonpublic entities. The amendments in ASU 2016-09 are effective for fiscal years beginning after December 15, 2016, and interim periods within those fiscal years. Early application is permitted for all entities. The Company is currently evaluating the impact of this new standard on its consolidated financial statements.

In February 2016, the FASB issued ASU 2016-02, Leases (Topic 842), which supersedes the existing guidance for lease accounting, Leases (Topic 840). ASU 2016-02 requires lessees to recognize leases on their balance sheets, and leaves lessor accounting largely unchanged. The amendments in this ASU are effective for fiscal years beginning after December 15, 2018 and interim periods within those fiscal years. Early application is permitted for all entities. ASU 2016-02 requires a modified retrospective approach for all leases existing at, or entered into after, the date of initial application, with an option to elect to use certain transition relief. The Company is currently evaluating the impact of this new standard on its consolidated financial statements.

In August 2014, the FASB issued ASU 2014-15, which requires management of public and private companies to evaluate whether there is substantial doubt about the entity's ability to continue as a going concern within one year after the date that the financial statements are issued. Management's evaluation should be based on relevant conditions and events that are known and reasonably knowable at the date that the financial statements are issued. If conditions or events raise substantial doubt about an entity's ability to continue as a going concern, and substantial doubt is not alleviated after consideration of management's plans, an entity should include a statement in the footnotes indicating that there is substantial doubt about the entity's ability to continue as a going concern within one year after the date that the financial statements are issued. The new standard is effective for annual periods ending after December 15, 2016, and for annual periods and interim periods thereafter. Early application is permitted. While the Company intends to adopt the standard as of December 31, 2016, if this standard had been adopted as of September 30, 2016, management of the Company believes that it would have concluded there is substantial doubt about the Company's ability to continue as a going concern one year from the date of filing of this Form 10-Q. See Note 1 for additional information on our liquidity risks and management's plans.

In May 2014, the FASB issued a new revenue recognition standard which amends revenue recognition principles and provides a single, comprehensive set of criteria for revenue recognition within and across all industries. The new standard provides a five step framework whereby revenue is recognized when promised goods or services are transferred to a customer at an amount that reflects the consideration to which the entity expects to be entitled in exchange for those goods or services. The standard also requires enhanced disclosures pertaining to revenue recognition in both interim and annual periods. In August 2015, the FASB deferred the effective date of the new revenue standard from January 1, 2017 to January 1, 2018. Early adoption is permitted any time after the original effective date, which for us is January 1, 2017. The standard allows for adoption using a full retrospective method or a modified retrospective method. The Company is currently evaluating the timing, method of adoption and the expected impact that the standard could have on our consolidated financial statements and related disclosures.

Segment Information

Operating segments are defined as components of an enterprise about which separate discrete information is available for evaluation by the chief operating decision maker, or decision-making group, in deciding how to allocate resources and in assessing performance. The Company views its operations and manages its business in one operating segment, which is the business of developing and commercializing proprietary therapeutics based on HIF biology.

Use of Estimates

The preparation of financial statements in conformity with U.S. GAAP requires management to make estimates and assumptions that affect the reported amounts of assets and liabilities and disclosure of contingent assets and liabilities at the date of the financial statements and the reported amounts of revenues and expenses during the reporting period. Actual results may differ from those estimates. Management considers many factors in selecting appropriate financial accounting policies and controls, and in developing the estimates and assumptions that are used in the preparation of these financial statements. Management must apply significant judgment in this process. In addition, other factors may affect estimates, including: 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. The estimation process often may yield a range of potentially reasonable estimates of the ultimate future outcomes, and management must select an amount that falls within that range of reasonable estimates. Estimates are used in the following areas, among others: prepaid and accrued research and development expense, stock-based compensation expense, accrued expenses and income taxes.

Prior to the initial public offering, the Company utilized significant estimates and assumptions in determining the fair value of its common stock. The Company granted stock options at exercise prices not less than the fair market value of its common stock as determined by the Board of Directors contemporaneously at the date such grants were made, with input from management. Prior to the Company's initial public offering in March 2014, the fair value of common stock at the grant date was adjusted in connection with the Company's retrospective fair value assessment for financial reporting purposes. Accordingly, the Board of Directors determined the estimated fair value of the Company's common stock based on a number of objective and subjective factors, including external market conditions affecting the biotechnology industry sector and the prices at which the Company sold shares of preferred stock, the superior rights and preferences of securities senior to the Company's common stock at the time and the likelihood of achieving a liquidity event, such as an initial public offering or sale of the Company.

Cash and Cash Equivalents

Cash and cash equivalents consist of all cash on hand, deposits and funds invested in available-for-sale securities with original maturities of three months or less at the time of purchase. At September 30, 2016, the Company's cash is primarily in money market funds. The Company may maintain balances with its banks in excess of federally insured limits.

Investments

Management determines the appropriate classification of securities at the time of purchase and reevaluates such designation as of each balance sheet date. Currently, the Company classifies all securities as available-for-sale which are included in current assets as they are intended to fund current operations. The Company carries available-for-sale securities at fair value. The Company conducts periodic reviews to identify and evaluate each investment that has an unrealized loss, in accordance with the meaning of other-than-temporary impairment and its application to certain investments. When assessing whether a decline in the fair value of a security is other-than-temporary, the Company considers the fair market value of the security, the duration of the security's decline, and prospects for the underlying business. Based on these considerations, the Company did not identify any other-than-temporary unrealized losses at September 30, 2016. Unrealized losses on available-for-sale securities that are determined to be temporary, and not related to credit loss, are recorded in accumulated other comprehensive loss, a component of stockholders' equity. The amortized cost of debt securities in this category reflects amortization of premiums and accretion of discounts to maturity computed under the effective interest method. The Company includes this amortization in the caption "Interest income" within the consolidated statements of operations and comprehensive loss. The Company also includes in net investment income, realized gains and losses and declines in value determined to be other than temporary. The Company bases the cost of securities sold upon the specific identification method, and includes interest and dividends on securities in interest income.

Revenue Recognition

To date, the Company has not generated any revenue from the sales of products or other means. For the foreseeable future, the Company expects substantially all of its revenues will be generated from its collaboration with MTPC (see Note 9) and any other collaborations the Company may enter into.

The Company will recognize revenue in accordance with ASC Topic 605, Revenue Recognition (ASC 605). Accordingly, revenue will be recognized for each unit of accounting when all of the following criteria are met:

- Persuasive evidence of an arrangement exists;
- Delivery has occurred or services have been rendered;
- The seller's price to the buyer is fixed or determinable; and
- Collectability is reasonably assured.

Amounts received prior to satisfying the revenue recognition criteria are recorded as deferred revenue. Amounts expected to be recognized as revenue within the 12 months following the balance sheet date are classified in current liabilities. Amounts not expected to be recognized as revenue within the 12 months following the balance sheet date are classified as deferred revenue, net of current portion.

The Company evaluates multiple element arrangements based on the guidance in ASC Topic 605 25, Revenue Recognition Multiple Element Arrangements (ASC 605 25). Pursuant to the guidance in ASC 605 25, the Company evaluates multiple element arrangements to determine (1) the deliverables included in the arrangement and (2) whether the individual deliverables represent separate units of accounting or whether they must be accounted for as a combined unit of accounting. This evaluation involves subjective determinations and requires the Company to make judgments about the individual deliverables and whether such deliverables are separable from the other aspects of the contractual relationship. Deliverables are considered separate units of accounting provided that the delivered item has

value to the customer on a standalone basis and, if the arrangement includes a general right of return relative to the delivered item, delivery or performance of the undelivered item is considered probable and substantially in the Company's control. In assessing whether an item has standalone value, the Company considers factors such as the research, development, manufacturing and commercialization capabilities of the collaboration partner and the availability of the associated expertise in the general marketplace. In addition, the Company considers whether the collaboration partner can use a deliverable for its intended purpose without the receipt of the remaining deliverable, whether the value of the deliverable is dependent on the undelivered item and whether there are other vendors that can provide the undelivered items.

The consideration received under the arrangement that is fixed or determinable is then allocated among the separate units of accounting using the relative selling price method. The Company determines the estimated selling price for units of accounting within each arrangement using vendor specific objective evidence (VSOE) of selling price, if available, third party evidence (TPE) of selling price if VSOE is not available, or best estimate of selling price (BESP) if neither VSOE nor TPE is available. Determining the BESP

for a unit of accounting requires significant judgment. In developing the BESP for a unit of accounting, the Company considers applicable market conditions and relevant entity specific factors, including factors that were contemplated in negotiating the agreement with the customer and estimated costs. The Company validates the BESP for units of accounting by evaluating whether changes in the key assumptions used to determine the BESP will have a significant effect on the allocation of arrangement consideration between multiple units of accounting.

The Company will recognize arrangement consideration allocated to each unit of accounting when all of the revenue recognition criteria in ASC 605 are satisfied for that particular unit of accounting. In the event that a deliverable does not represent a separate unit of accounting, the Company will recognize revenue from the combined unit of accounting over the Company's contractual or estimated performance period for the undelivered elements, which is typically the term of the Company's research and development obligations. If there is no discernible pattern of performance or objectively measurable performance measures do not exist, then the Company will recognize revenue under the arrangement on a straight line basis over the period the Company is expected to complete its performance obligations. Conversely, if the pattern of performance in which the service is provided to the customer can be determined and objectively measurable performance measures exist, then the Company recognizes revenue under the arrangement using the proportional performance method. Revenue to be recognized is limited to the lesser of the cumulative amount of payments received or the cumulative amount of revenue earned, as determined using the straight line method or proportional performance method, as applicable, as of the period ending date.

At the inception of an arrangement that includes milestone payments, the Company evaluates whether each milestone is substantive and at risk to both parties on the basis of the contingent nature of the milestone. This evaluation includes an assessment of whether: (1) the consideration is commensurate with either the Company's performance to achieve the milestone or the enhancement of the value of the delivered item(s) as a result of a specific outcome resulting from its performance to achieve the milestone, (2) the consideration relates solely to past performance and (3) the consideration is reasonable relative to all of the deliverables and payment terms within the arrangement. The Company evaluates factors such as the scientific, clinical, regulatory, commercial, and other risks that must be overcome to achieve the particular milestone and the level of effort and investment required to achieve the particular milestone in making this assessment. There is considerable judgment involved in determining whether a milestone satisfies all of the criteria required to conclude that a milestone is substantive. Milestones that are not considered substantive are recognized as earned if there are no remaining performance obligations or over the remaining period of performance, assuming all other revenue recognition criteria are met.

Options are considered substantive if, at the inception of the arrangement, the Company is at risk as to whether the collaboration partner will choose to exercise the option. Factors that the Company considers in evaluating whether an option is substantive include the cost to exercise the option, the overall objective of the arrangement, the benefit the collaborator might obtain from the arrangement without exercising the option and the likelihood the option will be exercised. When an option is considered substantive, the Company does not consider the option or item underlying the option to be a deliverable at the inception of the arrangement and the associated option fees are not included in allocable consideration, assuming the option is not priced at a significant and incremental discount. Conversely, when an option is not considered substantive, the Company would consider the option, including other deliverables contingent upon the exercise of the option, to be a deliverable at the inception of the arrangement and a corresponding amount would be included in allocable arrangement consideration. In addition, if the price of the option includes a significant incremental discount, the discount would be included as a deliverable at the inception of the arrangement.

The Company will recognize royalty revenue in the period of sale of the related product(s), based on the underlying contract terms, provided that the reported sales are reliably measurable and the Company has no remaining performance obligations, assuming all other revenue recognition criteria are met.

Patents

Costs incurred in connection with the application for and issuance of patents are expensed as incurred.

Income Taxes

Income taxes are recorded in accordance with ASC Topic 740, Income Taxes (ASC 740), which provides for deferred taxes using an asset and liability approach. The Company recognizes deferred tax assets and liabilities for the expected future tax consequences of events that have been included in the financial statements or tax returns. Deferred tax assets and liabilities are determined based on the difference between the financial statement and tax bases of assets and liabilities using enacted tax rates in effect for the year in which the differences are expected to reverse. Valuation allowances are provided, if, based upon the weight of available evidence, it is more likely than not that some or all of the deferred tax assets will not be realized.

The Company accounts for uncertain tax positions in accordance with the provisions of ASC 740. When uncertain tax positions exist, the Company recognizes the tax benefit of tax positions to the extent that the benefit will more likely than not be realized. The determination as to whether the tax benefit will more likely than not be realized is based upon the technical merits of the tax position,

as well as consideration of the available facts and circumstances. As of September 30, 2016 and 2015, the Company does not have any significant uncertain tax positions. The Company recognizes interest and penalties related to uncertain tax positions in income tax expense.

Stock-Based Compensation

The Company accounts for its stock-based compensation awards in accordance with ASC Topic 718, Compensation—Stock Compensation (ASC 718). ASC 718 requires all stock-based payments to employees, including grants of employee stock options, restricted stock, restricted stock units, or RSUs, and modifications to existing stock awards, to be recognized in the statements of operations and comprehensive loss based on their fair values. The Company accounts for stock-based awards to non-employees in accordance with ASC Topic 505-50, Equity-Based Payments to Non-Employees (ASC 505-50), which requires the fair value of the award to be re-measured at fair value until a performance commitment is reached or counterparty performance is complete. The Company's stock-based awards are comprised of stock options, shares of restricted stock and shares of common stock. The Company estimates the fair value of options granted using the Black-Scholes option pricing model. The Company uses the quoted market price of comparable public companies to determine the fair value of restricted stock awards and common stock awards.

The Black-Scholes option pricing model requires the input of certain subjective assumptions, including (a) the expected stock price volatility, (b) the calculation of expected term of the award, (c) the risk-free interest rate and (d) expected dividends. Due to the lack of company-specific historical and implied volatility data for trading the Company's stock in the public market, the Company has based its estimate of expected volatility on the historical volatility of a group of similar companies that are publicly traded. The historical volatility is calculated based on a period of time commensurate with the expected term assumption. The computation of expected volatility is based on the historical volatility of a representative group of companies with similar characteristics to the Company, including stage of product development and life science industry focus. The Company is in the product development stage with no revenue and the representative group of companies has certain similar characteristics to the Company. The Company believes the group selected has sufficient similar economic and industry characteristics, and includes companies that are most representative of the Company. The Company uses the simplified method as prescribed by the SEC Staff Accounting Bulletin No. 107, Share-Based Payment, to calculate the expected term for options granted to employees as it does not have sufficient historical exercise data to provide a reasonable basis upon which to estimate the expected term. The expected term is applied to the stock option grant group as a whole, as the Company does not expect substantially different exercise or post-vesting termination behavior among its employee population. For options granted to non-employees, the Company utilizes the contractual term of the arrangement as the basis for the expected term assumption. The risk-free interest rate is based on a treasury instrument whose term is consistent with the expected life of the stock options. The expected dividend yield is assumed to be zero as the Company has never paid dividends and has no current plans to pay any dividends on its common stock, which is similar to the Company's peer group.

The Company's stock-based awards are subject to either service- or performance-based vesting conditions. Compensation expense related to awards to employees with service-based vesting conditions is recognized on a straight-line basis based on the grant date fair value over the associated service period of the award, which is generally the vesting term. Consistent with the guidance in ASC 505- 50, compensation expense related to awards to non-employees with service-based vesting conditions is recognized on a straight-line basis based on the then-current fair value at each financial reporting date prior to the measurement date over the associated service period of the award, which is generally the vesting term. Compensation expense related to awards to employees with performance-based vesting conditions is recognized based on the grant date fair value over the requisite service period using the accelerated attribution method to the extent achievement of the performance condition is probable. Consistent with the guidance in ASC 505-50, compensation expense related to awards to non-employees with performance-based vesting conditions is recognized based on the then-current fair value at each financial reporting date prior to the measurement date over the requisite service period using the accelerated attribution method to the

extent achievement of the performance condition is probable.

The Company is also required to estimate forfeitures at the time of grant, and revise those estimates in the subsequent periods if actual forfeitures differ from its estimates. The Company uses historical data to estimate pre-vesting forfeitures and record stock-based compensation expense only for those awards that are expected to vest. To the extent that actual forfeitures differ from the Company's estimates, the difference is recorded as a cumulative adjustment in the period the estimates were revised. Stock-based compensation expense recognized in the condensed consolidated financial statements is based on awards that are ultimately expected to vest.

Fair Value of Financial Instruments

The Company is required to disclose information on all assets and liabilities reported at fair value that enables an assessment of the inputs used in determining the reported fair values. ASC Topic 820, Fair Value Measurements and Disclosures (ASC 820), establishes a hierarchy of inputs used in measuring fair value that maximizes the use of observable inputs and minimizes the use of unobservable inputs by requiring that the observable inputs be used when available.

Observable inputs are inputs that market participants would use in pricing the asset or liability based on market data obtained from sources independent of the Company. Unobservable inputs are inputs that reflect the Company's assumptions about the inputs that

market participants would use in pricing the asset or liability, and are developed based on the best information available in the circumstances. The fair value hierarchy applies only to the valuation inputs used in determining the reported fair value of the investments, and is not a measure of the investment credit quality. The three levels of the fair value hierarchy are described below:

- Level 1 – Valuations based on unadjusted quoted prices in active markets for identical assets or liabilities that the Company has the ability to access at the measurement date.
- Level 2 – Valuations based on quoted prices for similar assets or liabilities in markets that are not active, or for which all significant inputs are observable, either directly or indirectly.
- Level 3 – Valuations that require inputs that reflect the Company's own assumptions that are both significant to the fair value measurement and unobservable.

To the extent that valuation is based on models or inputs that are less observable or unobservable in the market, the determination of fair value requires more judgment. Accordingly, the degree of judgment exercised by the Company in determining fair value is greatest for instruments categorized in Level 3. A financial instrument's level within the fair value hierarchy is based on the lowest level of any input that is significant to the fair value measurement.

Items measured at fair value on a recurring basis include short-term investments (see Note 4). The carrying amounts of prepaid expenses and other current assets, accounts payable and accrued expenses approximate their fair values due to their short-term maturities. The rate implicit within the Company's capital lease obligation approximates market interest rates.

Concentrations of Credit Risk and Off-Balance Sheet Risk

Cash and investments are the only financial instruments that potentially subject the Company to concentrations of credit risk. The Company maintains its cash with high quality, accredited financial institutions and, accordingly, such funds are subject to minimal credit risk. The Company has no significant off-balance sheet concentrations of credit risk, such as foreign currency exchange contracts, option contracts or other hedging arrangements.

Net Loss per Share

Basic net loss per share is calculated by dividing net loss attributable to common stockholders by the weighted-average shares outstanding during the period, without consideration for common stock equivalents. Diluted net loss per share is calculated by adjusting weighted-average shares outstanding for the dilutive effect of common stock equivalents outstanding for the period, determined using the treasury-stock method. For purposes of the diluted net loss per share calculation, preferred stock, stock options, unvested restricted stock and RSUs are considered to be common stock equivalents, but have been excluded from the calculation of diluted net loss per share, as their effect would be anti-dilutive for all periods presented. Therefore, basic and diluted net loss per share were the same for all periods presented.

Property and Equipment

Property and equipment is stated at cost, less accumulated depreciation. Assets under capital lease are included in property and equipment. Property and equipment is depreciated using the straight-line method over the estimated useful lives of the assets, generally three to seven years. Such costs are periodically reviewed for recoverability when impairment indicators are present. Such indicators include, among other factors, operating losses, unused capacity, market value declines and technological obsolescence. Recorded values of asset groups of equipment that are not expected to be recovered through undiscounted future net cash flows are written down to current fair value, which generally is determined from estimated discounted future net cash flows (assets held for use) or net realizable value (assets held for sale).

The following is the summary of property and equipment and related accumulated depreciation as of September 30, 2016 and December 31, 2015.

	Useful Life	September 30, 2016	December 31, 2015
		(in thousands)	
Computer equipment and software	3	$466	$ 300
Furniture and fixtures	5	724	243
Equipment	7	50	50
Leasehold improvements	Shorter of the useful life or remaining lease term (10 years)	1,730	70
Office equipment under capital lease	3	36	24
		3,006	687
Less accumulated depreciation		(318)	(147)
Net property and equipment		$2,688	$ 540

Depreciation expense, including expense associated with assets under capital leases, was approximately $93,000 and $35,000 for the three months ended September 30, 2016 and 2015, respectively and $171,000 and $85,000 for the nine months ended September 30, 2016 and 2015, respectively.

3. Available for sale securities

Available for sale securities at September 30, 2016 and December 31, 2015 consist of the following:

	Amortized Cost	Gross Unrealized Gains	Gross Unrealized Losses	Fair Value
	(in thousands)			
September 30, 2016				
Cash and cash equivalents	$65,830	$ —	$ —	$65,830
Available for sale securities:				
Certificates of deposit	$17,575	—	—	$17,575
U.S. Government debt securities	71,968	12	(14)	71,966
Corporate debt securities	5,956	—	(5)	5,951
Total available for sale securities	$95,499	$ 12	$ (19)	$95,492

Total cash, cash equivalents, and available for sale securities	$161,329	$ 12	$ (19)	$161,322

	Amortized Cost	Gross Unrealized Gains	Gross Unrealized Losses	Fair Value
	(in thousands)			
December 31, 2015				
Cash and cash equivalents	$49,778	$ —	$ —	$49,778
Available for sale securities:				
Certificates of deposit	$21,505	—	—	$21,505
U.S. Government debt securities	46,461	—	(185)	46,276
Corporate debt securities	20,944	1	(50)	20,895
Total available for sale securities	$88,910	$ 1	$ (235)	$88,676
Total cash, cash equivalents, and available for sale securities	$138,688	$ 1	$ (235)	$138,454

The estimated fair value of the Company's available for sale securities balance at September 30, 2016, by contractual maturity, is as follows:

Due in one year or less	$78,818
Due after one year	16,674
Total available for sale securities	$95,492

4. Fair Value of Financial Instruments

The Company utilizes a portfolio management company for the valuation of the majority of its investments. This company is an independent, third-party vendor recognized to be an industry leader with access to market information that obtains or computes fair market values from quoted market prices, pricing for similar securities, recently executed transactions, cash flow models with yield curves and other pricing models. For valuations obtained from the pricing service, the Company performs due diligence to understand how the valuation was calculated or derived, focusing on the valuation technique used and the nature of the inputs.

Based on the fair value hierarchy, the Company classifies its cash equivalents and marketable securities within Level 1 or Level 2. This is because the Company values its cash equivalents and marketable securities using quoted market prices or alternative pricing sources and models utilizing market observable inputs.

Assets measured or disclosed at fair value on a recurring basis as of September 30, 2016 and December 31, 2015 are summarized below:

	Fair Value Measurements Using			
	Level 1	Level 2	Level 3	Total
	(in thousands)			
September 30, 2016				
Assets:				
Cash and cash equivalents	$65,830	$—	$ —	$65,830
Certificates of deposit	—	17,575	—	17,575
U.S. Government debt securities	—	71,966	—	71,966
Corporate debt securities	—	5,951	—	5,951
	$65,830	$95,492	$ —	$161,322

	Fair Value Measurements Using			
	Level 1	Level 2	Level 3	Total
	(in thousands)			

December 31, 2015				
Assets:				
Cash and cash equivalents	$49,778	$—	$ —	$49,778
Certificates of deposit	—	21,505	—	21,505
U.S. Government debt securities	—	46,276	—	46,276
Corporate debt securities	—	20,895	—	20,895
	$49,778	$88,676	$ —	$138,454

The Company had no assets or liabilities measured at fair value on a recurring basis using significant unobservable inputs (Level 3) at September 30, 2016 and December 31, 2015.

The Company’s corporate debt securities are all investment grade.

Investment securities are exposed to various risks such as interest rate, market and credit risks. Due to the level of risk associated with certain investment securities and the level of uncertainty related to changes in the value of investment securities, it is at least reasonably possible that changes in risks in the near term would result in material changes in the fair value of investments.

5. Accrued Expenses

Accrued expenses are as follows:

	September 30, 2016	December 31, 2015
	(in thousands)	
Accrued clinical	$12,490	$ 4,536
Accrued bonus	1,876	2,178
Professional fees	619	647
Accrued vacation	378	310
Accrued payroll	259	518
Accrued severance	44	—
Other	1,949	1,366
Total accrued expenses	$17,615	$ 9,555

In January 2016, the Company entered into separation agreements with two employees. During the first quarter of 2016, the Company recorded severance expense in the amount of $0.4 million, of which $0.2 million was recorded to general and administrative expense and $0.2 million was recorded to research and development expense. During the three months ended September 30, 2016, approximately $0.2 million was paid out of the severance accrual and during the nine months ended September 30, 2016, approximately $0.4 was paid out of the severance accrual.

6. Stockholders' Equity

Authorized and Outstanding Capital Stock

As of December 31, 2014, the authorized capital stock of the Company included 175,000,000 shares of common stock, par value $0.00001 per share, of which 38,253,190 and 30,662,218 shares are issued and outstanding at September 30, 2016 and December 31, 2015, respectively; and 25,000,000 shares of undesignated preferred stock, par value $0.00001 per share, of which 0 shares are issued and outstanding at September 30, 2016 and December 31, 2015.

Equity Plans

On February 28, 2014, the Company's Board of Directors adopted its 2014 Incentive Plan (2014 Plan) and its 2014 Employee Stock Purchase Plan (ESPP), which were subsequently approved by its stockholders and became effective upon the closing of the Company's initial public offering on March 25, 2014. The 2014 Plan replaces the 2008 Equity Incentive Plan (2008 Plan), however, any options or awards outstanding under the 2008 Plan at the time of adoption of the 2014 Plan remain outstanding and effective.

The 2014 Plan allows for the granting of stock options, stock appreciation rights, or SARs, restricted stock, unrestricted stock, stock units, performance awards and other awards convertible into or otherwise based on shares of our common stock. Dividend equivalents may also be provided in connection with an award under the 2014 Plan. The

Company's employees, officers, directors and consultants and advisors are eligible to receive awards under the 2014 Plan. The Company initially reserved 1,785,000 shares of its common stock for the issuance of awards under the 2014 Plan. The 2014 Plan provides that the number of shares reserved and available for issuance under the 2014 Plan will automatically increase annually on January 1st of each calendar year, by an amount equal to three percent (3%) of the number of shares of stock outstanding on a fully diluted basis as of the close of business on the immediately preceding December 31st (2014 Plan Evergreen Provision). The Company's Board of Directors may act prior to January 1st of any year to provide that there will be no automatic increase in the number of shares available for grant under the 2014 Plan for that year (or that the increase will be less than the amount that would otherwise have automatically been made). Subject to adjustment, no more than 1,131,937 shares of our common stock may be delivered in satisfaction of incentive stock options awarded under the 2014 Plan. During the first nine months of 2016, the Company granted 1,092,275 stock options to employees, 449,838 RSUs to employees and 112,500 stock options to directors under the 2014 Plan.

The ESPP authorizes the initial issuance of up to a total of 262,500 shares of the Company's common stock to participating employees. The maximum aggregate number of shares of common stock available for purchase pursuant to the exercise of options granted under the ESPP will be the lesser of (a) 262,500 shares, increased on each anniversary of the adoption of the ESPP by one percent (1%) of the total shares of common stock then outstanding and (b) 739,611 shares (which is equal to five percent (5%) of the total shares of common stock outstanding on the date of the adoption of the ESPP on a fully diluted, as converted basis (ESPP Evergreen Provision). Under the ESPP, each offering period is six months, at the end of which employees may purchase shares of common stock through payroll deductions made over the term of the offering. The per-share purchase price at the end of each offering period is equal to the lesser of 85% of the closing price of our common stock at the beginning or end of the offering period.

Shares Reserved for Future Issuance

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

	September 30, 2016	December 31, 2015
Common stock options and RSU's outstanding	3,416,546	2,231,057
Shares available for issuance under the 2014 Plan [1]	1,035,605	1,318,732
Shares available for issuance under the ESPP [2]	803,105	440,304
Total	5,255,256	3,990,093

[1] On January 1, 2016, the shares reserved for future grants under the 2014 Plan increased by 986,800 shares pursuant to the 2014 Plan Evergreen Provision.

[2] On February 28, 2016, the shares reserved for future issuance under the ESPP increased by 379,430 shares pursuant to the ESPP Evergreen Provision.

Stock-Based Compensation

Stock Options

On February 22, 2016, as part of the Company's annual grant of equity, the Company issued 624,275 stock options to employees. In addition, the Company issues stock options to new hires and occasionally to other employees not in connection with the annual grant process. Options granted by the Company vest over periods of between 12 and 48 months. Options vest in installments of (i) 25% at the one year anniversary and (ii) in either 36 or 48 equal monthly or 12 equal quarterly installments beginning in the thirteenth month after the initial Vesting Commencement Date (as defined) or grant date, subject to the employee's continuous service with the Company. Options generally expire ten years after the date of grant. The Company recorded approximately $1.2 million of stock-based compensation expense related to stock options during the three months ended September 30, 2016 and approximately $3.4 million during the nine months ended September 30, 2016.

Restricted Stock

On December 23, 2013, the Company issued 450,224 shares of restricted stock to employees and 79,067 shares of restricted stock to non-employees at a grant date fair value of $7.42 per share. The aggregate grant date fair value for the shares of restricted stock issued on December 23, 2013 totaled approximately $3.9 million. The awards of restricted stock contained a performance condition wherein vesting is contingent upon the Company's consummation of a liquidity event, as defined, prior to the fifth anniversary of the date of grant. Certain of the awards of restricted stock have a requisite service period that was complete upon grant. The remainder of the awards of restricted stock have a requisite service period of four years whereby the award vests 25% on the one year anniversary of the Vesting Commencement Date (as defined), then ratably on the first day of each calendar quarter for 12 quarters, subject to continuous service by the individual and achievement of the performance target. Due to the nature of the performance condition, the Company had concluded that the performance condition was not probable of achievement and therefore, recognition of compensation cost had been deferred until the occurrence of a liquidity event, as defined. Compensation expense related to the restricted stock awards is being recognized over the associated requisite service period which commenced on March 25, 2014. The Company recorded approximately $0.1 million of stock-based compensation expense related to restricted stock during the three months ended September 30, 2016 and recorded approximately $0.1 million during the nine months ended September 30, 2016 as a result of mark to market adjustments related to non-employees.

Restricted Stock Units

On February 22, 2016, as part of the Company's annual grant of equity, the Company issued 382,338 RSUs to employees. In addition, the Company occasionally issues RSUs not in connection with the annual grant process to employees. The RSUs vest 100% on the third year anniversary of the grant date. Total stock-compensation expense to be recognized over the life of the RSUs is $2.9 million and will be recognized on a straight-line basis over the vesting period. The Company recorded approximately $0.2 million of stock-based compensation expense related to the RSUs during the three months ended September 30, 2016 and approximately $0.5 million during the nine months ended September 30, 2016.

ESPP

The first offering period under the ESPP opened on January 2, 2015. There were 16,629 shares issued during the second quarter of 2016. The Company recorded approximately $32,000 of stock-based compensation expense related to ESPP during the three months ended September 30, 2016 and approximately $79,000 during the nine months ended September 30, 2016.

Compensation Expense Summary

The Company has recognized the following compensation cost related to share-based awards:

	Three Months Ended		Nine Months Ended	
	September 30, 2016	September 30, 2015	September 30, 2016	September 30, 2015
	(in thousands)		(in thousands)	
Research and development	$636	$ 468	$1,393	$ 1,431
General and administrative	992	694	2,708	1,955
Total	$1,628	$ 1,162	$4,101	$ 3,386

Compensation expense by type of award:

	Three Months Ended		Nine Months Ended	
	September 30, 2016	September 30, 2015	September 30, 2016	September 30, 2015
	(in thousands)		(in thousands)	
Stock options	$1,229	$ 1,065	$3,378	$ 2,673
Restricted stock	135	61	148	604
Restricted stock units	232	13	496	44
Employee stock purchase plan	32	23	79	65
Total	$1,628	$ 1,162	$4,101	$ 3,386

7. 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 assets will not be realized. There were no significant income tax provisions or benefits for the three or nine months ended September 30, 2016 and 2015. Due to the uncertainty surrounding the realization of the 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.

8. Commitments and Contingencies

The Company leases approximately 45,362 square feet of office and lab space in Cambridge, Massachusetts under a lease which was most recently amended in July 2016, collectively, the Lease. Total monthly lease payments for base rent are approximately $242,000 per month which is subject to annual rent escalations. In addition to such annual rent

escalations, base rent payments for a portion of said premises are scheduled to commence on or about January 1, 2017 in the monthly amount of approximately $22,000. Landlord contributions included in the Lease from the landlord totaled $2,169,920, including $256,765 in leasehold improvements not yet utilized. The landlord contributions are being accounted for as a deferred lease incentive and reduction in monthly rent expense over the term of the Lease. The term of the Lease with respect to the office space expires on September 11, 2026, with one five year extension option available. The term of the Lease for the lab space is five years, with an extension option for one additional period of two years. The total security deposit in connection with the Lease of $1,280,857 is included in other assets in the Company's condensed consolidated balance sheets as of September 30, 2016 and December 31, 2015.

The Company recognizes rent expense for the space which it currently occupies and records a deferred lease obligation representing the cumulative difference between actual facility lease payments and lease expense recognized ratably over the lease period, which is included in the Company's condensed consolidated balance sheets as of September 30, 2016 and December 31, 2015. The Company will begin recognizing rent expense for the lab space and the remaining office space, which the Company did not yet occupy as of September 30, 2016, subsequent to taking possession of the space.

The Company leases office equipment under three year capital leases with payments commencing in February 2014, April 2015 and February 2016, respectively. The capital lease amounts are included in accrued expenses and other liabilities.

At September 30, 2016, the Company's future minimum payments required under these leases are as follows:

	Operating Lease	Capital Lease	Total
	(in thousands)		
2016	$714	$ 3	$717
2017	3,122	9	3,131
2018	3,122	5	3,127
2019	3,122	—	3,122
2020	3,122	—	3,122
Thereafter	17,789	—	17,789
Total	$30,991	17	$31,008
Less amount representing interest		—	
Present value of minimum lease payments at September 30, 2016		$ 17	

The Company recorded approximately $0.6 million and $0.2 million in rent expense for the three months ended September 30, 2016 and 2015, respectively and $1.7 million and $0.6 million in rent expense for the nine months ended September 30, 2016 and 2015, respectively.

Under the Company's agreement with a subsidiary of Quintiles IMS Holdings, Inc., or Quintiles, to provide services for the PRO_2TECT and $INNO_2$VATE programs, the total remaining contract costs as of September 30, 2016 were approximately $423.9 million. The estimated period of performance for the committed work with Quintiles is through the third quarter of 2019. The Company contracts with various other organizations to conduct research and development activities with remaining contract costs to the Company of approximately $22.0 million and $5.2 million at September 30, 2016 and December 31, 2015, respectively. The scope of the services under the research and development contracts can be modified and the contracts cancelled by the Company upon written notice. In some instances, the contracts may be cancelled by the third party upon written notice.

In September 2015, a purported securities class action lawsuit was filed against the Company, including its Chief Executive Officer, its Chief Financial Officer, and members of the Company's Board of Directors, in the Business Litigation Section of the Suffolk County Superior Court of Massachusetts. The complaint is brought on behalf of an alleged class of those who purchased common stock of the Company pursuant or traceable to the Company's initial public offering, and purports to allege claims arising under Sections 11, 12(a)(2) and 15 of the Securities Act of 1933, as amended. The complaint generally alleges that the defendants violated the federal securities laws by, among other things, making material misstatements or omissions concerning the Phase 2b clinical study of vadadustat. The complaint seeks, among other relief, unspecified compensatory damages, rescission of certain stock purchases, attorneys' fees, and costs. In October 2015, the Company removed the case to the United States District Court for the District of Massachusetts, and the plaintiff filed a motion to remand the case back to the Business Litigation Section of the Suffolk County Superior Court of Massachusetts. The plaintiff's motion to remand was granted in April 2016. The plaintiff filed an amended complaint in the Suffolk County Superior Court on August 15, 2016, and the Company served a memorandum in support of its motion to dismiss the amended complaint on October 14, 2016. The Company believes such claims are without merit and will engage in a vigorous defense of such litigation.

The Company has had a number of positive developments in our opposition and invalidity proceedings against FibroGen, Inc., or FibroGen. With regard to the opposition that the Company filed in Europe against FibroGen's European Patent No. 1463823, or the '823 patent, an oral proceeding took place March 8 and 9, 2016. Following the

oral proceeding, the European Opposition Division ruled that the patent as granted did not meet the requirements for patentability under the European Patent Convention and, therefore, revoked the patent in its entirety. FibroGen has appealed that decision. Likewise, with regard to the invalidity proceeding that the Company filed in Japan against certain claims of FibroGen's Japanese Patent No. 4804131, or the '131 patent, which is the Japanese counterpart to the '823 patent, the Japan Patent Office, or JPO, issued a preliminary decision finding all of the challenged claims to be invalid. FibroGen subsequently amended the claims and the JPO accepted the amendments. The resulting FibroGen Japanese '131 patent does not cover vadadustat or any pyridine carboxamide compounds. To date, FibroGen has been unsuccessful in its attempts to obtain a patent in the United States covering the same claim scope as it obtained initially in Europe and Japan in the '823 and '131 patents. In the event FibroGen were to obtain such a patent in the United States, the Company may decide to challenge them like the Company has done in Europe and Japan.

On May 13, 2015, May 20, 2015 and July 6, 2015 the Company filed oppositions to FibroGen's European Patent Nos. 2322153, 2322155, and 1633333, or the '153 patent, the '155 patent, and the '333 patent, respectively, requesting the patents be revoked in their entirety. These related patents claim, among other things, various compounds that either stabilize HIF or inhibit a HIF hydroxylase or a HIF prolyl hydroxylase for treating or preventing various conditions, including, inter alia, iron deficiency, microcytosis associated with iron deficiency, anemia of chronic disease, anemia wherein the subject has a transferrin saturation of less than 20%, anemia

refractory to treatment with exogenously administered erythropoietin, or EPO, and microcytosis in microcytic anemia. Oppositions to the ’155 patent and to the ’153 patent were also filed by Glaxo Group Limited and by Bayer Intellectual Property GmbH, Bayer Pharma Aktiengesellschaft, and Bayer Animal Health GmbH. While, for the reasons set forth in our oppositions, the Company believes that the ’153 patent, the ’155 patent, and the ’333 patent should be revoked in their entirety, the ultimate outcomes of the oppositions remains uncertain. If the European Patent Office decides not to revoke the ’153 patent, the ’155 patent, or the ’333 patent in their entirety, or only certain claims of those patents, and any surviving claims are determined to encompass the Company’s intended use of the Company’s lead product candidate, the Company may not be able to commercialize the Company’s lead product candidate in the European Union for its intended use, which could materially adversely affect the Company’s business, operating results and financial condition.

The Company’s policy is to record a liability if a loss in a significant legal dispute is considered probable and an amount can be reasonably estimated. The Company provides disclosure when a loss in excess of any reserve is reasonably possible, and the Company is in a position to estimate the potential loss or range of possible loss. Significant judgment is required to assess the likelihood of various potential outcomes and the quantification of loss in those scenarios. The Company’s estimates change as litigation progresses and new information comes to light. Changes in Company estimates could have a material impact on the Company’s results and financial position.

9. Significant Agreements

Mitsubishi Tanabe Pharma Collaboration Agreement

On December 11, 2015, the Company and MTPC, entered into a collaboration agreement (Collaboration Agreement) providing MTPC with exclusive development and commercialization rights to vadadustat, the Company’s product candidate for the treatment of anemia related to chronic kidney disease, in Japan and certain other Asian countries (collectively, the Territory).

Pursuant to the Collaboration Agreement, MTPC will have an exclusive license to develop and commercialize vadadustat in the Territory. In addition, the Company will supply vadadustat for both clinical and commercial use in the Territory. The countries included in the Territory are Japan, Taiwan, South Korea, Singapore, Malaysia, India, Indonesia, East Timor, Mongolia, the Philippines, Vietnam, Laos, Cambodia, Thailand, Brunei, Myanmar, Nepal, Sri Lanka, Bangladesh, Bhutan, Maldives, Palau and Tonga.

In consideration for the exclusive license and other rights contained in the Collaboration Agreement, MTPC will make payments totaling up to $100.0 million to fund the vadadustat global Phase 3 program, including $40.0 million paid in January 2016. If Japanese patients are not included in either the global Phase 3 PRO_2TECT or $INNO_2$VATE programs, $20.0 million of the $40.0 million received would be used to fund further local development of vadadustat in Japan or be refunded to MTPC. The Company is also eligible to receive up to approximately $250.0 million in additional milestone payments, based upon achievement of certain development and sales milestones as well as tiered royalty payments, from low teens up to twenty percent, on sales of vadadustat in the Territory.

The Company and MTPC have established a joint steering committee to oversee development and commercialization of vadadustat in the Territory, including approval of any development or commercialization plans. Unless earlier terminated, the Collaboration Agreement will continue in effect on a country-by-country basis until the later of: expiration of the last-to-expire patent covering vadadustat in such country in the Territory; expiration of marketing or regulatory exclusivity in such country in the Territory; or ten (10) years after the first commercial sale of vadadustat in such country in the Territory. MTPC may terminate the Collaboration Agreement upon twelve (12) months’ notice at

any time after the second anniversary of the effective date of the Collaboration Agreement. Either party may terminate the Collaboration Agreement upon the material breach of the other party that is not cured within a specified time period or upon the insolvency of the other party.

As of September 30, 2016, there is $40.0 million of deferred revenue related to the Company's Collaboration Agreement, all of which is classified as long term in the accompanying condensed consolidated balance sheet. Revenue recognition for the Collaboration Agreement will commence when all criteria as required under ASC 605 have been satisfied, which the Company expects will be in 2017 when the scope of the Phase 3 program is agreed upon with Japanese regulatory authorities.

10. Employee Retirement Plan

During 2008, the Company established a retirement plan (the Plan) authorized by Section 401(k) of the Internal Revenue Code. In accordance with the Plan, all employees who have attained the age of 21 are eligible to participate in the Plan as of the first Entry Date, as defined, following their date of employment. Each employee can contribute a percentage of compensation up to a maximum of the statutory limits per year. Company contributions are discretionary and contributions in the amount of approximately $21,000

and $71,000 were made during the three months ended September 30, 2016 and 2015, respectively, and $141,000 and $71,000 were made during the nine months ended September 30, 2016 and 2015, respectively.

11. Net Loss per Share

The shares in the table below were excluded from the calculation of diluted net loss per share, prior to the use of the treasury stock method, due to their anti-dilutive effect:

	Three Months Ended		Nine Months Ended	
	September 30, 2016	September 30, 2015	September 30, 2016	September 30, 2015
Outstanding stock options	2,977,986	2,251,908	2,977,986	2,251,908
Unvested restricted stock	132,739	265,402	132,739	265,402
Unvested restricted stock units	438,563	24,425	438,563	24,425
Total	3,549,288	2,541,735	3,549,288	2,541,735

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

The following information should be read in conjunction with the unaudited financial information and the notes thereto included in this Quarterly Report on Form 10-Q and the condensed consolidated financial statements and notes thereto for the year ended December 31, 2015, and the related Management's Discussion and Analysis of Financial Condition and Results of Operations, contained in our annual report on Form 10-K filed with the United States Securities and Exchange Commission, or the SEC, on March 14, 2016, which we refer to as our annual report.

This report contains forward-looking statements that are being made pursuant to the safe harbor provisions of the Private Securities Litigation Reform Act of 1995, or PSLRA, with the intention of obtaining the benefits of the "safe harbor" provisions of the PSLRA. Forward-looking statements involve risks and uncertainties. In this Quarterly Report on Form 10-Q, words such as "may," "will," "expect," "anticipate," "estimate," "intend," and similar expressions (as well as ot words or expressions referencing future events, conditions or circumstances) are intended to identify forward-looking statements.

Our actual results and the timing of certain events may differ materially from the results discussed, projected, anticipated, or indicated in any forward-looking statements. We caution our readers 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 those expressed or implied by the forward-looking statements contained in this Quarterly Report on Form 10-Q.

The following information, including all forward-looking statements, should be considered in light of factors discussed elsewhere in this 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.

Operating Overview

We are a biopharmaceutical company focused on the development of novel proprietary therapeutics based on hypoxia inducible factor, or HIF, biology and the commercialization of these products for patients with serious unmet medical needs. HIF is the primary regulator of the production of red blood cells, or RBCs, in the body and a potentially novel mechanism for the treatment of anemia secondary to chronic kidney disease, or CKD. Pharmacologic modulation of the HIF pathway may also have broader therapeutic applications in acute renal failure, organ protection, ischemia-reperfusion injury, cancer, ophthalmology, and inflammatory diseases.

Anemia is a serious medical condition in which blood is deficient in RBCs and hemoglobin, each of which is critical in delivering oxygen to tissue. Anemia generally exists when hemoglobin, a protein in RBCs that carries oxygen, is less than 13 g/dL in men or 12 g/dL in women. Untreated anemia is associated with chronic fatigue, increased risk of progression of multiple diseases and death. Anemia is common in patients with CKD, cancer, heart failure, inflammatory diseases and other critical illnesses, as well as in the elderly.

More than 30 million people in the United States have CKD, with estimates that over 1.8 million of these patients suffer from anemia. Anemia from these indications is currently treated with injectable recombinant erythropoiesis-stimulating agents, or rESAs,—including Epogen®, Procrit® and Aranesp® — iron supplementation or RBC

transfusions. Based on the reported revenues of companies that market and sell rESAs, we estimate that global sales of injectable rESAs were $7.0 billion in 2014; the vast majority of which were for renal indications.

rESAs are designed to stimulate production of RBCs by binding directly to and saturating erythropoietin, or EPO, receptors. While injectable rESAs and transfusions may be effective in raising hemoglobin levels, they carry significant potential side effects and need to be delivered subcutaneously or intravenously. In particular, injectable rESAs may lead to thrombosis, stroke, myocardial infarction and death. These risks are described in black box warnings on the prescribing information of all products marketed in this class. These safety concerns, which became evident starting in 2006, have led to a significant reduction in the use of injectable rESAs. Today, anemia is either not treated or inadequately treated in the majority of non-dialysis dependent CKD patients. As a result, we believe that a safe, effective, oral therapeutic option will take significant market share and meaningfully grow the market in patients not requiring dialysis.

Given the burdens of the current standard of care and costs associated with administering an injectable rESA, we believe our lead product candidate, vadadustat, is a promising cost-effective alternative for the treatment of anemia in CKD. Vadadustat is being developed as a once-daily, oral therapy and has successfully completed Phase 2 development demonstrating that vadadustat can safely and predictably raise hemoglobin levels in patients with anemia related to CKD. Vadadustat works by a differentiated mechanism of action that we believe has the potential to be safer than that of injectable rESAs and may offer additional beneficial

therapeutics effects beyond anemia including delaying CKD progression. This novel mechanism of action is referred to as HIF prolyl-hydroxylase, or HIF-PH, inhibition. Instead of binding directly to the EPO receptors on cells in the bone marrow, vadadustat leads to activation of critical pathways for hemoglobin and RBC production. This approach mimics the physiological adjustment made by the body when exposed to reduced oxygen levels at higher altitudes.

We have commenced Phase 3 development of vadadustat in non-dialysis CKD patients. Positive results from our Phase 2b study in non-dialysis CKD patients demonstrated that vadadustat raised hemoglobin levels with no safety signal observed. In December 2015, we began dosing patients in our Phase 3 vadadustat program in non-dialysis patients with anemia related to CKD, PRO_2TECT. If the results from the PRO_2TECT program support the results observed across our previous clinical studies, including 29,000 days of patient exposure, we anticipate submitting a New Drug Application, or NDA, to the United States Food and Drug Administration, or FDA, for vadadustat in 2019.

We have also completed a Phase 2 study of vadadustat for the treatment of anemia in patients undergoing dialysis, which found that vadadustat, dosed either once daily or three times per week, maintained stable hemoglobin levels following conversion from rESA therapy with no safety signal observed. We initiated our Phase 3 vadadustat program in dialysis-dependent patients with anemia related to CKD, $INNO_2VATE$, in August 2016, and we anticipate full enrollment by early 2018.

We have engaged Quintiles IMS Holdings, Inc., or Quintiles, as our primary clinical research organization, or CRO, for the PRO_2TECT and $INNO_2VATE$ programs. We expect the cost of the Phase 3 program to be in the range of $80,000 to $85,000 per patient and we plan to enroll approximately 3,100 patients in the PRO_2TECT program and approximately 2,600 patients in the $INNO_2VATE$ program. In September 2016, the Independent Data Monitoring Committee, IDMC, for our global Phase 3 PRO2TECT program the initial meeting according to its charter and recommended continuing the studies without modification.

A subset of dialysis-dependent CKD patients have shown an inadequate hemoglobin response despite receiving high doses of rESAs. Previous studies have shown that rESA hyporesponsiveness is associated with poor clinical outcomes including increased mortality risk. By increasing iron mobilization, in addition to increasing erythropoietin levels, vadadustat may allow for a more consistent hemoglobin response in these patients. We expect to generate clinical data in hyporesponsive dialysis-dependent patients in 2017.

If approved by regulatory authorities, we plan to commercialize vadadustat in the United States for the treatment of anemia in patients with CKD. These patients are primarily treated by approximately 7,000 nephrologists, and we believe we can reach most of this market with a specialty sales force of approximately 125 people. In Japan and certain other Asian countries, we plan to commercialize vadadustat through our collaboration with Mitsubishi Tanabe Pharma Corporation, or MTPC. We intend to seek one or more collaborators to commercialize vadadustat in additional markets.

Our second clinical candidate, AKB-6899, is designed as a small molecule HIF-PH inhibitor with potential therapeutic benefit in anemia, oncology and ophthalmology. We anticipate initiation of a Phase 1 study in 2017.

Since our inception in 2007, we have devoted the largest portion of our resources to our development efforts relating to vadadustat, including preparing for and conducting clinical studies of vadadustat, providing general and administrative support for these operations and protecting our intellectual property. We do not have any products approved for sale and have not generated any revenue from product sales. We have funded our operations primarily through equity offerings.

We have never been profitable and have incurred net losses in each year since inception. Our net losses were $97.9 million and $40.9 million for the nine months ended September 30, 2016 and 2015, respectively. Substantially all of our net losses resulted from costs incurred in connection with our research and development programs and from general and administrative costs associated with our operations.

We expect to continue to incur significant operating expenses and increased operating losses for at least the next several years. We expect our expenses will increase substantially in connection with our ongoing activities, as we:

- conduct a global Phase 3 development program of vadadustat for the treatment of anemia secondary to CKD;
- seek regulatory approvals for our product candidates that successfully complete clinical trials;
- have our product candidates manufactured for clinical trials and for commercial sale;
- establish a sales, marketing and distribution infrastructure to commercialize any products for which we may obtain marketing approval;
- continue preclinical and clinical development of AKB-6899;
- initiate additional preclinical, clinical or other studies for additional indications for vadadustat, AKB 6899 and other product candidates that we may develop or acquire;

- seek to discover and develop additional product candidates;
- acquire or in-license other commercial products, product candidates and technologies;
- make royalty, milestone or other payments under any future in-license agreements;
- maintain, protect and expand our intellectual property portfolio;
- attract and retain skilled personnel; and
- create additional infrastructure to support our operations as a public company.

We do not expect to generate revenue from product sales unless and until we successfully complete development and obtain regulatory approval for one or more of our product candidates, which we expect will take a number of years and is subject to significant uncertainty. We have no manufacturing facilities, and all of our manufacturing activities are contracted out to third parties. Additionally, we currently utilize third-party contract research organizations, or CROs, to carry out our clinical development activities, and we do not yet have a sales organization. If we obtain regulatory approval for any of our product candidates, we expect to incur significant commercialization expenses related to product sales, marketing, manufacturing and distribution. Accordingly, we will seek to fund our operations through public or private equity or debt financings or other sources including geographic partnerships. However, we may be unable to raise additional funds or enter into other arrangements when needed on favorable terms or at all. Our failure to raise capital or enter into such other arrangements as and when needed would have a negative impact on our financial condition and our ability to develop our products.

In December 2015, we entered into a collaboration agreement with MTPC to develop and commercialize vadadustat in Japan and certain other countries in Asia for total milestone payments of up to $350.0 million, including up to $100.0 million in upfront and development payments, of which $40.0 million was received in January 2016. If Japanese patients are not included in either the global Phase 3 PRO_2TECT or $INNO_2$VATE programs, $20.0 million of the $40.0 million received would be used to fund further local development of vadadustat in Japan or be refunded to MTPC. In addition, we are also eligible to receive tiered double-digit royalty payments on vadadustat sales.

Through the end of 2015 we have raised approximately $187.4 million of net proceeds from three underwritten public offerings, including our initial public offering. In January 2016, we completed a follow-on public offering whereby we sold 7,250,000 shares of common stock at a price of $9.00 per share. The aggregate net proceeds received by us from the offering were approximately $61.0 million, net of underwriting discounts and commissions and estimated offering expenses payable by us.

In May 2016, we entered into a Sales Agreement with Cantor Fitzgerald & Co. to periodically sell up to $75 million of shares of our common stock in an at-the-market, or ATM, offering. During the third quarter of 2016, we sold 239,906 shares of common stock pursuant to the Sales Agreement. The aggregate net proceeds received by us were approximately $2.2 million, net of commissions.

Financial Overview

In the quarter ended December 31, 2015, we identified and corrected an error in the historical classification of certain operating costs between research and development and general and administrative expenses. We concluded the effect of this classification error was not material to our consolidated financial statements for any prior period. The classification correction had no effect on our current or historical total operating expenses or net loss.

Revenue

To date, we have not generated any revenue from the sales of products or other means. Our ability to generate product revenue and become profitable depends upon our ability to successfully develop and commercialize products. We expect to incur losses for the foreseeable future, and we expect these losses to increase as we continue our development of, and seek regulatory approvals for, our product candidates and begin to commercialize any approved products. 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 revenue from the sale of our products, 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 our operations.

For the foreseeable future, we expect substantially all of our revenue will be generated from our collaboration with MTPC and any other collaborations we may enter into.

Research and Development Expenses

Research and development expenses consist primarily of costs incurred for the development of our product candidates, which include:

- employee-related expenses, including salaries, benefits, recruiting fees, travel and stock-based compensation expense;
- expenses incurred under agreements with the CROs and investigative sites that conduct our clinical studies;
- the cost of acquiring, developing and manufacturing clinical study materials;
    - facilities, depreciation and other expenses, which include direct and allocated expenses for rent and maintenance of facilities, insurance and other supplies; and
- costs associated with preclinical and clinical activities.

Research and development costs are expensed as incurred. Costs for certain development activities are recognized based on an evaluation of the progress to completion of specific tasks using information and data provided to us by our vendors and our clinical sites.

We cannot determine with certainty the duration and completion costs of the current or future clinical studies of our product candidates or if, when, or to what extent we will generate revenue from the commercialization and sale of any of our product candidates that obtain regulatory approval. We may never succeed in achieving regulatory approval for any of our product candidates.

The duration, costs and timing of clinical studies and development of our product candidates will depend on a variety of factors, including:

- the results of our meetings with the FDA and the EMA and other regulatory authorities and the consequential effect on our study design, study size and resulting operating costs;
- the size, rate of progress, results and costs of initiating and completing our global Phase 3 development of vadadustat;
- difficulties or delays in enrolling patients in our clinical trials;
- assuming favorable Phase 3 clinical results, the timing of, and the costs involved in, obtaining regulatory approvals for vadadustat in dialysis and non-dialysis indications, including to fund the preparation and filing of regulatory submissions for vadadustat with the FDA, the EMA and other regulatory authorities, and whether we will seek regulatory approval for both indications simultaneously;
- the cost, timing and outcome of our efforts to obtain marketing approval for vadadustat in the United States, Europe and in other jurisdictions;
- the scope, progress, results and costs of additional preclinical, clinical, or other studies for additional indications for vadadustat, AKB-6899 and other product candidates that we may develop or acquire;
- the timing of, and the costs involved in, obtaining regulatory approvals for AKB-6899 if clinical studies are successful;
- the cost and timing of future commercialization activities for our products, if any of our product candidates are approved for marketing, including product manufacturing, marketing, sales and distribution costs;
- the cost of having our product candidates manufactured for clinical trials;
- our ability to establish and maintain strategic collaborations, licensing or other arrangements and the financial terms of such agreements; and
- unanticipated changes to laws or regulations applicable to our clinical trials.

A change in the outcome of any of these variables with respect to the development of a product candidate could mean a significant change in the costs and timing associated with the development of that product candidate. For example, if the FDA, EMA or another regulatory authority were to require us to conduct clinical studies in addition to or different from those that we currently anticipate, or if we experience significant delays in enrollment in any of our clinical studies, we could be required to expend significant additional financial resources and time on the completion of clinical development.

From inception through September 30, 2016, we have incurred $198.4 million in research and development expenses. We plan to increase our research and development expenditures for the foreseeable future as we continue the development of vadadustat and AKB-6899. Our current and/or planned research and development activities include the following:

- global development of vadadustat, including the PRO2TECT and INNO2VATE clinical programs; and
- the initiation of a Phase 1 study of AKB-6899.

Our direct research and development expenses consist principally of external costs, such as fees paid to clinical trial sites, consultants, central laboratories and CROs in connection with our clinical studies, and costs related to acquiring and manufacturing clinical study materials.

We currently have two programs to which our research and development costs are attributable. Historically, we have not accumulated and tracked our research and development costs or our personnel and personnel-related costs on a program-by-program basis. Our employee and infrastructure resources, and many of our costs, were directed broadly to applicable research endeavors. As a result, we are unable to specify precisely the historical costs incurred for each of our programs on a program-by-program basis.

General and Administrative Expenses

General and administrative expenses consist primarily of salaries and related costs for personnel, including stock-based compensation and travel expenses. Other general and administrative expense include facility-related costs, fees for directors, accounting and legal services fees, recruiting fees and expenses associated with obtaining and maintaining patents.

We anticipate that our general and administrative expenses will increase in the future as we increase our headcount to support our continued research and development and potential commercialization of our product candidates. We also anticipate increased expenses related to finance, legal, regulatory and tax-related services associated with maintaining compliance with exchange listing and SEC requirements, and our other costs associated with being a public company. Additionally, we anticipate an increase in payroll and related expenses if and when we prepare for commercial operations, especially in sales and marketing.

Critical Accounting Policies and Significant Judgments and Estimates

Our management's discussion and analysis of our financial condition and results of operations are based on our consolidated financial statements, which have been prepared in accordance with U.S. generally accepted accounting principles. The preparation of these consolidated financial statements requires us to make estimates and judgments that affect the reported amounts of assets, liabilities, and expenses and the disclosure of contingent assets and liabilities in our consolidated financial statements. On an ongoing basis, we evaluate our estimates and judgments, including those related to prepaid and accrued research and development expenses and stock-based compensation. We base our estimates on historical experience, known trends and events, and various other factors that are believed to be reasonable under the circumstances, the results of which form the basis for making judgments about the carrying values of assets and liabilities that are not readily apparent from other sources. Actual results may differ from these estimates under different assumptions or conditions.

While our significant accounting policies are described in more detail in the notes to our consolidated financial statements appearing elsewhere in this Quarterly Report on Form 10-Q, we believe the following accounting policies to be most critical to the judgments and estimates used in the preparation of our consolidated financial statements.

Revenue

We will recognize revenue in accordance with Financial Accounting Standards Board, or FASB, Accounting Standards Codification, or ASC, Topic 605, Revenue Recognition, or ASC 605. Accordingly, revenue will be recognized for each unit of accounting when all of the following criteria are met:

- persuasive evidence of an arrangement exists;
- delivery has occurred or services have been rendered;
- the seller's price to the buyer is fixed or determinable; and
- collectability is reasonably assured.

Amounts received prior to satisfying the revenue recognition criteria are recorded as deferred revenue in our consolidated balance sheets.

Multiple Element Arrangements

Determination of Accounting Units

We analyze multiple element arrangements based on the guidance in ASC Topic 605 25, Revenue Recognition—Multiple Element Arrangements, or ASC 605 25. Pursuant to the guidance in ASC 605 25, we evaluate multiple element arrangements to determine (1) the deliverables included in the arrangement and (2) whether the individual deliverables represent separate units of accounting or whether they must be accounted for as a combined unit of accounting. This evaluation involves subjective determinations and requires management to make judgments about the individual deliverables and whether such deliverables are separate from other aspects of the

contractual relationship. Deliverables are considered separate units of accounting provided that: (i) the delivered item(s) has value to the customer on a standalone basis and (ii) if the arrangement includes a general right of return relative to the delivered item(s), delivery or performance of the undelivered item(s) is considered probable and substantially within our control. In assessing whether an item under a collaboration has standalone value, we consider factors such as the research, manufacturing, and commercialization capabilities of the collaboration partner and the availability of the associated expertise in the general marketplace. We also consider whether our collaboration partner can use the other deliverable(s) for their intended purpose without the receipt of the remaining element(s), whether the value of the deliverable is dependent on the undelivered item(s), and whether there are other vendors that can provide the undelivered element(s).

Options under a collaboration are considered substantive if, at the inception of the arrangement, we are at risk as to whether the collaboration partner will choose to exercise the option. Factors that we consider in evaluating whether an option is substantive include the cost to exercise the option, the overall objective of the arrangement, the benefit the collaboration partner might obtain from the arrangement without exercising the option, and the likelihood the option will be exercised. When an option is considered substantive, we would not consider the option or item underlying the option to be a deliverable at the inception of the arrangement and the associated option fees are not included in allocable consideration, assuming the option is not priced at a significant and incremental discount. Conversely, when an option is not considered substantive, we would consider the option, including other deliverables contingent upon the exercise of the option, to be a deliverable at the inception of the arrangement and a corresponding amount would be included in allocable arrangement consideration. In addition, if the price of the option includes a significant incremental discount, the discount would be included as a deliverable at the inception of the arrangement.

Allocation of Arrangement Consideration

Arrangement consideration that is fixed or determinable is allocated among the separate units of accounting using the relative selling price method. The applicable revenue recognition criteria in ASC 605 are applied to each of the separate units of accounting in determining the appropriate period and pattern of recognition. We determine the selling price of a unit of accounting following the hierarchy of evidence prescribed by ASC 605 25. Accordingly, we determine the estimated selling price for units of accounting within each arrangement using vendor specific objective evidence, or VSOE, of selling price, if available, third party evidence, or TPE, of selling price if VSOE is not available, or best estimate of selling price, or BESP, if neither VSOE or TPE is available. We have only used BESP to estimate selling price, since we have not had VSOE or TPE of selling price for any units of accounting to date. Determining BESP for a unit of accounting requires significant judgment. In developing the BESP for a unit of accounting, we consider applicable market conditions and relevant entity specific factors, including factors that were contemplated in negotiating the applicable agreement and estimated costs. We validate BESP for units of accounting by evaluating whether changes in the key assumptions used by us to determine the BESP will have a significant effect on the allocation of arrangement consideration between multiple units of accounting.

Pattern of Recognition

We will recognize the arrangement’s consideration allocated to each unit of accounting when all of the revenue recognition criteria in ASC 605 are satisfied for that particular unit of accounting. We will recognize revenue associated with licenses, license options, or the discount related to a license option upon (i) delivery of the license or (ii) the earlier of exercise or expiration of the license option, if the underlying license has standalone value from the other deliverables to be provided after delivering that license. If the license does not have standalone value, the amounts allocated to the license will be combined with the related undelivered items as a single unit of accounting.

We will recognize the amounts associated with collaboration research and development services, joint research committees, or other services ratably over the associated period of performance. If there is no discernible pattern of performance or objectively measurable performance measures do not exist, then we recognize revenue under the arrangement on a straight line basis over the period that we are expected to complete our performance obligations.

Conversely, if the pattern of performance in which the service is provided to the collaboration partner can be determined and objectively measureable performance exists, then we would recognize revenue under the arrangement using the proportional performance method. Revenue to be recognized would be limited to the lesser of the cumulative amount of payments received or the cumulative revenue earned determined using the straight line method or proportional performance, as applicable, as of the period end date.

Recognition of Milestones and Royalties

At the inception of an arrangement that includes milestone payments, we evaluate whether each milestone is substantive and at risk to both parties on the basis of the contingent nature of the milestone. This evaluation includes an assessment of whether: (1) the consideration is commensurate with either our performance to achieve the milestone or the enhancement of the value of the delivered item(s) as a result of a specific outcome resulting from our performance to achieve the milestone, (2) the consideration relates solely to past performance, and (3) the consideration is reasonably relative to all of the deliverables and payment terms within the arrangement. We evaluate factors such as clinical, regulatory, commercial, and other risks that must be overcome to achieve the respective

milestones and the level of effort and investment required to achieve the respective milestones in making this assessment. There is considerable judgment involved in determining whether a milestone satisfies all of the criteria required to conclude that a milestone is substantive. In accordance with ASC Topic 605 28, Revenue Recognition—Milestone Method, or ASC 605 28, a clinical or regulatory milestone that is considered substantive will be recognized as revenue in its entirety upon successful accomplishment of the milestone, assuming all other revenue recognition criteria are met. Milestones that are not considered substantive would be recognized as revenue over the remaining period of performance, assuming all other revenue recognition criteria are met. Revenue from a commercial milestone payment will be accounted for as royalties and recorded as revenue upon achievement of the milestone, assuming all other revenue recognition criteria are met.

We will recognize royalty revenue in the period of sale of the related product(s), based on the underlying contract terms, provided that the reported sales are reliably measurable, we have no remaining performance obligations, and assuming all other revenue recognition criteria are met.

Prepaid and Accrued Research and Development Expenses

As part of the process of preparing our consolidated financial statements, we are required to estimate our prepaid and accrued research and development expenses. This process involves reviewing open contracts and purchase orders, communicating with our personnel to identify services that have been performed on our behalf and estimating the level of service performed and the associated cost incurred for the service when we have not yet been invoiced or otherwise notified of the actual cost. The majority of our service providers invoice us monthly in arrears for services performed. We make estimates of our prepaid and accrued research and development expenses as of each balance sheet date in our consolidated financial statements based on facts and circumstances known to us at that time. We confirm the accuracy of our estimates with the service providers and make adjustments if necessary. Examples of estimated prepaid and accrued research and development expenses include expenses for:

- CROs in connection with clinical studies;
- investigative sites in connection with clinical studies;
- vendors in connection with preclinical development activities; and
- vendors related to product manufacturing, development and distribution of clinical materials.

We base our expenses related to clinical studies on our estimates of the services received and efforts expended pursuant to contracts with multiple CROs that conduct and manage clinical studies on our behalf. The financial terms of these agreements are subject to negotiation, vary from contract to contract and may result in uneven payment flows. The scope of services under these contracts can be modified and some of the agreements may be cancelled by either party upon written notice. There may be instances in which payments made to our vendors will exceed the level of services provided and result in a prepayment of the clinical expense. Payments under some of these contracts depend on factors such as the successful enrollment of subjects and the completion of clinical study milestones. In accruing service fees, we estimate the time period over which services will be performed and the level of effort to be expended in each period. If the actual timing of the performance of services or the level of effort varies from our estimate, we adjust the accrual or prepaid accordingly.

Although we do not expect our estimates to be materially different from amounts actually incurred, if our estimates of the status and timing of services performed differ from the actual status and timing of services performed we may report amounts that are too high or too low in any particular period. To date, there have been no material differences between our estimates and the amount actually incurred.

Stock-Based Compensation

Stock-Based Awards

We issue stock-based awards to employees and non-employees, generally in the form of stock options, restricted stock, RSUs and shares of common stock. We account for our stock-based compensation awards in accordance with Financial Accounting Standards Board, (FASB) ASC Topic 718, Compensation—Stock Compensation, or ASC 718. ASC 718 requires all stock-based payments to employees, including grants of employee stock options and restricted stock and modifications to existing stock awards, to be recognized in the statements of operations and comprehensive loss based on their fair values. We account for stock-based awards to non-employees in accordance with ASC Topic 505-50, Equity-Based-Payments to Non-Employees, or ASC 505-50, which requires the fair value of the award to be re-measured at fair value until a performance commitment is reached or counterparty performance is complete. Described below is the methodology we have utilized in measuring stock-based compensation expense. Stock option, common stock and restricted stock values are determined based on the quoted market price of our comparable public companies.

We estimate the fair value of our stock-based awards of options to purchase shares of common stock to employees and non-employees using the Black-Scholes option pricing model, which requires the input of highly subjective assumptions, including (a) the expected

stock price volatility, (b) the calculation of the expected term of the award, (c) the risk-free interest rate and (d) expected dividends. Due to the lack of company-specific historical and implied volatility data for trading our stock in the public market, we have based our estimate of expected volatility on the historical volatility of a group of similar companies that are publicly traded. The historical volatility is calculated based on a period of time commensurate with the expected term assumption. The computation of expected volatility is based on the historical volatility of a representative group of companies with similar characteristics to our company, including stage of product development and life science industry focus. We are a company in the product development stage with no revenue and the representative group of companies has certain similar characteristics. We believe the group selected has sufficient similar economic and industry characteristics, and includes companies that are most representative of our company. We use the simplified method as prescribed by the SEC Staff Accounting Bulletin No. 107, Share-Based Payment, to calculate the expected term for options granted to employees as we do not have sufficient historical exercise data to provide a reasonable basis upon which to estimate the expected term. The expected term is applied to the stock option grant group as a whole, as we do not expect substantially different exercise or post-vesting termination behavior among our employee population. For options granted to non-employees, we utilize the contractual term of the arrangement as the basis for the expected term assumption. The risk-free interest rate is based on a treasury instrument whose term is consistent with the expected life of the stock options. The expected dividend yield is assumed to be zero as we have never paid dividends and have no current plans to pay any dividends on our common stock, similar to our peer group. We estimate grant date fair value of restricted stock awards with corresponding promissory notes using the Black-Scholes option pricing model. The grant date fair value of restricted stock awards and awards of common stock has been based on the estimated value of our common stock at the date of grant.

Our stock-based awards are subject to either service or performance-based vesting conditions. Compensation expense related to awards to employees with service-based vesting conditions is recognized on a straight-line basis based on the grant date fair value over the associated service period of the award, which is generally the vesting term. Consistent with the guidance in ASC 505-50, compensation expense related to awards to non-employees with service-based vesting conditions is recognized on a straight-line basis based on the then-current fair value at each financial reporting date prior to the measurement date over the associated service period of the award, which is generally the vesting term. Compensation expense related to awards to employees with performance-based vesting conditions is recognized based on the grant date fair value over the requisite service period using the accelerated attribution method to the extent achievement of the performance condition is probable. Consistent with the guidance in ASC 505-50, compensation expense related to awards to non-employees with performance-based vesting conditions is recognized based on the then-current fair value at each financial reporting date prior to the measurement date over the requisite service period using the accelerated attribution method to the extent achievement of the performance condition is probable.

We are also required to estimate forfeitures at the time of grant, and revise those estimates in subsequent periods if actual forfeitures differ from our estimates. We use historical data to estimate pre-vesting forfeitures and record stock-based compensation expense only for those awards that are expected to vest. To the extent that actual forfeitures differ from our estimates, the difference is recorded as a cumulative adjustment in the period the estimates were revised. Stock-based compensation expense recognized in the consolidated financial statements is based on awards that are ultimately expected to vest.

Stock-based compensation expense totaled approximately $1.6 million and $1.2 million for the three months ended September 30, 2016 and 2015, respectively, and approximately $4.1 million and $3.4 million for the nine months ended September 30, 2016, respectively.

We expect the impact of our stock-based compensation expense for stock options and restricted stock granted to employees and non-employees to grow in future periods due to the potential increases in the fair value of our common stock and the increase in the number of grants as a result of an increase in headcount.

Emerging Growth Company Status

The JOBS Act permits an “emerging growth company” to take advantage of an extended transition period to comply with new or revised accounting standards applicable to public companies. We chose to “opt out” of this provision and, as a result, we will comply with new or revised accounting standards as required when they are adopted. This decision to opt out of the extended transition period under the JOBS Act is irrevocable.

Results of Operations

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

	Three Months Ended		Increase
	September 30, 2016	September 30, 2015	(Decrease)
	(In Thousands)		
Operating expenses:			
Research and development	$31,238	$ 15,604	$ 15,634
General and administrative	4,944	$ 4,074	870
Total operating expenses	36,182	19,678	16,504
Loss from operations	(36,182)	(19,678)	16,504
Other income, net	(126)	203	(329)
Net loss	$(36,308)	$ (19,475)	$ 16,833

Research and Development Expenses. Research and development expenses were $31.2 million for the three months ended September 30, 2016, compared to $15.6 million for the three months ended September 30, 2015, an increase of $15.6 million. The increase was primarily due to the following:

	(in millions)
Vadadustat development	$ 14.6
Manufacture of drug substance	0.8
Regulatory and other clinical and non-clinical activities	(1.3)
Total increase related to the continued development of vadadustat	14.1
Headcount, consulting and facilities	1.3
Other	0.2
Total net increase	$ 15.6

General and Administrative Expenses. General and administrative expenses were $4.9 million for the three months ended September 30, 2016, compared to $4.1 million for the three months ended September 30, 2015. The increase of $0.9 million was primarily due to an increase in costs to support our Phase 3 program, including headcount and compensation-related costs, and associated facility-related costs.

Other Income, Net. Other expense, net, was $0.1 million for the three months ended September 30, 2016 and other income, net, was $0.2 million for the three months ended September 30, 2015. Other expense, net for the three months ended September 30, 2016 is primarily comprised of interest income offset by expenses related to the write-off of capitalized software. Other income, net for the three months ended September 30, 2015, is primarily comprised of $0.1 million of interest income and $0.1 million of reimbursements under a services agreement for consulting services.

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

	Nine Months Ended		
	September 30, 2016	September 30, 2015	Increase (Decrease)
	(In Thousands)		
Operating expenses:			
Research and development	$82,350	$ 28,772	$ 53,578
General and administrative	16,066	12,691	3,375
Total operating expenses	98,416	41,463	56,953
Loss from operations	(98,416)	(41,463)	56,953
Other income, net	531	604	(73)
Net loss	$(97,885)	$ (40,859)	$ 57,026

Research and Development Expenses. Research and development expenses were $82.3 million for the nine months ended September 30, 2016, compared to $28.8 million for the nine months ended September 30, 2015, an increase of $53.6 million. The increase was primarily due to the following:

	(in millions)
Vadadustat development	$ 49.3
Manufacture of drug substance	2.4
Regulatory and other clinical and non-clinical activities	(3.1)
Total increase related to the continued development of vadadustat	48.6
Headcount, consulting and facilities	4.6
Other	0.4
Total net increase	$ 53.6

General and Administrative Expenses. General and administrative expenses were $16.1 million for the nine months ended September 30, 2016, compared to $12.7 million for the nine months ended September 30, 2015. The increase of $3.4 million was primarily due to an increase in costs to support our Phase 3 program, including headcount and compensation-related costs, and associated facility-related costs as well as commercial planning costs.

Other Income, Net. Other income, net, was $0.5 million for the nine months ended September 30, 2016, compared to $0.6 million for the nine months ended September 30, 2015. Other income, net for the nine months ended September 30, 2016 is primarily comprised of interest income partially offset by expenses related to the write-off of capitalized software. Other income, net for the nine months ended September 30, 2015, is primarily comprised of $0.3 million of interest income and $0.3 million of reimbursements under a services agreement for consulting services.

Liquidity and Capital Resources

We have incurred losses and cumulative negative cash flows from operations since our inception in February 2007, and as of September 30, 2016, we had an accumulated deficit of $259.3 million. We anticipate that we will continue to incur losses for at least the next several years. We expect that our research and development and general and administrative expenses will continue to increase and, as a result, we will need additional capital to fund our operations, which we may raise through a combination of equity offerings, debt financings, other third-party funding, marketing and distribution arrangements and other collaborations, strategic alliances and licensing arrangements.

We have funded our operations principally through equity offerings. As of September 30, 2016, we had cash and cash equivalents and available for sale securities of approximately $161.3 million. Cash in excess of immediate requirements is invested in accordance with our investment policy, primarily with a view to liquidity and capital preservation. Accordingly, available for sale securities, consisting principally of corporate and government debt securities stated at fair value, are also available as a source of liquidity.

Cash Flows

The following table sets forth the primary sources and uses of cash for each of the periods set forth below:

	Nine Months Ended