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

Form 10-Q

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

For the quarterly period ended September 30, 2014

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: 001-36510

Zafgen, Inc.
(Exact name of registrant as specified in its charter)

Delaware	**20-3857670**
(State or other jurisdiction of incorporation or organization)	**(I.R.S. Employer Identification No.)**

175 Portland Street, 4th Floor
Boston, Massachusetts 02114
(Address of principal executive office) (Zip Code)

Registrant's telephone number, including area code: (617) 622-4003

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

Indicate by check mark whether the registrant has submitted electronically and posted on its corporate Web site, if any, every Interactive Data File required to be submitted and posted pursuant to Rule 405 of Regulation S-T (§ 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 the 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	☒ (Do not check if a smaller reporting company)	Smaller reporting company	☐

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

As of November 11, 2014, there were 22,707,410 shares of the registrant's Common Stock, $0.001 par value per share, outstanding.

FORWARD-LOOKING STATEMENTS

This Quarterly Report on Form 10-Q contains forward-looking statements that involve risks and uncertainties. We make such forward-looking statements pursuant to the safe harbor provisions of the Private Securities Litigation Reform Act of 1995 and other federal securities laws. 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 terminology such as "may," "will," "should," "expects," "intends," "plans," "anticipates," "believes," "estimates," "predicts," "potential," "continue" or the negative of these terms or other comparable terminology. These forward-looking statements include, but are not limited to, statements about:

- the accuracy of our estimates regarding expenses, future revenues and capital requirements;
- our plans to develop and commercialize beloranib as a treatment for obesity that is a co-morbidity of an underlying rare condition such as Prader Willi syndrome; or hypothalamic injury-associated obesity, including craniopharyngioma-associated obesity; or severe obesity in the general population, or at all;
- our ability to advance beloranib into pivotal trials, and successfully complete such clinical trials;
- regulatory developments in the United States and foreign countries;
- the performance of our third-party manufacturers and clinical research organizations;
- our ability to obtain and maintain intellectual property protection for our proprietary assets;
- the size of the potential markets for beloranib and our ability to serve those markets;
- the rate and degree of market acceptance of beloranib for any indication once approved;
- our ability to obtain additional financing;
- the success of competing products that are or become available for the indications that we are pursuing;
- the loss of key scientific or management personnel; and
- other risks and uncertainties, including those listed under Part II, Item 1A. Risk Factors.

Any forward-looking statements in this Quarterly Report on Form 10-Q reflect our current views with respect to future events or to our future financial performance and 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.

Zafgen, Inc.

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PART I — FINANCIAL INFORMATION

Item 1. Financial Statements

Zafgen, Inc.

Consolidated Balance Sheets
(In thousands, except share and per share data)
(Unaudited)

	September 30, 2014	December 31, 2013
Assets		
Current assets:		
Cash and cash equivalents	$ 127,030	$ 35,517
Prepaid expenses and other current assets	1,221	224
Tax incentive receivable	1,218	1,617
Total current assets	129,469	37,358
Property and equipment, net	69	37
Other assets	98	743
Total assets	$ 129,636	$ 38,138
Liabilities, Redeemable Convertible Preferred Stock and Stockholders' Equity (Deficit)		
Current liabilities:		
Accounts payable	$ 1,052	$ 2,015
Accrued expenses	9,885	900
Notes payable, current	684	—
Total current liabilities	11,621	2,915
Notes payable, net of discount, long-term	6,819	—
Total liabilities	18,440	2,915
Commitments and contingencies (Note 7)		
Redeemable convertible preferred stock (Series A, B, C, D and E), $0.001 par value; No shares and 99,292,610 shares authorized at September 30, 2014 and December 31, 2013, respectively; no shares and 94,483,404 shares issued and outstanding at September 30, 2014 and December 31, 2013, respectively; aggregate liquidation preference of $104,588 at December 31, 2013	—	103,797
Stockholders' equity (deficit):		
Preferred stock; $0.001 par value; 5,000,000 and no shares authorized at September 30, 2014 and December 31, 2013, respectively; no shares issued and outstanding at September 30, 2014 and December 31, 2013	—	—
Common stock, $0.001 par value; 115,000,000 shares authorized at September 30, 2014 and December 31, 2013; 22,707,012 and 729,391 shares issued and outstanding at September 30, 2014 and December 31, 2013, respectively	23	1
Additional paid-in capital	205,627	332
Accumulated deficit	(94,454)	(68,907)
Total stockholders' equity (deficit)	111,196	(68,574)
Total liabilities, redeemable convertible preferred stock and stockholders' equity (deficit)	$ 129,636	$ 38,138

The accompanying notes are an integral part of these consolidated financial statements.

Zafgen, Inc.

Consolidated Statements of Operations and Comprehensive Loss
(In thousands, except share and per share data)
(Unaudited)

	Three Months Ended September 30,		Nine Months Ended September 30,	
	2014	2013	2014	2013
Revenue	$ —	$ —	$ —	$ —
Operating expenses:				
Research and development	12,076	2,444	20,046	7,038
General and administrative	2,285	1,080	4,822	2,981
Total operating expenses	14,361	3,524	24,868	10,019
Loss from operations	(14,361)	(3,524)	(24,868)	(10,019)
Other income (expense):				
Interest income	1	—	2	—
Interest expense	(213)	—	(658)	—
Foreign currency transaction gains (losses), net	(116)	20	(23)	(162)
Total other income (expense), net	(328)	20	(679)	(162)
Net loss and comprehensive loss	(14,689)	(3,504)	(25,547)	(10,181)
Accretion of redeemable convertible preferred stock to redemption value	—	(53)	(92)	(160)
Net loss attributable to common stockholders	$ (14,689)	$ (3,557)	$ (25,639)	$ (10,341)
Net loss per share attributable to common stockholders, basic and diluted	$ (0.65)	$ (4.88)	$ (2.97)	$ (14.19)
Weighted average common shares outstanding, basic and diluted	22,707,012	729,391	8,618,793	728,862

The accompanying notes are an integral part of these consolidated financial statements.

Zafgen, Inc.

Consolidated Statements of Cash Flows
(In thousands)
(Unaudited)

	Nine Months Ended September 30,	
	2014	2013
Cash flows from operating activities:		
Net loss	$ (25,547)	$(10,181)
Adjustments to reconcile net loss to net cash used in operating activities		
Stock-based compensation expense	910	252
Non-cash interest expense	36	—
Depreciation expense	10	8
Unrealized foreign currency transaction losses	31	169
Changes in operating assets and liabilities:		
Prepaid expenses and other current assets	(997)	160
Tax incentive receivable	368	(1,230)
Accounts payable	(416)	122
Accrued expenses	9,078	(171)
Net cash used in operating activities	(16,527)	(10,871)
Cash flows from investing activities:		
Purchases of property and equipment	(42)	(9)
Deposits for leased property	(57)	—
Net cash used in investing activities	(99)	(9)
Cash flows from financing activities:		
Proceeds from issuance of redeemable convertible preferred stock, net of issuance costs	442	5,955
Proceeds from issuance of notes payable, net of issuance costs	7,386	—
Payments of debt offering costs	(49)	—
Proceeds from initial public offering costs, net of commissions and underwriting discounts	102,672	—
Payments of initial public offering costs	(2,312)	(165)
Net cash provided by financing activities	108,139	5,790
Net increase (decrease) in cash and cash equivalents	91,513	(5,090)
Cash and cash equivalents at beginning of period	35,517	9,935
Cash and cash equivalents at end of period	$127,030	$ 4,845
Supplemental disclosure of non-cash investing and financing activities:		
Accretion of redeemable convertible preferred stock to redemption values	$ 92	$ 160
Deferred offering costs included in accounts payable and accrued expenses	$ —	$ 14
Conversion of redeemable preferred stock to common stock	$104,331	$ —
Supplemental disclosure of cash flow information:		
Cash paid for interest	$ 480	$ —

The accompanying notes are an integral part of these consolidated financial statements.

Zafgen, Inc.

Notes to the Consolidated Financial Statements
(Amounts in thousands, except share and per share data)
(Unaudited)

1. Nature of the Business and Basis of Presentation

Zafgen, Inc. (the "Company") was incorporated on November 22, 2005 under the laws of the State of Delaware. The Company is a biopharmaceutical company dedicated to significantly improving the health and well-being of patients affected by obesity. Beloranib, the Company's lead product candidate, is a novel, first-in-class, twice-weekly subcutaneous injection being developed for the treatment of multiple indications, including obesity and hyperphagia in Prader-Willi syndrome patients, hypothalamic injury-associated obesity including craniopharyngioma-associated obesity, and severe obesity in the general population. Since its inception, the Company has devoted substantially all of its efforts to research and development, recruiting management, acquiring operating assets and raising capital.

The Company was previously classified as a "development stage entity" in the Accounting Standards Codification and, as such, was required to present inception-to-date information in the Company's consolidated statements of operations and comprehensive loss, redeemable convertible preferred stock and stockholders' deficit, and cash flows. In June 2014, the Financial Accounting Standards Board ("FASB") issued an accounting standards update that eliminates the concept of a development stage entity from U.S. generally accepted accounting principles and removes the related incremental reporting requirements. See Note 2 below for additional information on this new standard. The Company elected to early adopt the new standard. Accordingly, in contrast to the Company's consolidated financial statements and the notes thereto for the year ended December 31, 2013 included in the Company's Registration Statement on Form S-1 on file with the Securities and Exchange Commission ("SEC"), the consolidated financial statements contained in this report do not include inception-to-date information.

The Company is subject to risks common to companies in the biotechnology industry including, but not limited to, new technological innovations, protection of proprietary technology, dependence on key personnel, compliance with government regulations and the need to obtain additional financing. Product candidates currently under development will require significant additional research and development efforts, including extensive pre-clinical and clinical testing and regulatory approval, prior to commercialization. These efforts require significant amounts of additional capital, adequate personnel infrastructure, and extensive compliance-reporting capabilities.

The Company's product candidates are all in the development stage. There can be no assurance that the Company's research and development will be successfully completed, that adequate protection for the Company's intellectual property will be obtained, that any products developed will obtain necessary government regulatory approval or that any approved products will be commercially viable. Even if the Company's product development efforts are successful, it is uncertain when, if ever, the Company will generate significant revenue from product sales. The Company operates in an environment of rapid change in technology and substantial competition from pharmaceutical and biotechnology companies. In addition, the Company is dependent upon the services of its employees and consultants.

The consolidated financial statements include the accounts of the Company and its wholly owned subsidiaries Zafgen Securities Corporation, Zafgen Australia Pty Limited, and Zafgen Animal Health, LLC. All significant intercompany balances and transactions have been eliminated.

The accompanying consolidated financial statements have been prepared in conformity with accounting principles generally accepted in the United States of America ("GAAP").

On June 24, 2014, the Company completed an initial public offering ("IPO") of its common stock, which resulted in the sale of 6,900,000 shares at a price of $16.00 per share. The Company received net proceeds from the IPO of approximately $102,672 based upon the price of $16.00 per share and after deducting underwriting discounts and commissions paid by the Company. The Company also incurred offering costs of $2,508 related to the IPO.

Unaudited Interim Financial Information

The consolidated balance sheet at December 31, 2013 was derived from audited financial statements, but does not include all disclosures required by GAAP. The accompanying unaudited consolidated financial statements as of September 30, 2014, and for the three months and nine months ended September 30, 2014 and 2013, have been prepared by the

Company, pursuant to the rules and regulations of the SEC for interim financial statements. Certain information and footnote disclosures normally included in financial statements prepared in accordance with GAAP have been condensed or omitted pursuant to such rules and regulations. However, the Company believes that the disclosures are adequate to make the information presented not misleading. These consolidated financial statements should be read in conjunction with the Company's audited consolidated financial statements and the notes thereto for the year ended December 31, 2013, included in the Company's Registration Statement on Form S-1, File Number 333-195391 on file with the SEC. In the opinion of management, all adjustments, consisting only of normal recurring adjustments necessary for a fair statement of the Company's consolidated financial position as of September 30, 2014 and consolidated results of operations for the three and nine months ended September 30, 2014 and 2013 and consolidated cash flows for the nine months ended September 30, 2014 and 2013 have been made. The results of operations for the three and nine months ended September 30, 2014 are not necessarily indicative of the results of operations that may be expected for the year ending December 31, 2014.

2. Summary of Significant Accounting Policies

Use of Estimates

The preparation of financial statements in conformity with GAAP requires management to make estimates and assumptions that affect the reported amounts of assets and liabilities, the disclosure of contingent assets and liabilities at the date of the consolidated financial statements, and the reported amounts of expenses during the reporting periods. Significant estimates and assumptions reflected in these consolidated financial statements include, but are not limited to, the accrual of research and development expenses and the valuation of common stock prior to the IPO and stock-based awards. Estimates are periodically reviewed in light of changes in circumstances, facts and experience. Actual results could differ from the Company's estimates.

Cash Equivalents

The Company considers all short-term, highly liquid investments with original maturities of ninety days or less at acquisition date to be cash equivalents. Cash equivalents, which consist of money market accounts, are stated at fair value.

Concentration of Credit Risk and of Significant Suppliers

Financial instruments that potentially expose the Company to concentrations of credit risk consist primarily of cash and cash equivalents. The Company has all cash and cash equivalents balances at one accredited financial institution, in amounts that exceed federally insured limits. The Company does not believe that it is subject to unusual credit risk beyond the normal credit risk associated with commercial banking relationships.

The Company is dependent on third-party manufacturers to supply products for research and development activities in its programs. In particular, the Company relies and expects to continue to rely on a small number of manufacturers to supply it with its requirements for the active pharmaceutical ingredients and formulated drugs related to these programs. These programs could be adversely affected by a significant interruption in the supply of active pharmaceutical ingredients and formulated drugs.

Fair Value Measurements

Certain assets and liabilities are carried at fair value under GAAP. Fair value is defined as the exchange price that would be received for an asset or paid to transfer a liability (an exit price) in the principal or most advantageous market for the asset or liability in an orderly transaction between market participants on the measurement date. Valuation techniques used to measure fair value must maximize the use of observable inputs and minimize the use of unobservable inputs. Financial assets and liabilities carried at fair value are to be classified and disclosed in one of the following three levels of the fair value hierarchy, of which the first two are considered observable and the last is considered unobservable:

- Level 1—Quoted prices in active markets for identical assets or liabilities.
- Level 2—Observable inputs (other than Level 1 quoted prices) such as quoted prices in active markets for similar assets or liabilities, quoted prices in markets that are not active for identical or similar assets or liabilities, or other inputs that are observable or can be corroborated by observable market data.
- Level 3—Unobservable inputs that are supported by little or no market activity and that are significant to determining the fair value of the assets or liabilities, including pricing models, discounted cash flow methodologies and similar techniques.

The Company's cash equivalents of $100,000 and $26,501 as of September 30, 2014 and December 31, 2013, respectively, were carried at fair value based on quoted prices in active markets, a Level 1 measurement. The carrying values of accounts payable and accrued expenses approximate their fair value due to the short-term nature of these liabilities. The Company's carrying value of outstanding debt issued in the first quarter of 2014 approximates fair value based on the recent execution date of the credit facility agreement, and is considered a Level 2 measurement.

Deferred Offering Costs

The Company capitalizes certain legal, accounting and other third-party fees that are directly associated with in-process equity financings as other assets until such financings are consummated. After consummation of the equity financing, these costs are recorded in stockholders' equity (deficit) as a reduction of additional paid-in capital generated as a result of the offering or as a reduction to the carrying value of preferred stock issued. As of December 31, 2013, the Company had recorded $743 of deferred offering costs, included in other assets in the accompanying consolidated balance sheet in contemplation of the Company's IPO of its common stock which closed in June 2014. The Company has no deferred offering costs as of September 30, 2014.

Property and Equipment

Property and equipment are stated at cost less accumulated depreciation. Depreciation expense is recognized using the straight-line method over a five-year estimated useful life for both furniture and fixtures and office equipment. Expenditures for repairs and maintenance of assets are charged to expense as incurred. Upon retirement or sale, the cost and related accumulated depreciation of assets disposed of are removed from the accounts and any resulting gain or loss is included in loss from operations.

Impairment of Long-Lived Assets

Long-lived assets consist of property and equipment. Long-lived assets to be held and used are tested for recoverability whenever events or changes in business circumstances indicate that the carrying amount of the assets may not be fully recoverable. Factors that the Company considers in deciding when to perform an impairment review include significant underperformance of the business in relation to expectations, significant negative industry or economic trends, and significant changes or planned changes in the use of the assets. If an impairment review is performed to evaluate a long-lived asset for recoverability, the Company compares forecasts of undiscounted cash flows expected to result from the use and eventual disposition of the long-lived asset to its carrying value. An impairment loss would be recognized when estimated undiscounted future cash flows expected to result from the use of an asset are less than its carrying amount. The impairment loss would be based on the excess of the carrying value of the impaired asset over its fair value, determined based on discounted cash flows. To date, the Company has not recorded any impairment losses on long-lived assets.

Research and Development Costs

Research and development costs are expensed as incurred. Included in research and development expenses are wages, stock-based compensation and benefits of employees, third-party license fees and milestones and other operational costs related to the Company's research and development activities, including facility-related expenses and external costs of outside vendors engaged to conduct both pre-clinical studies and clinical trials. The Company records research and development expenses net of any research and development tax incentives the Company is entitled to receive from government authorities.

Research Contract Costs and Accruals

The Company has entered into various research and development contracts with research institutions and other companies both inside and outside of the United States. These agreements are generally cancelable, and related payments are recorded as research and development expenses as incurred. The Company records accruals for estimated ongoing research costs. When evaluating the adequacy of the accrued liabilities, the Company analyzes progress of the studies, including the phase or completion of events, invoices received and contracted costs. Significant judgments and estimates are made in determining the accrued balances at the end of any reporting period. Actual results could differ from the Company's estimates. The Company's historical accrual estimates have not been materially different from the actual costs.

Patent Costs

All patent-related costs incurred in connection with filing and prosecuting patent applications are recorded as general and administrative expenses as incurred, as recoverability of such expenditures is uncertain.

Accounting for Stock-Based Compensation

The Company measures all stock options and other stock-based awards granted to employees and directors at the fair value on the date of the grant using the Black-Scholes option-pricing model. The fair value of the awards is recognized as expense, net of estimated forfeitures, over the requisite service period, which is generally the vesting period of the respective award. The straight-line method of expense recognition is applied to all awards with service-only conditions.

For stock-based awards granted to consultants and nonemployees, compensation expense is recognized over the period during which services are rendered by such consultants and nonemployees until completed. At the end of each financial reporting period prior to completion of the service, the fair value of these awards is re-measured using the then-current fair value of the Company's common stock and updated assumption inputs in the Black-Scholes option-pricing model.

The Company classifies stock-based compensation expense in its consolidated statement of operations and comprehensive loss in the same manner in which the award recipient's payroll costs are classified or in which the award recipients' service payments are classified.

The Company recognizes compensation expense for only the portion of awards that are expected to vest. In developing a forfeiture rate estimate, the Company has considered its historical experience to estimate pre-vesting forfeitures for service-based awards. The impact of a forfeiture rate adjustment will be recognized in full in the period of adjustment, and if the actual forfeiture rate is materially different from the Company's estimate, the Company may be required to record adjustments to stock-based compensation expense in future periods.

Income Taxes

The Company accounts for income taxes using the asset and liability method, which requires the recognition of deferred tax assets and liabilities for the expected future tax consequences of events that have been recognized in the consolidated financial statements or in the Company's tax returns. Deferred taxes are determined based on the difference between the financial statement and tax basis of assets and liabilities using enacted tax rates in effect in the years in which the differences are expected to reverse. Changes in deferred tax assets and liabilities are recorded in the provision for income taxes. The Company assesses the likelihood that its deferred tax assets will be recovered from future taxable income and, to the extent it believes, based upon the weight of available evidence, that it is more likely than not that all or a portion of deferred tax assets will not be realized, a valuation allowance is established through a charge to income tax expense. Potential for recovery of deferred tax assets is evaluated by estimating the future taxable profits expected and considering prudent and feasible tax planning strategies.

The Company accounts for uncertainty in income taxes recognized in the consolidated financial statements by applying a two-step process to determine the amount of tax benefit to be recognized. First, the tax position must be evaluated to determine the likelihood that it will be sustained upon external examination by the taxing authorities. If the tax position is deemed more-likely-than-not to be sustained, the tax position is then assessed to determine the amount of benefit to recognize in the consolidated financial statements. The amount of the benefit that may be recognized is the largest amount that has a greater than 50% likelihood of being realized upon ultimate settlement. The provision for income taxes includes the effects of any resulting tax reserves, or unrecognized tax benefits, that are considered appropriate as well as the related net interest and penalties.

Segment Data

The Company manages its operations as a single segment for the purposes of assessing performance and making operating decisions. The Company's singular focus is on advancing novel therapeutics for patients suffering from severe obesity and obesity-related disorders. No revenue has been generated since inception, and all tangible assets are held in the United States.

Comprehensive Loss

Comprehensive loss includes net loss as well as other changes in stockholders' equity (deficit) that result from transactions and economic events other than those with stockholders. For the three and nine months ended September 30, 2014 and 2013, there was no difference between net loss and comprehensive loss.

Net Income (Loss) Per Share

Upon the closing of the Company's IPO in June 2014, all of the Company's outstanding redeemable convertible preferred shares were converted into shares of common stock. Prior to this conversion, the Company followed the two-class method when computing net income (loss) per share as the Company had issued shares that meet the definition of participating securities. The two-class method determines net income (loss) per share for each class of common and participating securities according to dividends declared or accumulated and participation rights in undistributed earnings. The two-class method requires income available to common shareholders for the period to be allocated between common and participating securities based upon their respective rights to receive dividends as if all income for the period had been distributed. The Company's redeemable convertible preferred shares contractually entitled the holders of such shares to participate in dividends, but did not contractually require the holders of such shares to participate in losses of the Company. Accordingly, the two-class method did not apply for periods in which the Company reported a net loss or a net loss attributable to common shareholders resulting from dividends or accretion related to its redeemable convertible preferred shares.

Basic net income (loss) per share attributable to common shareholders is computed by dividing the net income (loss) attributable to common shareholders by the weighted average number of common shares outstanding for the period. Diluted net income (loss) per share attributable to common shareholders is computed by dividing the diluted net income (loss) attributable to common shareholders by the weighted average number of common shares, including potential dilutive common shares assuming the dilutive effect of outstanding stock options and unvested restricted common shares, as determined using the treasury stock method. For periods in which the Company has reported net losses, diluted net loss per common share attributable to common shareholders is the same as basic net loss per common share attributable to common shareholders, since dilutive common shares are not assumed to have been issued if their effect is antidilutive.

The Company reported a net loss attributable to common stockholders for the three and nine months ended September 30, 2014 and 2013.

The following common stock equivalents outstanding as of September 30, 2014 and 2013, were excluded from the computation of diluted net loss per share for the three and nine months ended September 30, 2014 and 2013, because they had an anti-dilutive impact:

	As of September 30,	
	2014	2013
Options to purchase common stock	1,839,895	1,283,264
Redeemable convertible preferred stock	—	78,372,931
Total options and redeemable convertible preferred stock exercisable or convertible into common stock	1,839,895	79,656,195

Recently Issued and Adopted Accounting Pronouncements

In June 2014, the FASB issued Accounting Standards Update ("ASU") No. 2014-10, Development Stage Entities (Topic 915): Elimination of Certain Financial Reporting Requirements, Including an Amendment to Variable Interest Entities Guidance in Topic 810, Consolidation. The amendments in this guidance remove all incremental financial reporting requirements for development stage entities. Among other changes, this guidance will no longer require development stage entities to present inception-to-date information about income statement line items, cash flows, and equity transactions. This guidance is effective for public companies in the first annual period beginning after December 15, 2014. Early application is permitted for interim and annual periods for which financial statements have not yet been issued. The Company elected to apply this disclosure guidance to its consolidated financial statements for the three months ended September 30, 2014 and as a result, no longer discloses inception-to-date information in its Consolidated Statements of Operations and Comprehensive Loss, Cash Flows and Stockholders' Deficit and the related notes thereto.

In August 2014, the FASB issued ASU No. 2014-15, Presentation of Financial Statements — Going Concern (Subtopic 205-40). The new guidance addresses management's responsibility to evaluate whether there is substantial doubt about an entity's ability to continue as a going concern and to provide related footnote disclosures. 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. The standard will be effective for the first interim period within annual reporting periods beginning after December 15, 2016. Early adoption is permitted. The Company is evaluating the effect that this guidance will have on its consolidated financial statements.

3. Accrued Expenses

Accrued expenses consisted of the following:

	September 30, 2014	December 31, 2013
Accrued licensing milestones	$ 6,700	$ —
Accrued research and development expenses	1,759	616
Accrued payroll and related expenses	741	49
Accrued professional fees	542	196
Accrued interest	51	—
Accrued other	92	39
	$ 9,885	$ 900

4. Notes Payable

On March 31, 2014, the Company entered into a loan and security agreement with Oxford Finance LLC and Midcap Financial (the "Credit Facility"). The Credit Facility provides for initial borrowings of $7,500 under a term loan ("Term Loan A") and additional borrowings of up to $12,500 under other term loans, for a maximum of $20,000. On March 31, 2014, the Company received proceeds of $7,500 from the issuance of promissory notes under the Term Loan A. Of the additional $12,500 amount that was available, $7,500 ("Term Loan B") was available to be drawn down until September 30, 2014 and $5,000 ("Term Loan C") was available to be drawn down for a 30-day period upon the completion of the Company's IPO that occurred in June 2014. The Company elected not to draw down Term Loan B or Term Loan C and these amounts are no longer available to the Company. All promissory notes issued under the Credit Facility are collateralized by substantially all of the Company's personal property, other than its intellectual property.

Upon entering into this Credit Facility, the Company was obligated to make monthly, interest-only payments on any term loans funded under the Credit Facility until December 1, 2014 and, thereafter, to pay 36 consecutive, equal monthly installments of principal and interest from January 1, 2015 through December 1, 2017. As per the terms of the agreement, in June 2014, upon the completion of the Company's IPO, the term of monthly, interest-only payments were extended until June 1, 2015. Outstanding term loans under the Credit Facility bear interest at an annual rate of 8.1%. In addition, a final payment equal to 6.0% of any amounts drawn under the Credit Facility is due upon the earlier of the maturity date, acceleration of the term loans or prepayment of all or part of the term loans. The Company accrues the amount due relating to Term Loan A of $450, to outstanding debt by charges to interest expense using the effective-interest method from the date of issuance through the maturity date.

Term Loan A was recorded in the balance sheet net of debt discount of $114 that was related to fees assessed by the lender at the time of borrowing. The debt discount is being accreted to the principal amount of the debt. In addition, deferred financing costs of $49 are being amortized to interest expense using the effective-interest method over the same term. For both the three and nine months ended September 30, 2014, the Company recorded additional interest expense of $17 and $36, respectively, related the accretion of the debt discount and amortization of deferred financing costs.

The Company was obligated to pay a separate fee upon any IPO; a sale of substantially all of the Company's assets; or a merger, reorganization or sale of the Company's voting equity securities where existing voting stockholders hold less than 50% of voting equity securities after such transaction. As of September 30, 2014, the Company recorded interest expense of $225 relating to the fee payable upon completion of the Company's IPO in June 2014.

There are no financial covenants associated with the debt facility; however, there are negative covenants restricting the Company's activities, including limitations on dispositions, mergers or acquisitions; encumbering or granting a security interest in its intellectual property; incurring indebtedness or liens; paying dividends; making certain investments; and certain other business transactions.

The Credit Facility also includes events of default, the occurrence and continuation of any of which provides the lenders the right to exercise remedies against the Company and the collateral securing the loans under the Credit Facility, including cash. These events of default include, among other things, failure to pay any amounts due under the Credit Facility, insolvency, the occurrence of a material adverse event, the occurrence of any default under certain other indebtedness and a final judgment against the Company in an amount greater than $250.

Estimated future principal payments due under the Term Loan A are as follows:

Years Ending December 31,	
Remainder of 2014	$ —
2015	1,381
2016	2,936
2017	3,183
Total	$7,500

During the three and nine months ended September 30, 2014, the Company recognized $213 and $658, respectively, of interest expense related to the Credit Facility.

The Company had no debt outstanding as of December 31, 2013.

5. Stockholders' Equity

On June 5, 2014, the Company effected a 1-for-6.28 reverse stock split of its issued and outstanding shares of common stock and a proportional adjustment to the existing conversion ratios for each series of redeemable convertible preferred stock. Accordingly, all share and per share amounts for all periods presented in these consolidated financial statements and notes thereto have been adjusted retroactively, where applicable, to reflect this reverse stock split and adjustment of the redeemable convertible preferred stock conversion ratios.

On June 24, 2014, the Company completed an IPO of its common stock, which resulted in the sale of 6,900,000 shares at a price of $16.00 per share. The Company received net proceeds from the IPO of approximately $102,672 based upon the price of $16.00 per share and after deducting underwriting discounts and commissions paid by the Company. The Company also incurred offering costs of $2,508 related to the IPO.

Upon closing of the IPO, all outstanding shares of the Company's redeemable convertible preferred stock were converted into 15,077,621 shares of common stock.

As of September 30, 2014, the Company's Certificate of Incorporation, as amended and restated, authorizes the Company to issue 5,000,000 shares of $0.001 par value preferred stock. The rights, preferences, restrictions, qualifications and limitations of such stock are determined by the board.

As of September 30, 2014, and December 31, 2013, the Company's Certificate of Incorporation, as amended and restated, authorizes the Company to issue 115,000,000 shares of $0.001 par value common stock.

During the nine months ended September 30, 2013, the Company reacquired and retired 6,635 shares of restricted common stock, at cost, that were forfeited by a former employee.

6. Stock-Based Awards

Stock Option Plans

The Company's Amended and Restated 2006 Stock Option Plan (the "2006 Plan") provided for the Company to sell or issue common stock or restricted common stock, or to grant incentive stock options or nonqualified stock options for the purchase of common stock, to employees, members of the board of directors and consultants of the Company. The 2006 Plan is administered by the board of directors, or at the discretion of the board of directors, by a committee of the board. The exercise prices, vesting and other restrictions are determined at the discretion of the board of directors, or a committee of the board if so delegated, except that the exercise price per share of stock options may not be less than 100% of the fair market value of

the share of common stock on the date of grant and the term of stock option may not be greater than ten years. The total number of shares of common stock that could be issued under the 2006 Plan was 1,889,150 shares. Upon closing of the Company's IPO, 168,221 shares reserved and not then subject to outstanding options were transferred to the 2014 Stock Option and Incentive Plan, and no further awards will be made under the 2006 Plan.

On June 5, 2014, the Company's stockholders approved the 2014 Stock Option and Incentive Plan (the "2014 Stock Option Plan"), which became effective upon the completion of the IPO of the Company's shares of common stock in June 2014. The 2014 Stock Option Plan provides for the grant of stock options, stock appreciation rights, restricted stock, restricted stock units, unrestricted stock, performance-share awards and cash-based awards. The number of shares initially reserved for issuance under the 2014 Stock Option Plan is 2,168,221 shares of common stock and may be increased by the number of shares under the 2006 Plan that are not needed to fulfill the Company's obligations for awards issued under the 2006 Plan as a result of forfeiture, expiration, cancellation, termination or net issuances of awards thereunder. The number of shares of common stock that may be issued under the plan is also subject to increase on the first day of each fiscal year by the lesser of (i) 4% of the Company's outstanding shares of common stock as of that date, or (ii) an amount determined by the board of directors.

The Company generally grants stock-based awards with service conditions only ("service-based" awards).

As required by the 2006 Plan and 2014 Stock Option Plan, the exercise price for stock options granted is not to be less than the fair value of common shares as of the date of grant. Prior to the IPO, the value of common stock was determined by the board of directors by taking into consideration its most recently available valuation of common shares performed by management and the board of directors as well as additional factors which might have changed since the date of the most recent contemporaneous valuation through the date of grant.

For the three months ended September 30, 2014, the Company did not grant any stock options.

2014 Employee Stock Purchase Plan

On June 5, 2014, the Company's stockholders approved the 2014 Employee Stock Purchase Plan. A total of 265,000 shares of common stock were reserved for issuance under this plan. The 2014 Employee Stock Purchase Plan became effective upon the completion of the IPO of the Company's shares of common stock. The first offering period commenced on September 1, 2014. The per share purchase price for offerings is equal to the lesser of 85% of the closing market price of the Company's common stock on the first day or last day of the offering period. As of September 30, 2014, there are 265,000 shares of common stock available for issuance to participating employees under the plan.

Stock Option Valuation

The fair value of each stock option grant is estimated on the date of grant using the Black-Scholes option-pricing model. The Company historically has been a private company and lacks company-specific historical and implied volatility information. Therefore, it estimates its expected stock volatility based on the historical volatility of a publicly traded set of peer companies and expects to continue to do so until such time as it has adequate historical data regarding the volatility of its own traded stock price. The expected term of the Company's stock options has been determined utilizing the "simplified" method for awards that qualify as "plain-vanilla" options. The expected term of stock options granted to nonemployees is equal to the contractual term of the option award. The risk-free interest rate is determined by reference to the U.S. Treasury yield curve in effect at the time of grant of the award for time periods approximately equal to the expected term of the award. Expected dividend yield is based on the fact that the Company has never paid cash dividends and does not expect to pay any cash dividends in the foreseeable future.

As of September 30, 2014, there were outstanding unvested service-based stock options held by nonemployees for the purchase of 17,513 shares of common stock. Additionally as of September 30, 2014, there were outstanding unvested performance-based stock options held by nonemployees for the purchase of 796 shares of common stock.

Stock-based Compensation

The Company recorded stock-based compensation expense related to stock options and restricted common stock in the following expense categories of its statements of operations:

	Three Months Ended September 30,		Nine Months Ended September 30,	
	2014	2013	2014	2013
Research and development	$ 91	$ 40	$ 259	$ 109
General and administrative	375	59	651	143
	$ 466	$ 99	$ 910	$ 252

As of September 30, 2014, the Company had an aggregate of $6,224 of unrecognized stock-based compensation cost, which is expected to be recognized over a weighted average period of 3.4 years.

7. Commitments and Contingencies

Leases

On May 15, 2014, the Company entered into a new lease for office space in Boston, Massachusetts, effective as of July 28, 2014, with a term expiring July 31, 2017 and an option to extend the lease for three additional years.

Future minimum lease payments for its operating leases as of September 30, 2014, are as follows:

Year Ending December 31,	
Remainder of 2014	$ 57
2015	229
2016	235
2017	139
Total	$660

During the three months ended September 30, 2014 and 2013, the Company recognized $52 and $23, respectively, of rental expense related to office space. During the nine months ended September 30, 2014 and 2013, the Company recognized $104 and $87, respectively, of rental expense related to office space.

Intellectual Property Licenses

The Company has acquired exclusive rights to develop patented compounds and related know-how for beloranib under two licensing agreements with two third parties in the course of its research and development activities. The licensing rights obligate the Company to make payments to the licensors for license fees, milestones, license maintenance fees and royalties. The Company is also responsible for patent prosecution costs.

As of September 30, 2014, the Company has a liability of $6,700 relating to milestones achieved in September 2014, with the initiation of a first Phase 3 clinical trial. Additionally, as of September 30, 2014, the Company is obligated to make milestone payments of up to $12,250 upon reaching certain pre-commercialization milestones, such as clinical trials and government approvals, and up to $12,500 upon reaching certain product commercialization milestones. Under one of the license agreements, the Company is also obligated to pay up to $1,250 with respect to each subsequent licensed product, if any, that is a new chemical entity. In addition, the Company will owe single-digit royalties on sales of commercial products developed using these licensed technologies, if any. The Company is also obligated to pay to the licensors a percentage of fees received if and when the Company sublicenses the technology. As of September 30, 2014, the Company has not yet developed a commercial product using the licensed technologies and it has not entered into any sublicense agreements for the technologies.

Indemnification Agreements

In the ordinary course of business, the Company may provide indemnification of varying scope and terms to vendors, lessors, business partners, and other parties with respect to certain matters including, but not limited to, losses arising out of breach of such agreements or from intellectual property infringement claims made by third parties. In addition, the Company

has entered into indemnification agreements with members of its board of directors that will require the Company, among other things, to indemnify them against certain liabilities that may arise by reason of their status or service as directors or officers. The maximum potential amount of future payments the Company could be required to make under these indemnification agreements is, in many cases, unlimited. To date, the Company has not incurred any material costs as a result of such indemnifications. The Company does not believe that the outcome of any claims under indemnification arrangements will have a material effect on its financial position, results of operations or cash flows, and it has not accrued any liabilities related to such obligations in its consolidated financial statements as of September 30, 2014.

8. Retirement Plan

The Company has a Savings Incentive Match Plan for employees. Under the terms of the plan, the Company contributes 2% of an employee's annual base salary, up to a maximum of the annual Internal Revenue Service compensation limits, for all full-time employees.

During the three months ended September 30, 2014 and 2013, the Company recognized $17 and $6, respectively, of expense related to its contributions to this plan. During the nine months ended September 30, 2014 and 2013, the Company recognized $43 and $20, respectively, of expense related to its contributions to the plan.

9. Australia Research and Development Tax Incentive

The Company's wholly owned subsidiary, Zafgen Australia Pty Limited, which conducts core research and development activities on behalf of the Company is eligible to receive a 45% refundable tax incentive for qualified research and development activities. For the three months ended September 30, 2014 and 2013, $91 and $812, respectively, was recorded as a reduction to research and development expenses in the consolidated statements of operations and, for the nine months ended September 30, 2014 and 2013, $368 and $1,230, respectively, was recorded as a reduction to research and development expenses in the consolidated statements of operations. These amounts represented 45% of the Company's qualified research and development spending in Australia. The refund is denominated in Australian dollars and, therefore, the receivable is re-measured into U.S. dollars as of each reporting date. For the three months ended September 30, 2014 and 2013, the Company recorded in its consolidated statements of operations unrealized foreign currency exchange (gains) losses of $95 and $(36), respectively, related to this tax incentive receivable. For the nine months ended September 30, 2014 and 2013, the Company recorded in its consolidated statements of operations unrealized foreign currency exchange (gains) losses of $31 and $169, respectively, related to this tax incentive receivable. As of September 30, 2014 and December 31, 2013, the Company's tax incentive receivable from the Australian government was $1,218 and $1,617, respectively.

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

The following discussion and analysis of our financial condition and results of operations should be read in conjunction with our consolidated financial statements and related notes appearing elsewhere in this Quarterly Report on Form 10-Q and our final prospectus for our initial public offering filed pursuant to Rule 424(b) under the Securities Act of 1933, as amended, with the Securities and Exchange Commission on June 19, 2014, or the Prospectus.

Our actual results and timing of certain events may differ materially from the results discussed, projected, anticipated, or indicated in any forward-looking statements. We caution you that forward-looking statements are not guarantees of future performance and that our actual results of operations, financial condition and liquidity, and the development of the industry in which we operate may differ materially from the forward-looking statements contained in this Quarterly Report on Form 10-Q. In addition, even if our results of operations, financial condition and liquidity, and the development of the industry in which we operate are consistent with the forward-looking statements contained in this Quarterly Report on Form 10-Q, they may not be predictive of results or developments in future periods.

The following information and any forward-looking statements should be considered in light of factors discussed elsewhere in 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.

Overview

We are a biopharmaceutical company dedicated to significantly improving the health and well-being of patients affected by obesity and complex metabolic disorders. Beloranib, our lead product candidate, is a novel, first-in-class, twice-weekly subcutaneous injection being developed for the treatment of multiple indications, including severe obesity in two rare diseases, Prader-Willi syndrome, or PWS, and hypothalamic injury-associated obesity, including craniopharyngioma-associated obesity; and severe obesity in the general population.

Obesity is a complex medical disorder involving appetite dysregulation and altered lipid and energy metabolism that results in excessive accumulation of fat tissue. Weight loss and hunger control are urgently needed for certain subpopulations of obese patients, in which obesity is life-threatening and a co-morbidity of an underlying condition such as PWS and obesity related to injury of the hypothalamus that, while rare, occurs most commonly as a consequence of treatment for craniopharyngioma and other mid-brain tumors. These conditions are characterized by uncontrollable hunger resulting from damage to or impaired functioning of the hypothalamus, an area of the brain responsible for many functions including the neurophysiological drive to eat.

PWS is a rare and complex genetic disorder characterized by physiologic, cognitive and behavioral symptoms, including hyperphagia, and severe obesity. We recently completed two Phase 2a clinical trials evaluating beloranib's ability to reduce body weight and to improve hyperphagia, one in patients with PWS and one in severely obese patients. In our Phase 2a clinical trials, we observed reductions in body weight, body mass and body fat content in both patient populations and reductions in hyperphagia-related behaviors in patients with PWS. In January 2013 and July 2014, the U.S. Food and Drug Administration, or FDA, and European Commission, respectively, granted orphan designation for our application to treat PWS with beloranib. We initiated our Phase 3 clinical program, consisting of two Phase 3 clinical trials, of beloranib in patients with PWS, with the first Phase 3 trial in the United States having started in September 2014. We believe that rare conditions such as PWS afford us an opportunity to rapidly develop and commercialize beloranib using smaller, more focused and less costly clinical trials, relative to those required to develop beloranib for the broader severe obesity population.

Published population studies estimate that the prevalence of PWS in the United States and in the European Union ranges from 1 in 8,000 to 1 in 50,000. The neurophysiological drive to eat in patients with PWS is so powerful that they will go to great lengths to eat large quantities of food, even if it is spoiled, indigestible or unpalatable to others. Unsupervised patients will often eat to the point that it causes serious physical harm or death. As a result, caregivers are often forced to place locks and alarms on refrigerators and pantries that contain food. Despite attempts to control the access to food, the typical adult patient with PWS is morbidly obese and, based on our evaluation of published survival data, has an average life expectancy of 32 years of age. Unfortunately, neither dietary intervention nor currently available pharmaceutical therapies bring meaningful benefit to patients with PWS and, as a result, they experience severe and life-threatening consequences of their condition. Furthermore, existing surgical techniques such as bariatric surgery are contraindicated in PWS.

Since beloranib works to reduce hunger and body weight through a novel mechanism that does not appear to require fully functioning hypothalamic control pathways, we believe that obese patients with conditions in addition to PWS in which pathologically increased hunger is a key component of their condition may respond well to treatment with beloranib. Certain patients suffering from hypothalamic injury-associated obesity display many characteristics of patients with PWS, and sometimes are referred to as having 'acquired PWS'. Hypothalamic injury-associated obesity could include patients with craniopharyngioma or other types of hypothalamic injuries, such as other types of central nervous system tumors and infiltrative diseases such as sarcoidosis. Accordingly, patients with this form of obesity are of interest to our beloranib development program and we are pursuing obesity caused by injury to the hypothalamus as a potential additional indication for the use of beloranib.

In addition to these rare disorders, we also are pursuing clinical development of beloranib as a treatment for severely obese patients in the general population, including patients otherwise eligible for bariatric surgery. Bariatric surgery eligibility criteria generally identify surgical candidates as those patients with body mass indices, or BMIs, greater than 40 kg/m^2, or those with BMIs over 35 kg/m^2 who also have a significant and uncontrolled co-morbid condition. Based on these criteria, it is estimated conservatively that there will be at least 16 million adults in the United States eligible for bariatric surgery by the time beloranib or another methionine aminopeptidase 2, or MetAP2, inhibitor could become available commercially. Bariatric surgery results in significant weight loss, but the financial expense and the potential for complications, adverse events and longer-term side effects limit its overall adoption, with only a few hundred thousand patients in the United States undergoing bariatric surgery each year. Existing pharmacotherapies result in less weight loss than surgical options, and these therapies not only have undesirable side effects, but also have risk of abuse.

Due to the significant barriers associated with bariatric surgery and the limited weight loss potential of currently marketed pharmaceutical products, there is a significant unmet medical need for the treatment of patients with severe obesity. We believe this patient population would benefit from MetAP2 inhibitor treatment through the reduction of body weight and through improvement of other co-morbid conditions. In 2013, we completed a 12-week Phase 2a clinical trial of beloranib administered twice weekly in obese patients. We observed placebo-adjusted weight loss, or weight loss observed beyond that seen in the control arm, of up to 10.3% after 12 weeks of treatment with beloranib. In addition, we observed reductions in levels of low density lipoprotein cholesterol, C-reactive protein and systolic blood pressure. Patients treated with beloranib also reported reduced hunger, as assessed using a visual analog scale, a widely used self-reported measure of hunger and related endpoints. We intend to initiate a Phase 2b clinical trial of beloranib as a treatment for severe obesity in the general population in the fourth quarter of 2014.

We are also evaluating additional proprietary MetAP2 inhibitors beyond beloranib as potential development candidates that would provide increased patient convenience in the form of oral dosing, or an otherwise improved clinical profile. A decision on whether to subsequently advance beloranib into pivotal trials for severe obesity or to leverage the opportunity to advance another MetAP2 inhibitor into early development for severe obesity is anticipated to be made on the basis of results obtained from our Phase 3 clinical program of beloranib in patients with PWS and discussions with regulatory authorities. MetAP2 inhibitors may also have utility in the treatment of other metabolic diseases, such as nonalcoholic steatohepatitis, or NASH, and type 2 diabetes. In a mouse model of diabetes and NASH, our second product candidate, ZGN-839, a MetAP2 inhibitor, reduced the severity of NASH and reduced plasma glucose.

Since our inception in November 2005, we have devoted substantially all of our resources to developing beloranib and ZGN-839, building our intellectual property portfolio, developing our supply chain, business planning, raising capital, and providing general and administrative support for these operations. Prior to our initial public offering, or IPO, in June 2014, we funded our operations primarily through sales of redeemable convertible preferred stock and, to a lesser extent, through the issuances of convertible promissory notes. From our inception through September 30, 2014, we have received gross proceeds of $104.0 million from such transactions. During June 2014, we completed our IPO with net proceeds of $102.7 million.

We have never generated any revenue and have incurred net losses in each year since our inception. We have an accumulated deficit of $94.5 million as of September 30, 2014. Our net losses were $25.5 million for the nine months ended September 30, 2014. These losses to date have resulted principally from costs incurred in connection with in-licensing our product candidates, research and development activities and general and administrative costs associated with our operations. We expect to incur significant expenses and increasing operating losses for the foreseeable future.

We expect that our expenses will increase substantially in connection with our ongoing activities, as we:

- advance the clinical development of beloranib as a treatment for obesity and hyperphagia in patients with PWS through our Phase 3 clinical program;
- advance the clinical development of beloranib as a treatment for hypothalamic injury-associated obesity through our current Phase 2a clinical trial and if such Phase 2a trial is successful, the initiation of pivotal clinical trials;
- initiate Investigational New Drug Application, or IND, enabling studies and clinical development of ZGN-839 and our second-generation MetAP2 inhibitors;
- initiate a Phase 2b clinical trial of beloranib as a treatment for severe obesity in the general population;
- seek to identify additional indications for beloranib;
- seek to obtain regulatory approvals for our product candidates;
- add operational, financial and management information systems;
- add personnel, including personnel to support our product development and future commercialization; and
- maintain, leverage and expand our intellectual property portfolio.

As a result, we will need additional financing to support our continuing operations. Until such time that we can generate significant revenue from product sales, if ever, we expect to finance our operations through a combination of public or private equity or debt financings or other sources, which may include collaborations with third parties. Arrangements with collaborators or others may require us to relinquish rights to certain of our technologies or product candidates. In addition, we may never successfully complete development of any of our product candidates, obtain adequate patent protection for our technology, obtain necessary regulatory approval for our product candidates or achieve commercial viability for any approved product candidates. Adequate additional financing may not be available to us on acceptable terms, or at all. Our failure to raise capital as and when needed would have a negative impact on our financial condition and our ability to pursue our business strategy. We will need to generate significant revenue to achieve profitability, and we may never do so.

We expect that our existing cash and cash equivalents as of September 30, 2014 will enable us to fund our operating expenses and capital expenditures requirements for at least the next 12 months. See "—Liquidity and Capital Resources."

Financial Operations Overview

Revenue

We have not generated any revenue from product sales since our inception, and do not expect to generate any revenue from the sale of products in the near future. If our development efforts result in clinical success and regulatory approval or collaboration agreements with third parties for our product candidates, we may generate revenue from those product candidates.

Operating Expenses

The majority of our operating expenses since inception have consisted primarily of in-licensing costs of beloranib, milestone payments, research and development activities, and general and administrative costs.

Research and Development Expenses

Research and development expenses, which consist primarily of costs associated with our product research and development efforts, are expensed as incurred. Research and development expenses consist primarily of:

- personnel costs, including salaries, related benefits and stock-based compensation for employees engaged in scientific research and development functions;
- third-party contract costs relating to research, formulation, manufacturing, pre-clinical studies and clinical trial activities;
- external costs of outside consultants;
- payments made under our third-party licensing agreements;
- laboratory consumables; and
- allocated facility-related costs.

We have been developing beloranib, ZGN-839, and our second-generation MetAP2 inhibitors, and typically use our employee, consultant and infrastructure resources across our development programs. We track outsourced development costs by product candidate or development program, but we do not allocate personnel costs, external consultant costs, payments made under our licensing agreements or other internal costs to specific development programs or product candidates. We record our research and development expenses net of any research and development tax incentives we are entitled to receive from government authorities.

The following table summarizes our research and development expenses by program:

	Three Months Ended September 30,		Nine Months Ended September 30,	
	2014	2013	2014	2013
	(in thousands)			
Beloranib	$ 10,479	$ 1,656	$15,177	$4,365
ZGN-839 and other early stage development	158	138	668	152
Unallocated expenses	1,439	650	4,201	2,521
Total research and development expenses	$ 12,076	$ 2,444	$20,046	$7,038

Research and development activities are central to our business. Product candidates in later stages of clinical development generally have higher development costs than those in earlier stages of clinical development, primarily due to the increased size and duration of later-stage clinical trials. We expect that our research and development expenses will continue to increase in the foreseeable future as we pursue later stages of clinical development of our product candidates.

We cannot determine with certainty the duration and completion costs of the current or future clinical trials 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 scope, rate of progress, and expense of our ongoing as well as any additional clinical trials and other research and development activities;
- future clinical trial results;
- uncertainties in clinical trial enrollment rate or design;
- significant and changing government regulation;
- the timing and receipt of any regulatory approvals; and
- the FDA's or other regulatory authority's influence on trial design.

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, or another regulatory authority were to require us to conduct clinical trials beyond those that we currently anticipate will be required for the completion of clinical development of a product candidate, or if we experience significant delays in enrollment in any of our clinical trials, we could be required to expend significant additional financial resources and time on the completion of clinical development.

General and Administrative Expenses

General and administrative expenses consist primarily of personnel costs, consisting of salaries, related benefits and stock-based compensation, of our executive, finance, business and corporate development and other administrative functions. General and administrative expenses also include travel expenses, allocated facility-related costs not otherwise included in research and development expenses, and professional fees for auditing, tax and legal services, including legal expenses to pursue patent protection of our intellectual property.

We expect that general and administrative expenses will increase in the future as we expand our operating activities and incur additional costs associated with operating as a public company. These public company related increases will likely include additional costs related to personnel; legal, accounting and audit services; directors' and officers' liability insurance premiums; and investor relations.

Other Income (Expense)

Interest income. Interest income consists of interest earned on our cash and cash equivalents. Our interest income has not been significant due to low interest earned on invested balances. We anticipate that our interest income will increase in the future due to increased balances from cash proceeds from our IPO received in June 2014.

Interest expense. Interest expense consists of interest expense on our outstanding borrowings under a credit facility that we entered into on March 31, 2014, consisting of the stated interest of 8.1% per year due on outstanding borrowings, a final payment of 6% of amounts drawn down that is being recorded as interest expense over the term through the maturity date using the effective-interest method, the amortization of deferred financing costs and debt discount relating to the credit facility and fees payable upon the completion of our IPO in June 2014.

Foreign currency transaction gains (losses), net. Foreign currency transaction gains (losses), net consists of the realized and unrealized gains and losses from foreign currency-denominated cash balances, vendor payables and tax-related receivables from the Australian government. We currently do not engage in hedging activities related to our foreign currency-denominated receivables and payables; as such, we cannot predict the impact of future foreign currency transaction gains and losses on our operating results. See "—Quantitative and Qualitative Disclosures about Market Risk."

Income Taxes

Since our inception in 2005, we have not recorded any U.S. federal or state income tax benefits for the net losses we have incurred in each year or our earned research and development tax credits, due to our uncertainty of realizing a benefit from those items. As of December 31, 2013, we had federal and state net operating loss carryforwards of $10.5 million and $8.2 million, respectively. Our federal net operating loss carryforwards begin to expire in 2026, and our state net operating loss carryforwards began to expire in 2014. We also had federal and state research and development tax credit carryforwards of $4.7 million and $1.3 million, respectively, as of December 31, 2013, which begin to expire in 2026 and 2021, respectively.

Results of Operations

Comparison of Three Months Ended September 30, 2014 and 2013

The following table summarizes our results of operations for the three months ended September 30, 2014 and 2013:

	Three Months Ended September 30,		Increase (Decrease)
	2014	2013	
	(in thousands)		
Revenue	$ —	$ —	$ —
Operating expenses:			
Research and development	12,076	2,444	9,632
General and administrative	2,285	1,080	1,205
Total operating expenses	14,361	3,524	10,837
Loss from operations	(14,361)	(3,524)	(10,837)
Other income (expense):			
Interest income	1	—	1
Interest expense	(213)	—	(213)
Foreign currency transaction gains (losses), net	(116)	20	(136)
Total other income (expense), net	(328)	20	(348)
Net loss	$(14,689)	$(3,504)	$ (11,185)

Research and development expenses

	Three Months Ended September 30,		Increase
	2014	2013	(Decrease)
	(in thousands)		
Direct research and development expenses by program:			
Beloranib:			
Pre-clinical and manufacturing	$ 2,443	$ 971	$ 1,472
Clinical trials	1,336	685	651
Licensing, milestone and licensing maintenance fees	6,700	—	6,700
Subtotal	10,479	1,656	8,823
ZGN-839 and other early stage development	158	138	20
Subtotal	10,637	1,794	8,843
Unallocated expenses:			
Personnel related	794	289	505
Consultants	519	342	177
Other	126	19	107
Subtotal	1,439	650	789
Total research and development expenses	$ 12,076	$ 2,444	$ 9,632

Research and development expenses for the three months ended September 30, 2014 increased $9.6 million compared to the three months ended September 30, 2013. The increase was primarily due to increased costs of $8.8 million associated with our beloranib program and a $0.8 million increase in our unallocated expenses. Of the increase in our beloranib program, pre-clinical and manufacturing costs increased by $1.5 million period over period as a result of our focus on drug manufacturing and other pre-clinical activities related to beloranib in order to prepare for future clinical trials. Additionally, our clinical trial costs related to beloranib increased $0.7 million as a result of the timing of clinical trials in 2013 and 2014. During the 2013 period we had one ongoing clinical trial, our Phase 2a trial in patients with PWS. As of September 30, 2014, we had two ongoing clinical trials, our Phase 2a trial in patients with obesity caused by injury to the hypothalamus and our U.S. Phase 3 trial in patients with PWS. Clinical trial activities undertaken by our Australian subsidiary are recorded net of a 45% research and development tax incentive from the Australian government. Lastly, licensing, milestone and licensing maintenance fees increased $6.7 million due to the achievement of a milestone related to the initiation of a Phase 3 clinical trial in beloranib, which we initiated in September 2014. Unallocated expenses increased period over period primarily due to an increase in personnel related costs of $0.5 million and an increase in consultant expense of $0.2. Personnel related costs increased period over period primarily due to the hiring of new employees to support our development efforts. Consultant costs increased period over period primarily due to expenses incurred in connection with preparation work for our current clinical trials.

General and administrative expenses

	Three Months Ended September 30,		Increase
	2014	2013	(Decrease)
	(in thousands)		
Personnel related	$ 969	$ 312	$ 657
Professional fees	746	672	74
Travel and other	570	96	474
Total general and administrative expenses	$ 2,285	$ 1,080	$ 1,205

General and administrative expenses for the three months ended September 30, 2014 increased $1.2 million compared to the three months ended September 30, 2013. The increase was primarily due to increased personnel related costs of $0.7 million and increased travel and other related costs of $0.5 million period over period. Personnel related costs increased period over period primarily due to hiring additional employees of $0.4 million and increases in stock-based compensation of $0.3 million related to the new employees and to the increase in our common stock value. The increase in travel and other related costs of $0.5 million is primarily due to increased insurance of $0.2 million due to becoming a public company and commercial marketing projects of $0.2 million, as well as information technology-related expenses to support our operating as a public company.

Other income (expense), net

Interest expense. Interest expense for the three months ended September 30, 2014 was related to interest expense on our outstanding borrowings under the credit facility that we entered into on March 31, 2014, consisting of the stated interest of 8.1% per year due on outstanding borrowings of $0.2 million, a final payment of 6% of amounts drawn down that is being recorded as interest expense over the term through the maturity date using the effective-interest method and the amortization of deferred financing costs and debt discount relating to the credit facility, both less than $0.1 million. We had no debt outstanding during 2013.

Foreign currency transaction gains (losses), net. Net foreign currency transaction losses of $0.1 million for the three months ended September 30, 2014 were primarily due to the re-measurement of receivables, denominated in Australian dollars, from the Australian government for research and development tax incentives, reflecting a weakening of the Australian dollar relative to the U.S. dollar.

Comparison of Nine Months Ended September 30, 2014 and 2013

The following table summarizes our results of operations for the nine months ended September 30, 2014 and 2013:

	Nine Months Ended September 30,		Increase (Decrease)
	2014	2013	
	(in thousands)		
Revenue	$ —	$ —	$ —
Operating expenses:			
Research and development	20,046	7,038	13,008
General and administrative	4,822	2,981	1,841
Total operating expenses	24,868	10,019	14,849
Loss from operations	(24,868)	(10,019)	(14,849)
Other income (expense):			
Interest income	2	—	2
Interest expense	(658)	—	(658)
Foreign currency transaction gains (losses), net	(23)	(162)	139
Total other income (expense), net	(679)	(162)	(517)
Net loss	$(25,547)	$(10,181)	$ (15,366)

Research and development expenses

	Nine Months Ended September 30,		Increase (Decrease)
	2014	2013	
	(in thousands)		
Direct research and development expenses by program:			
Beloranib:			
Pre-clinical and manufacturing	$ 5,947	$1,941	$ 4,006
Clinical trials	2,530	2,424	106
Licensing, milestone and licensing maintenance fees	6,700	—	6,700
Subtotal	15,177	4,365	10,812
ZGN-839 and other early stage development	668	152	516
Subtotal	15,845	4,517	11,328
Unallocated expenses:			
Personnel related	1,984	913	1,071
Consultants	1,929	1,511	418
Other	288	97	191
Subtotal	4,201	2,521	1,680
Total research and development expenses	$20,046	$7,038	$ 13,008

Research and development expenses for the nine months ended September 30, 2014 increased $13.0 million compared to the nine months ended September 30, 2013. The increase was primarily due to increased costs of $10.8 million associated with our beloranib program, $0.5 million associated with ZGN-839 and other early-stage development programs (consisting of our second-generation MetAP2 inhibitors), and $1.7 million in our unallocated expenses. Of the increase in our beloranib program, pre-clinical and manufacturing costs increased by $4.0 million period over period as a result of our focus on drug manufacturing and other pre-clinical activities related to beloranib in order to prepare for clinical trials, as well as toxicology studies required for our NDA submission. Additionally, clinical trial expenses for beloranib increased by $0.1 million period over period as a result of timing of our clinical trials in 2013 and 2014. Clinical trial activities undertaken by our Australian subsidiary are recorded net of a 45% research and development tax incentive from the Australian government. Lastly, licensing, milestone and licensing maintenance fees increased $6.7 million due to the achievement of a milestone related to the initiation of a first Phase 3 clinical trial in beloranib, which we initiated in September 2014. Costs related to ZGN-839 and other early-stage development programs increased in 2014 as a result of our increased focus on our early-stage programs in 2014. Unallocated expenses increased period over period primarily due to an increase in personnel related costs of $1.1 million and an increase in consultant expenses of $0.4 million. Personnel related costs increased period over period primarily due to the hiring of new employees of $0.9 million and increased stock-based compensation of $0.2 million. Consultant costs increased period over period primarily due to expenses incurred in connection with regulatory meetings and preparation work for clinical trials.

General and administrative expenses

	Nine Months Ended September 30,		Increase
	2014	2013	(Decrease)
	(in thousands)		
Personnel related	$ 2,157	$ 1,016	$ 1,141
Professional fees	1,772	1,678	94
Travel and other	893	287	606
Total general and administrative expenses	$ 4,822	$ 2,981	$ 1,841

General and administrative expenses for the nine months ended September 30, 2014 increased $1.8 million compared to the nine months ended September 30, 2013. The increase was primarily due to increased personnel related costs of $1.1 million and increased travel and other related costs of $0.6 million period over period. Personnel related costs increased primarily due to the hiring of new employees of $0.6 million and increases in stock-based compensation of $0.5 million related to the new employees and to the increase in our common stock value. The increase in travel and other related costs is primarily due to an increase in directors and officer's insurance of $0.3 million due to becoming a public company, $0.2 million relating to commercial marketing projects as well as various other increases including information technology-related expenses to support our operating as a public company and increased office rent due to the office move in July 2014.

Other income (expense), net

Interest expense. Interest expense for the nine months ended September 30, 2014 was related to interest expense on our outstanding borrowings under the credit facility that we entered into on March 31, 2014, consisting of the stated interest of 8.1% per year due on outstanding borrowings of $0.3 million, a final payment of 6% of amounts drawn down that is being recorded as interest expense over the term through the maturity date using the effective-interest method and the amortization of deferred financing costs and debt discount relating to the credit facility, both less than $0.1 million, and $0.2 million related to a fee of which was due upon the completion of our IPO. We had no debt outstanding during 2013.

Foreign currency transaction gains (losses), net. Net foreign currency transaction losses of $0.2 million for the nine months ended September 30, 2013 were primarily due to the re-measurement of receivables, denominated in Australian dollars, from the Australian government for research and development tax incentives, reflecting both a weakening of the Australian dollar relative to the U.S. dollar and an increase in our receivable balances for such tax incentives during the nine months ended September 30, 2013. During the nine months ended September 30, 2014 the Australian dollar strengthened relative to the U.S. dollar during the first eight months and then weakened dramatically during September 2014 leading to a very minimal net impact on foreign currency transaction gains (losses).

Liquidity and Capital Resources

As of September 30, 2014 and December 31, 2013, we had cash and cash equivalents totaling $127.0 million and $35.5 million, respectively.

Since our inception in November 2005, we have not generated any revenue and have incurred recurring net losses. As of September 30, 2014, we had an accumulated deficit of $94.5 million. We have funded our operations since inception primarily through sales of redeemable convertible preferred stock and, to a lesser extent, through the issuances of convertible promissory notes. In June 2014, we completed an IPO of common stock with net proceeds of $102.7 million.

On March 31, 2014, we entered into a loan and security agreement, or the 2014 Credit Facility, which provides for initial borrowings of $7.5 million and additional borrowings of up to $12.5 million. On that same date, we received proceeds of $7.5 million from the issuance of promissory notes under a term loan as part of the 2014 Credit Facility. Of the additional $12.5 million of borrowings that was available to us, $7.5 million was available to be drawn down until September 30, 2014 and $5.0 million was available to be drawn down for a 30-day period upon the completion of our IPO that occurred in June 2014. We elected not to draw down the $7.5 million or the $5.0 million and these amounts are no longer available to us. All promissory notes issued under the 2014 Credit Facility are collateralized by substantially all of our personal property, other than our intellectual property. There are no financial covenants associated with the debt facility; however, there are negative covenants restricting our activities, including limitations on dispositions, mergers or acquisitions; encumbering or granting a security interest in our intellectual property; incurring indebtedness or liens; paying dividends; making certain investments; and certain other business transactions.

Upon entering into this 2014 Credit Facility, we were obligated to make monthly, interest-only payments on any term loans funded under the 2014 Credit Facility until December 1, 2014 and, thereafter, to pay 36 consecutive, equal monthly installments of principal and interest from January 1, 2015 through December 1, 2017. As per the terms of the agreement, in June 2014, upon the completion of our IPO, the term of monthly, interest-only payments were extended until June 1, 2015. Outstanding term loans under the 2014 Credit Facility bear interest at an annual rate of 8.1%. In addition, a final payment equal to 6.0% of any amounts drawn under the facility is due upon the earlier of the maturity date, acceleration of the term loans or prepayment of all or part of the term loans. We were also obligated to pay a separate fee upon any initial public offering; a sale of substantially all of our assets; or a merger, reorganization or sale of our voting equity securities where existing voting stockholders hold less than 50% of voting equity securities after such transaction. As of September 30, 2014, we recorded interest expense of $225 relating to the fee payable upon completion of our IPO.

The following table summarizes our sources and uses of cash for each of the periods presented below:

	Nine Months Ended September 30,	
	2014	2013
Cash used in operating activities	$ (16,527)	$(10,871)
Cash used in investing activities	(99)	(9)
Cash provided by financing activities	108,139	5,790
Net increase (decrease) in cash and cash equivalents	$ 91,513	$ (5,090)

Net cash used in operating activities

During the nine months ended September 30, 2014, operating activities used $16.5 million of cash, resulting from our net loss of $25.5 million, partially offset by non-cash charges of $1.0 million and net cash provided by changes in our operating assets and liabilities of $8.0 million. Our net loss was primarily attributed to research and development activities related to our beloranib program, licensing milestones and our general and administrative expenses, as we had no revenue in the period. Our net non-cash charges during the nine months ended September 30, 2014 consisted primarily of stock-based compensation expense of $0.9 million. Net cash provided by changes in our operating assets and liabilities during the nine months ended September 30, 2014 consisted primarily of a $9.1 million increase in accrued expenses (of which $6.7 million relates to our licensing milestones), partially offset by a $1.0 million increase in prepaid expenses and other current assets.

During the nine months ended September 30, 2013, operating activities used $10.9 million of cash, primarily resulting from our net loss of $10.2 million and from net cash used by changes in our operating assets and liabilities of $1.1 million, partially offset by non-cash charges of $0.4 million. Our net loss was primarily attributed to research and development activities related to our beloranib and our general and administrative expenses, as we had no revenue in the period. Net cash used by changes in our operating assets and liabilities during the nine months ended September 30, 2013 consisted primarily of a $1.2 million increase in our research and development tax incentive receivable from the Australian government and a $0.2 million decrease in accrued expenses, partially offset by a $0.2 million increase in prepaid expenses and other current assets and $0.1 increase in accounts payable. Our net non-cash charges during the nine months ended September 30, 2013 consisted primarily of stock-based compensation expense of $0.3 million and unrealized foreign currency transaction gains of $0.2 million.

Our cash provided by operating activities is generally affected by the amount of our net loss, changes in operating asset accounts and add-backs of non-cash expense items such as stock-based compensation expense and unrealized foreign currency gains and losses. Changes in our operating asset accounts are generally driven by the timing of payments to our vendors.

Net cash used in investing activities

During the nine months ended September 30, 2014, we paid a security deposit on our new office lease for $0.1 million. We also used a small amount of cash during the nine months ended September 30, 2014 and 2013 related to purchases of property and equipment.

Net cash provided by financing activities

During the nine months ended September 30, 2014, net cash provided by financing activities was $108.1 million as a result of proceeds, net of underwriting discounts and commissions, of $102.7 million from our IPO, $7.4 million from the issuance of debt and $0.4 million from issuances of our Series E redeemable convertible preferred stock, partially offset by payments of $2.3 million of deferred offering costs related to our IPO that were paid during the period.

During the nine months ended September 30, 2013, net cash provided by financing activities was $5.8 million as a result of net proceeds of $6.0 million from issuances of our Series D redeemable convertible preferred stock, partially offset by payments of $0.2 million of deferred offering costs related to our IPO.

Operating Capital Requirements

Beloranib is still in clinical development and ZGN-839 and our second-generation MetAP2 inhibitors are in pre-clinical development. We expect to continue to incur significant expenses and increasing operating losses for the foreseeable future. We anticipate that our expenses will increase substantially if and as we:

- advance the clinical development of beloranib as a treatment for obesity and hyperphagia in patients with PWS through Phase 3 clinical trials;
- advance the clinical development of beloranib as a treatment for hypothalamic injury-associated obesity through our current Phase 2a clinical trial and if such trial is successful, the initiation of pivotal clinical trials;
- initiate IND-enabling studies and clinical development of ZGN-839 and our second-generation MetAP2 inhibitors;
- initiate a Phase 2b clinical trial of beloranib as a treatment for severe obesity in the general population;
- seek to identify additional indications for beloranib;
- seek to obtain regulatory approvals for our product candidates;
- add operational, financial and management information systems;
- add personnel, including personnel to support our product development and future commercialization; and
- maintain, leverage and expand our intellectual property portfolio.

Based on our current business plan, we believe our current cash and cash equivalents and anticipated cash flow from operations, will be sufficient to meet our anticipated cash requirements over at least the next 12 months. We have based this estimate on assumptions that may prove to be wrong, and we may use our available capital resources sooner than we

currently expect. Because of the numerous risks and uncertainties associated with the development of beloranib, ZGN-839 and our second-generation MetAP2 inhibitors and because the extent to which we may enter into collaborations with third parties for development of these product candidates is unknown, we are unable to estimate the amounts of increased capital outlays and operating expenses associated with completing the research and development of our product candidates. Our future capital requirements for beloranib, ZGN-839 and our second-generation MetAP2 inhibitors will depend on many factors, including:

- the costs, timing and outcome of regulatory review;
- the costs of future research and development activities, including clinical trials;
- the costs of preparing, filing and prosecuting patent applications, maintaining and enforcing our intellectual property rights and defending intellectual property-related claims;
- the extent to which we acquire or in-license other products and technologies; and
- our ability to establish any future collaboration arrangements on favorable terms, if at all.

Until such time, if ever, as we can generate substantial product revenue, we expect to finance our cash needs through a combination of equity offerings, debt financings, collaborations, strategic alliances and licensing arrangements. To the extent that we raise additional capital through the sale of equity or convertible debt securities, the ownership interest of our stockholders will be diluted, and the terms of these securities may include liquidation or other preferences that adversely affect your rights as a common stockholder. Debt financing, if available, may involve agreements that include covenants limiting or restricting our ability to take specific actions, such as incurring additional debt, making capital expenditures or declaring dividends and may require the issuance of warrants, which could potentially dilute your ownership interest. If we raise additional funds through collaborations, strategic alliances or licensing arrangements with third parties, we may have to relinquish valuable rights to our technologies, future revenue streams, research programs of beloranib, ZGN-839 or our second-generation MetAP2 inhibitors or grant licenses on terms that may not be favorable to us. If we are unable to raise additional funds through equity or debt financings when needed, we may be required to delay, limit, reduce or terminate our product development or future commercialization efforts or grant rights to develop and market beloranib, ZGN-839 or our second-generation MetAP2 inhibitors that we would otherwise prefer to develop and market ourselves.

Contractual Obligations and Commitments

The following table summarizes our contractual obligations at September 30, 2014 and the effect such obligations are expected to have on our liquidity and cash flow in future periods:

	Payments Due By Period				
	Total	Less Than 1 Year	1-3 Years	3-5 Years	More Than 5 Years
Operating lease commitments (1)	$ 660	$ 227	$ 433	$ —	$ —
Debt commitments	9,216	1,287	6,648	1,281	—
Licensing milestones (2)	6,700	6,700	—	—	—
Total (2) (3)	$16,576	$ 8,214	$7,081	$1,281	$ —

(1) We entered into an operating lease for new office space in Boston, Massachusetts on May 15, 2014, effective as of July 28, 2014 with a term expiring on July 31, 2017, and an option to extend the lease for three additional years.

(2) We have acquired exclusive rights to develop patented compounds and related know-how under licensing agreements for beloranib with two third parties. The licensing rights obligate us to make payments to the licensors for license fees, milestones, license maintenance fees and royalties. We are also responsible for patent prosecution costs. We are obligated to make future milestone payments under these agreements of up to $19.0 million, including $6.7 million accrued as of September 30, 2014, noted below upon achieving certain pre-commercialization milestones, such as clinical trials and government approvals, and up to $12.5 million upon achieving certain product commercialization milestones. In addition, under one of the license agreements, we are obligated to pay up to $1.3 million with respect to each subsequent licensed product, if any, that is a new chemical entity. As of September 30, 2014, we have a liability of $6.7 million in our consolidated financial statements for milestones achieved under these licensing agreements during September 2014. In addition, we will owe single-digit royalties on sales of commercial products developed using these licensed technologies, if any. We are obligated to pay to the licensors a percentage of fees received if and when we sublicense the technologies. As of September 30, 2014, we have not yet developed a commercial product using the licensed technologies and we have not entered into any sublicense agreements for the technologies.

(3) We enter into contracts in the normal course of business with clinical research organizations for clinical trials, pre-clinical research studies and testing, manufacturing and other services and products for operating purposes. These contracts generally provide for termination upon notice, and therefore we believe that our non-cancelable obligations under these agreements are not material.

Off-Balance Sheet Arrangements

We did not have during the periods presented, and we do not currently have, any off-balance sheet arrangements, as defined under SEC rules, such as relationships with unconsolidated entities or financial partnerships, which are often referred to as structured finance or special purpose entities, established for the purpose of facilitating financing transactions that are not required to be reflected on our balance sheets.

Application of Critical Accounting Policies

We have prepared our consolidated financial statements in accordance with U.S. generally accepted accounting principles. Our preparation of these consolidated financial statements requires us to make estimates, assumptions, and judgments that affect the reported amounts of assets, liabilities, expenses, and related disclosures at the date of the financial statements, as well as revenue and expenses recorded during the reporting periods. We evaluate our estimates and judgments on an ongoing basis. We base our estimates on historical experience and on various other factors that we believe are reasonable under the circumstances, the results of which form the basis for making judgments about the carrying value of assets and liabilities that are not readily apparent from other sources. Actual results could therefore differ materially from these estimates under different assumptions or conditions.

Recently Issued and Adopted Accounting Pronouncements

In June 2014, the FASB issued Accounting Standards Update ("ASU") No. 2014-10, Development Stage Entities (Topic 915): Elimination of Certain Financial Reporting Requirements, Including an Amendment to Variable Interest Entities Guidance in Topic 810, Consolidation. The amendments in this guidance remove all incremental financial reporting requirements for development stage entities. Among other changes, this guidance will no longer require development stage entities to present inception-to-date information about income statement line items, cash flows, and equity transactions. This guidance is effective for public companies in the first annual period beginning after December 15, 2014. Early application is permitted for interim and annual periods for which financial statements have not yet been issued. We elected to apply this disclosure guidance to our consolidated financial statements for the three months ended September 30, 2014 and as a result, we no longer disclose inception-to-date information in our Consolidated Statements of Operations and Comprehensive Loss, Cash Flows and Stockholders' Deficit and the related notes thereto.

In August 2014, the FASB issued ASU No. 2014-15, Presentation of Financial Statements — Going Concern (Subtopic 205-40). The new guidance addresses management's responsibility to evaluate whether there is substantial doubt about an entity's ability to continue as a going concern and to provide related footnote disclosures. 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. The standard will be effective for the first interim period within annual reporting periods beginning after December 15, 2016. Early adoption is permitted. We are evaluating the effect that this guidance will have on our consolidated financial statements.

For a discussion of our critical accounting policies and recent accounting pronouncements see Note 2 in our consolidated financial statements included in our prospectus filed on June 19, 2014, in connection with our IPO.

Item 3. Quantitative and Qualitative Disclosure about Market Risk

Interest Rate Fluctuation Risk

Our cash and cash equivalents as of September 30, 2014 consisted of cash and money market accounts. The primary objectives of our investment activities are to preserve principal, provide liquidity and maximize income without significantly increasing risk. Our primary exposure to market risk is interest income sensitivity, which is affected by changes in the general level of U.S. interest rates. However, because of the short-term nature of the instruments in our portfolio, a sudden change in market interest rates would not be expected to have a material impact on our financial condition and/or results of operation.

Foreign Currency Exchange Risk

Foreign currency transaction exposure results primarily from transactions with our contract research organizations and other providers related to our clinical trials that are denominated in currencies other than the functional currency of the legal entity in which the transaction is recorded by us, primarily the Australian dollar. Any transaction gains or losses resulting from currency fluctuations is recorded on a separate line in our consolidated statements of operations. Net foreign currency transaction losses of less than $0.1 million were recorded for the nine months ended September 30, 2014.

Currently, our largest foreign currency exposures are those with respect to the Australian dollar. Relative to foreign currency exposures existing as of September 30, 2014, a 10% unfavorable movement in foreign currency exchange rates would expose us to losses in earnings. For the nine months ended September 30, 2014, we estimated that a 10% unfavorable movement in foreign currency exchange rates would have increased our net loss by $0.1 million. This amount is based on a sensitivity analysis performed on our financial position as of September 30, 2014. We have experienced and we will continue to experience fluctuations in our net income (loss) as a result of revaluing our assets and liabilities that are not denominated in the functional currency of the entity that recorded the asset or liability. At this time, we do not hedge our foreign currency risk.

Item 4. Management's Evaluation of our Disclosure Controls and Procedures

We maintain disclosure controls and procedures (as defined in Rules 13a-15(e) or 15d-15(e) under the Securities Exchange Act of 1934, as amended, or the Exchange Act) that are designed to ensure that information required to be disclosed in the reports that we file or submit under the Exchange Act is (1) recorded, processed, summarized, and reported within the time periods specified in the SEC's rules and forms and (2) accumulated and communicated to our management, including our principal executive officer and principal financial officer, as appropriate, to allow timely decisions regarding required disclosure.

Based on this evaluation, our management, with the participation of our principal executive officer and principal financial officer, evaluated the effectiveness of our disclosure controls and procedures as of the end of the period covered by this report. In designing and evaluating our disclosure controls and procedures, our management recognizes that any controls and procedures, no matter how well designed and operated, can provide only reasonable assurance of achieving their objectives, and management necessarily applies its judgment in evaluating the cost-benefit relationship of possible controls and procedures. Our principal executive officer and principal financial officer have concluded based upon the evaluation described above that, as of September 30, 2014, our disclosure controls and procedures were effective at the reasonable assurance level.

We continue to review and document our disclosure controls and procedures, including our internal controls and procedures for financial reporting, and may from time to time make changes aimed at enhancing their effectiveness and to ensure that our systems evolve with our business.

Changes in Internal Control Over Financial Reporting

During the three months ended September 30, 2014, there have been no changes in our internal control over financial reporting, as such term is defined in Rules 13a-15(f) and 15(d)-15(f) promulgated under the Exchange Act, that have materially affected, or are reasonably likely to materially affect, our internal control over financial reporting.

PART II — OTHER INFORMATION

Item 1. Legal Proceedings

As of the date of this filing, we were not party to any legal matters or claims. In the future, we may become party to legal matters and claims arising in the ordinary course of business, the resolution of which we do not anticipate would have a material adverse impact on our financial position, results of operations or cash flows.

Item 1A. Risk Factors

Investing in our common stock involves a high degree of risk. You should carefully consider the risks described below, as well as the other information in this Quarterly Report on Form 10-Q and in our other public filings before making an investment decision. Our business, prospects, financial condition, or operating results could be harmed by any of these risks, as well as other risks not currently known to us or that we currently consider immaterial. If any such risks or uncertainties actually occur, our business, financial condition or operating results could differ materially from the plans, projections and

other forward-looking statements included in the section titled "Management's Discussion and Analysis of Financial Condition and Results of Operations" and elsewhere in this report and in our other public filings. The trading price of our common stock could decline due to any of these risks, and as a result, you may lose all or part of your investment.

Risks Related to Product Development, Regulatory Approval and Commercialization

We depend almost entirely on the success of one product candidate, beloranib, which is in Phase 3 clinical development for one indication. We cannot be certain that we will be able to obtain regulatory approval for, or successfully commercialize, beloranib.

We currently have only one product candidate, beloranib, in clinical development, and our business depends almost entirely on its successful clinical development, regulatory approval and commercialization. We currently have no drug products for sale and may never be able to develop marketable drug products. Beloranib, which is currently in clinical development as a treatment for obesity and hyperphagia (insatiable life-threatening hunger and hunger-related behaviors) in Prader-Willi syndrome, or PWS (recently initiated the first of two Phase 3 clinical trials, in the United States), hypothalamic injury-associated obesity (in Phase 2), and severe obesity in the general population (in Phase 2), will require substantial additional clinical development, testing and regulatory approval before we are permitted to commence its commercialization. Our other product candidates, including ZGN-839, are still in pre-clinical development stages. The clinical trials of our product candidates are, and the manufacturing and marketing of our product candidates will be, subject to extensive and rigorous review and regulation by numerous government authorities in the United States and in other countries where we intend to test and, if approved, market any product candidate. Before obtaining regulatory approvals for the commercial sale of any product candidate, we must demonstrate through pre-clinical testing and clinical trials that the product candidate is safe and effective for use in each target indication. This process can take many years and may include post-marketing studies and surveillance, which will require the expenditure of substantial resources beyond the proceeds we currently have on hand. Of the large number of drugs in development in the United States, only a small percentage will successfully complete the U.S. Food and Drug Administration, or FDA, regulatory approval process and will be commercialized. Accordingly, even if we are able to obtain the requisite financing to continue to fund our development and clinical trials, we cannot assure you that beloranib or any other of our product candidates will be successfully developed or commercialized.

We are not permitted to market beloranib in the United States until we receive approval of a New Drug Application, or an NDA, from the FDA, or in any foreign countries until we receive the requisite approval from such countries. We recently completed two Phase 2a clinical trials evaluating beloranib's ability to reduce body weight and to improve hyperphagia, one in patients with PWS and one in severely obese patients. We expect that the FDA will require us to conduct at least one pivotal trial to demonstrate safety and efficacy for beloranib as a treatment for patients with PWS. However, meeting requirements of the FDA or certain European regulatory authorities may require that we conduct additional pivotal trials. We expect that the FDA will also require us to complete Phase 2 assessments and at least two Phase 3 clinical trials to demonstrate safety and efficacy for beloranib as a treatment for severe obesity in the general population, and may require that we conduct a cardiovascular outcomes trial. Pursuant to the FDA's February 2007 draft guidance to industry on the development of weight management drugs, in order to reasonably estimate the safety of a weight-management drug, Phase 3 clinical trials must randomize approximately 3,000 subjects to active doses of the product and 1,500 subjects to placebo in clinical trials of one year duration. We have not yet commenced any of these clinical trials. We initiated our Phase 3 clinical program assessing beloranib in patients with PWS, with the first Phase 3 trial in the United States having started in September 2014. We are planning to initiate our second Phase 3 trial in Europe in patients with PWS in the middle of 2015. We intend to engage in discussions with the appropriate regulatory agencies to determine if our proposed Phase 3 clinical trial protocols would be sufficient to support an NDA submission to the FDA and a marketing authorization application, or MAA, to the European Medicines Agency, or EMA, seeking approval of beloranib for the treatment of PWS. To initiate our Phase 3 clinical program for beloranib in patients with PWS, we submitted pre-clinical animal studies and clinical studies for the FDA's review, and the FDA may require additional studies. We initiated a Phase 2a clinical trial of beloranib as a treatment for hypothalamic injury-associated obesity in June 2014. We intend to initiate a Phase 2b clinical trial of beloranib as a treatment for severe obesity in the general population in the fourth quarter of 2014. We are also evaluating additional proprietary methionine aminopeptidase 2, or MetAP2, inhibitors beyond beloranib as potential development candidates that would provide increased patient convenience in the form of oral dosing, or an otherwise improved clinical profile. A decision on whether to subsequently advance beloranib into pivotal trials for severe obesity or to leverage the opportunity to advance another MetAP2 inhibitor into early development for severe obesity is anticipated to be made on the basis of results obtained from our Phase 3 clinical program of beloranib in patients with PWS and discussions with regulatory authorities. Accordingly, obtaining approval of an NDA is a complex, lengthy, expensive and uncertain process, and the FDA and certain European regulatory authorities may delay, limit or deny approval of beloranib for many reasons, including, among others:

- we may not be able to demonstrate that beloranib is safe and effective in treating obesity and hyperphagia in patients with PWS, hypothalamic injury-associated obesity or severe obesity in the general population, to the satisfaction of the FDA and certain European regulatory authorities;

- the results of our clinical trials may not meet the level of statistical or clinical significance required by the FDA and certain European regulatory authorities for marketing approval;
- the FDA and certain European regulatory authorities may disagree with the number, design, size, duration, conduct or implementation of our clinical trials;
- the FDA and certain European regulatory authorities may require that we conduct additional clinical trials;
- the FDA and certain European regulatory authorities may not approve the formulation, labeling or specifications of beloranib;
- the contract research organizations, or CROs, that we retain to conduct our clinical trials may take actions outside of our control that materially adversely impact our clinical trials;
- the FDA and certain European regulatory authorities may find the data from pre-clinical studies and clinical trials insufficient to demonstrate that beloranib's clinical and other benefits outweigh its safety risks;
- the FDA and certain European regulatory authorities may disagree with our interpretation of data from our pre-clinical studies and clinical trials;
- the FDA and certain European regulatory authorities may not accept data generated at our clinical trial sites;
- if our NDA, if and when submitted, is reviewed by an advisory committee, the FDA may have difficulties scheduling an advisory committee meeting in a timely manner or the advisory committee may recommend against approval of our application or may recommend that the FDA require, as a condition of approval, additional pre-clinical studies or clinical trials, limitations on approved labeling or distribution and use restrictions;
- the FDA may require development of a Risk Evaluation and Mitigation Strategy, or REMS, as a condition of approval or post-approval;
- the FDA and certain European regulatory authorities may not approve the manufacturing processes or facilities of third-party manufacturers with which we contract; or
- the FDA and certain European regulatory authorities may change their approval policies or adopt new regulations.

Any of these factors, many of which are beyond our control, could jeopardize our ability to obtain regulatory approval for and successfully market beloranib. Moreover, because our business is almost entirely dependent upon this one product candidate, any such setback in our pursuit of regulatory approval would have a material adverse effect on our business and prospects.

Positive results from early clinical trials of beloranib are not necessarily predictive of the results of later clinical trials of beloranib. If we cannot replicate the positive results from our earlier clinical trials of beloranib in our later clinical trials, we may be unable to successfully develop, obtain regulatory approval for and commercialize beloranib. It may further be necessary to validate different or additional instruments for measuring subjective endpoints, and to show that beloranib has clinically meaningful impact on those endpoints in order to obtain regulatory approval.

Positive results from our Phase 1 and Phase 2a clinical trials of beloranib may not necessarily be predictive of the results from required later clinical trials. Similarly, even if we are able to complete our ongoing and planned Phase 2b or Phase 3 clinical trials of beloranib according to our current development timeline, the positive results from our Phase 1 and Phase 2a clinical trials of beloranib may not be replicated in our Phase 2b or Phase 3 clinical trial results. The design of our Phase 3 clinical program of beloranib as a treatment for obesity and hyperphagia in patients with PWS differs in several aspects from our recently completed Phase 2a clinical trial for PWS. For example, patients with PWS will not be limited to living in closely-controlled PWS-specific group homes like they were in Phase 2, but will be predominantly living in family homes. In addition, patients with PWS will be treated with beloranib for substantially longer than four weeks. One of the Phase 3 clinical trials will be conducted in Europe, where we have not previously conducted clinical trials. In later-stage clinical trials, we will utilize highly rigorous statistical analyses. For example, the results of our Phase 2a clinical trial for severe obesity were based on a per protocol analysis of patients who completed the 12-week dosing in the clinical trial. Later-stage clinical trials will be evaluated based on an intent-to-treat analysis that includes all patients randomized in the clinical trial, regardless of whether they complete the treatment, which may lead to different results. In addition, if we fail to appropriately

validate a caregiver-administered PWS-specific hyperphagia-related behaviors questionnaire, or PWS-HQ, in time for it to be an effective tool to support the data from our Phase 3 clinical trials, our Phase 3 clinical program and, in turn, our regulatory filing may be delayed until we validate the tool or develop and test a new one. This can be a lengthy process. Many companies in the pharmaceutical and biotechnology industries have suffered significant setbacks in late-stage clinical trials after achieving positive results in early-stage development, and we cannot be certain that we will not face similar setbacks. These setbacks have been caused by, among other things, pre-clinical findings made while clinical trials were underway or safety or efficacy observations made in clinical trials, including previously unreported adverse events. Moreover, pre-clinical and clinical data are often susceptible to varying interpretations and analyses, and many companies that believed their product candidates performed satisfactorily in pre-clinical studies and clinical trials nonetheless failed to obtain FDA approval. If we fail to produce positive results in our ongoing and planned Phase 2b or Phase 3 clinical trials of beloranib, the development timeline and regulatory approval and commercialization prospects for our leading product candidate, and, correspondingly, our business and financial prospects, would be materially adversely affected.

Failures or delays in the commencement or completion of our planned clinical trials of beloranib could result in increased costs to us and could delay, prevent or limit our ability to generate revenue and continue our business.

We initiated a Phase 2a clinical trial of beloranib as a treatment for hypothalamic injury-associated obesity in June 2014. We initiated our Phase 3 clinical program of beloranib as a treatment for obesity and hyperphagia in patients with PWS in the United States in September 2014, as the first of two planned Phase 3 clinical trials. We are planning to initiate our second Phase 3 trial in Europe in patients with PWS in the middle of 2015. We also plan to commence a Phase 2b clinical trial of beloranib as a treatment for severe obesity in the general population in the fourth quarter of 2014. Despite the guidance received from, and to be received from, these regulatory authorities, both the FDA and the European regulatory authorities can change their positions on the acceptability of our trial designs or the clinical endpoints selected, which may require us to complete additional clinical trials or impose stricter approval conditions than we currently expect. Successful completion of such clinical trials is a prerequisite to submitting an NDA to the FDA and, consequently, the ultimate approval and commercial marketing of beloranib. We do not know whether any of these Phase 2a, Phase 2b or Phase 3 clinical trials will begin or be completed on schedule, if at all, as the commencement and completion of clinical trials can be delayed or prevented for a number of reasons, including, among others:

- the FDA may place a clinical trial on hold on our Phase 2a or Phase 3 clinical trials or may deny permission to proceed with any other clinical trials we may initiate, ;
- delays in regulatory filings or receiving regulatory approvals or additional INDs that may be required;
- negative results from our ongoing pre-clinical studies or the FDA or European regulatory authorities may require additional pre-clinical studies;
- delays in reaching or failing to reach agreement on acceptable terms with prospective CROs and clinical trial sites, the terms of which can be subject to extensive negotiation and may vary significantly among different CROs and trial sites;
- inadequate quantity or quality of a product candidate or other materials necessary to conduct clinical trials, for example delays in the manufacturing of sufficient supply of finished drug product;
- difficulties obtaining Institutional Review Board, or IRB, approval to conduct a clinical trial at a prospective site or sites;
- challenges in recruiting and enrolling patients to participate in clinical trials, including the size and nature of the patient population, the proximity of patients to trial sites, eligibility criteria for the clinical trial, the nature of the clinical trial protocol, the availability of approved effective treatments for the relevant disease and competition from other clinical trial programs for similar indications;
- severe or unexpected drug-related side effects experienced by patients in a clinical trial, including side effects previously identified in our completed clinical trials;
- delays in validating the PWS-HQ or any other self-reported measures of hunger and related endpoints utilized in a clinical trial;
- the FDA may disagree with our clinical trial design and our interpretation of data from clinical trials, or may change the requirements for approval even after it has reviewed and commented on the design for our clinical trials;
- reports from pre-clinical or clinical testing of other weight loss therapies that raise safety or efficacy concerns; and
- difficulties retaining patients who have enrolled in a clinical trial but may be prone to withdraw due to rigors of the clinical trials, lack of efficacy, side effects, personal issues or loss of interest.

Clinical trials may also be delayed or terminated as a result of ambiguous or negative interim results. In addition, a clinical trial may be suspended or terminated by us, the FDA, other regulatory authorities, the IRBs, at the sites where the IRBs are overseeing a clinical trial, a data and safety monitoring board, or DSMB, overseeing the clinical trial at issue or other regulatory authorities due to a number of factors, including, among others:

- failure to conduct the clinical trial in accordance with regulatory requirements or our clinical protocols;
- inspection of the clinical trial operations or trial sites by the FDA or other regulatory authorities that reveals deficiencies or violations that require us to undertake corrective action, including the imposition of a clinical hold;
- unforeseen safety issues, including any that could be identified in our pre-clinical studies or ongoing pre-clinical carcinogenicity studies, adverse side effects or lack of effectiveness;
- changes in government regulations or administrative actions;
- problems with clinical supply materials; and
- lack of adequate funding to continue the clinical trial.

Changes in regulatory requirements, FDA guidance or guidance from certain European regulatory authorities or unanticipated events during our clinical trials of beloranib may occur, which may result in changes to clinical trial protocols or additional clinical trial requirements, which could result in increased costs to us and could delay our development timeline.

Changes in regulatory requirements, FDA guidance or guidance from certain European regulatory authorities or unanticipated events during our clinical trials may force us to adjust our clinical program or the FDA or certain European regulatory authorities may impose additional clinical trial requirements. For instance, the FDA issued draft guidance on developing products for weight management in February 2007, but this guidance may be revised in the near future. In March 2012, the FDA's Endrocrinologic and Metabolic Drugs Advisory Committee met to discuss possible changes to how the FDA evaluates the cardiovascular safety of weight-management drugs. Amendments to our clinical trial protocols would require resubmission to the FDA or certain European regulatory authorities and IRBs for review and approval, which may adversely impact the cost, timing or successful completion of a clinical trial. If we experience delays completing, or if we terminate, any of our clinical trials, or if we are required to conduct additional clinical trials, the commercial prospects for beloranib may be harmed and our ability to generate product revenue will be delayed.

We rely, and expect that we will continue to rely, on third parties to conduct any future clinical trials for beloranib. If these third parties do not successfully carry out their contractual duties or meet expected deadlines, we may not be able to obtain regulatory approval for or commercialize beloranib and our business could be substantially harmed.

We enter into agreements with third-party CROs to provide monitors for and to manage data for our ongoing clinical trials. We rely heavily on these parties for execution of clinical trials for beloranib and control only certain aspects of their activities. As a result, we have less direct control over the conduct, timing and completion of these clinical trials and the management of data developed through the clinical trials than would be the case if we were relying entirely upon our own staff. Communicating with outside parties can also be challenging, potentially leading to mistakes as well as difficulties in coordinating activities. Outside parties may:

- have staffing difficulties;
- fail to comply with contractual obligations;
- experience regulatory compliance issues;
- undergo changes in priorities or become financially distressed; or
- form relationships with other entities, some of which may be our competitors.

These factors may materially adversely affect the willingness or ability of third parties to conduct our clinical trials and may subject us to unexpected cost increases that are beyond our control. Nevertheless, we are responsible for ensuring that each of our studies is conducted in accordance with the applicable protocol, legal, regulatory and scientific standards, and our reliance on CROs does not relieve us of our regulatory responsibilities. We and our CROs are required to comply with

current Good Clinical Practices, or cGCPs, which are regulations and guidelines enforced by the FDA, the Competent Authorities of the Member States of the European Economic Area and comparable foreign regulatory authorities for any products in clinical development. The FDA enforces these cGCP regulations through periodic inspections of clinical trial sponsors, principal investigators and trial sites. If we or our CROs fail to comply with applicable cGCPs, the clinical data generated in our clinical trials may be deemed unreliable and the FDA or comparable foreign regulatory authorities may require us to perform additional clinical trials before approving our marketing applications. We cannot assure you that, upon inspection, the FDA will determine that any of our clinical trials comply with cGCPs. In addition, our clinical trials must be conducted with product produced under current Good Manufacturing Practices, or cGMPs, regulations and will require a large number of test subjects. Our failure or the failure of our CROs to comply with these regulations may require us to repeat clinical trials, which would delay the regulatory approval process and could also subject us to enforcement action up to and including civil and criminal penalties.

Although we do design our clinical trials for beloranib, CROs conduct all of the clinical trials. As a result, many important aspects of our drug development programs are outside of our direct control. In addition, the CROs may not perform all of their obligations under arrangements with us or in compliance with regulatory requirements, but we remain responsible and are subject to enforcement action that may include civil penalties up to and including criminal prosecution for any violations of FDA laws and regulations during the conduct of our clinical trials. If the CROs do not perform clinical trials in a satisfactory manner, breach their obligations to us or fail to comply with regulatory requirements, the development and commercialization of beloranib may be delayed or our development program materially and irreversibly harmed. We cannot control the amount and timing of resources these CROs devote to our program or beloranib. If we are unable to rely on clinical data collected by our CROs, we could be required to repeat, extend the duration of, or increase the size of our clinical trials and this could significantly delay commercialization and require significantly greater expenditures.

If any of our relationships with these third-party CROs terminate, we may not be able to enter into arrangements with alternative CROs. If CROs do not successfully carry out their contractual duties or obligations or meet expected deadlines, if they need to be replaced or if the quality or accuracy of the clinical data they obtain is compromised due to the failure to adhere to our clinical protocols, regulatory requirements or for other reasons, any such clinical trials may be extended, delayed or terminated, and we may not be able to obtain regulatory approval for or successfully commercialize beloranib. As a result, our financial results and the commercial prospects for beloranib in the subject indication would be harmed, our costs could increase and our ability to generate revenue could be delayed.

The number of patients suffering from PWS and hypothalamic injury-associated obesity is small and has not been established with precision. If the actual number of patients with either of these conditions is smaller than we estimate or if any approval that we obtain is based on a narrower definition of these patient populations, our revenue and ability to achieve profitability will be adversely affected, possibly materially.

There is no current comprehensive patient registry or other method of establishing with precision the actual number of patients with PWS or hypothalamic injury-associated obesity in any geography. Published population studies estimate that the prevalence of PWS in the United States and in the European Union ranges from 1 in 8,000 to 1 in 50,000. Published population studies estimate that the incidence of craniopharyngioma, the leading cause of obesity related to injury to the hypothalamus, is 0.13 to 0.17 per 100,000 per year, or approximately 400 to 500 cases per year in the United States and 650 to 850 cases per year in the European Union. The total addressable market opportunity for beloranib for the treatment of patients with PWS or hypothalamic injury-associated obesity will ultimately depend upon, among other things, the final label for beloranib if approved for sale for these indications, acceptance by the medical community and patient access, product pricing and reimbursement. If the actual number of patients with PWS or hypothalamic injury-associated obesity is lower than we believe or if any approval that we obtain is based on a narrower definition of these patient populations, then the potential markets for beloranib for these indications will be smaller than we anticipate.

In addition, we plan to seek approval of beloranib initially for the treatment of adolescent (12 years of age and older) and adult patients with PWS and in adult patients with hypothalamic injury-associated obesity. We believe that approximately 40-50% of patients with PWS are >12 years of age or older. To support approval for pediatric patients (younger than 12 years of age), we will need to conduct pediatric clinical trials of beloranib for the treatment of these patients with PWS, but we do not yet have plans regarding when these trials will commence. As a result, any FDA approval would likely, at least initially, be limited to use for treating adolescent and adult patients with PWS. This would limit our initial product revenue and may make it more difficult for us to achieve or maintain profitability.

We rely completely on third-party suppliers to manufacture our clinical drug supplies for beloranib, and we intend to rely on third parties to produce commercial supplies of beloranib and pre-clinical, clinical and commercial supplies of any future product candidate.

We do not currently have, nor do we currently plan to acquire, the infrastructure or capability to internally manufacture our clinical drug supply of beloranib, or any future product candidates, for use in the conduct of our pre-clinical studies and clinical trials, and we lack the internal resources and the capability to manufacture any product candidates on a clinical or commercial scale. The facilities used by our contract manufacturers, or CMOs to manufacture the active drug substance, sterile drug substance and final drug product must be approved by the FDA and other comparable foreign regulatory agencies pursuant to inspections that would be conducted after we submit our NDA or relevant foreign regulatory submission to the applicable regulatory agency.

We do not control the manufacturing process of, and are completely dependent on, our CMOs to comply with cGMPs for manufacture of drug substance, sterile drug substance and finished drug products. If our CMOs cannot successfully manufacture material that conforms to our specifications and the regulatory requirements of the FDA or applicable foreign regulatory agencies, the CMOs will not be able to secure and/or maintain regulatory approval for their manufacturing facilities. While we manage our quality expectations through a regular audit program for our vendors and suppliers, we have no direct control over our CMOs' ability to maintain adequate quality control, quality assurance and qualified personnel. Furthermore, our CMOs are engaged with third party vendors to supply and/or manufacture starting materials or components for such CMOs, which exposes our manufacturers to regulatory risks for the production of such materials and components. As a result, failure to satisfy global regulatory requirements for the production of those materials and products may affect the regulatory clearance of our CMOs' facilities generally. If the FDA or an applicable foreign regulatory agency does not approve these facilities for the manufacture of our product candidates or if it withdraws its approval in the future, we may need to find alternative manufacturing facilities, which would adversely impact our ability to develop, obtain regulatory approval for or market our product candidates.

We are currently in the process of manufacturing finished drug product for resupply of clinical trial materials. Along with drug product, we are directly sourcing two additional components for use in our clinical trial kits. If the manufacturer of either of these components fails to comply with global regulatory requirements, alternate vendors would need to be identified. The manufacturing process is under active development and could change based on delays encountered with manufacturing activities, equipment scheduling and material lead times. Any such delays in the manufacturing of finished drug product could delay our planned clinical trials of beloranib, which could delay, prevent or limit our ability to generate revenue and continue our business.

We do not have long-term supply agreements in place with our contractors, and each batch of beloranib is individually contracted under a quality and supply agreement. CMOs are currently supporting clinical activities, however CMOs that will manufacture commercial GMP batches for beloranib will need to be approved by the FDA and other applicable foreign regulatory agencies, prior to commercialization. We plan to continue to rely upon CMOs to manufacture commercial quantities of beloranib, if approved. Our current scale of manufacturing is adequate to support all of our needs for clinical trial supplies and launch for orphan markets. For peak usage in orphan markets and for indications with larger populations of affected patients, we will need to identify CMOs or partners to produce beloranib on a larger scale.

If we are unable to establish sales and marketing capabilities or enter into agreements with third parties to market and sell beloranib, we may not be able to generate any revenue.

We do not currently have an infrastructure for the sales, marketing and distribution of pharmaceutical products. In order to market beloranib, if approved by the FDA or any other regulatory body, we must build our sales, marketing, managerial and other non-technical capabilities or make arrangements with third parties to perform these services. If we are unable to establish adequate sales, marketing and distribution capabilities, whether independently or with third parties, or if we are unable to do so on commercially reasonable terms, our business, results of operations, financial condition and prospects will be materially adversely affected.

Even if we receive marketing approval for beloranib in the United States, we may never receive regulatory approval to market beloranib outside of the United States.

We intend to pursue marketing approval of beloranib in the European Union and in other countries worldwide. In order to market any product outside of the United States, we must establish and comply with the numerous and varying safety, efficacy and other regulatory requirements of other countries including potential additional clinical trials or non-clinical studies. Approval procedures vary among countries and can involve additional product candidate testing and additional

administrative review periods. The time required to obtain approvals in other countries might differ from that required to obtain FDA approval. The marketing approval processes in other countries may implicate all of the risks detailed above regarding FDA approval in the United States as well as other risks. In particular, in many countries outside of the United States, products must receive pricing and reimbursement approval before the product can be commercialized. Obtaining this approval can result in substantial delays in bringing products to market in such countries. Marketing approval in one country does not ensure marketing approval in another, but a failure or delay in obtaining marketing approval in one country may have a negative effect on the regulatory process in others. Failure to obtain marketing approval in other countries or any delay or other setback in obtaining such approval would impair our ability to market beloranib in such foreign markets. Any such impairment would reduce the size of our potential market, which could have a material adverse impact on our business, results of operations and prospects.

Even if we receive marketing approval for beloranib, it may not achieve broad market acceptance, which would limit the revenue that we generate from its sales.

The commercial success of beloranib, if approved by the FDA or EMA or other applicable regulatory authorities, will depend upon the awareness and acceptance of beloranib among the medical community, including physicians, patients and healthcare payors. Market acceptance of beloranib, if approved, will depend on a number of factors, including, among others:

- beloranib's demonstrated ability to treat obesity and hyperphagia in patients with PWS, hypothalamic injury-associated obesity, or severe obesity in the general population and, if required by any applicable regulatory authority in connection with the approval for these indications, to provide patients with incremental health benefits, as compared with other available weight loss therapies, devices or surgeries;
- the relative convenience and ease of subcutaneous injections as the necessary method of administration of beloranib, including as compared with other treatments for severely obese patients;
- the prevalence and severity of any adverse side effects associated with beloranib, such as nausea, vomiting, headaches and difficulty sleeping or falling asleep;
- limitations or warnings contained in the labeling approved for beloranib by the FDA or EMA;
- availability of alternative treatments, including a number of competitive obesity therapies already approved or expected to be commercially launched in the near future;
- the willingness of the target patient population to try new therapies and of physicians to prescribe these therapies;
- the strength of marketing and distribution support and timing of market introduction of competitive products;
- publicity concerning our products or competing products and treatments;
- pricing and cost effectiveness;
- the effectiveness of our sales and marketing strategies;
- our ability to increase awareness of beloranib through marketing efforts;
- our ability to obtain sufficient third-party coverage or reimbursement; and
- the willingness of patients to pay out-of-pocket in the absence of third-party coverage.

If beloranib is approved but does not achieve an adequate level of acceptance by patients, physicians and payors, we may not generate sufficient revenue from beloranib to become or remain profitable. Before granting reimbursement approval, healthcare payors may require us to demonstrate that, in addition to treating obesity in patients, beloranib also provides incremental health benefits to patients. Our efforts to educate the medical community and third-party payors about the benefits of beloranib may require significant resources and may never be successful.

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

Undesirable side effects caused by our product candidates could cause us or regulatory authorities to interrupt, delay or halt clinical trials and could result in a more restrictive label or the delay or denial of regulatory approval by the FDA or other regulatory authorities. In our recently completed Phase 2a clinical trials, the main adverse events, or AEs, including those leading to premature treatment discontinuation, in patients dosed with beloranib, have been sleep disturbances, principally manifested as delayed onset of sleep, as well as nausea and vomiting.

The safety data we have disclosed to date represents our interpretation of the data at the time of disclosure and it is subject to our further review and analysis. There have been no serious adverse events, or SAEs, attributed to beloranib in our clinical trials. However, SAEs that are not characterized by clinical investigators as possibly related to beloranib or SAEs that occur in small numbers may not be disclosed to the public until such time the various documents submitted to the FDA as part of the approval process are made public. We are unable to determine if the subsequent disclosure of SAEs will have an adverse effect on our stock price. In addition, our interpretation of the safety data from our clinical trials is contingent upon the review and ultimate approval of the FDA or other regulatory authorities. The FDA or other regulatory authorities may not agree with our methods of analysis or our interpretation of the results.

Further, if beloranib receives marketing approval and we or others identify undesirable side effects caused by the product (or any other similar product) after the approval, a number of potentially significant negative consequences could result, including:

- regulatory authorities may withdraw or limit their approval of the product;
- regulatory authorities may require the addition of labeling statements, such as a "boxed" warning or a contraindication;
- we may be required to change the way the product is distributed or administered, conduct additional clinical trials or change the labeling of the product;
- we may decide to remove the products from the marketplace;
- we could be sued and held liable for injury caused to individuals exposed to or taking our product candidates; and
- our reputation may suffer.

Any of these events could prevent us from achieving or maintaining market acceptance of the affected product candidate and could substantially increase the costs of commercializing our product candidates and significantly impact our ability to successfully commercialize our product candidates and generate revenues.

Even if we receive marketing approval for beloranib, we may still face future development and regulatory difficulties.

Even if we receive marketing approval for beloranib, regulatory authorities may still impose significant restrictions on beloranib's indicated uses or marketing or impose ongoing requirements for potentially costly post-approval studies. Beloranib will also be subject to ongoing FDA and EMA requirements governing the labeling, packaging, storage and promotion of the product and recordkeeping and submission of safety and other post-market information. The FDA has significant post-market authority, including, for example, the authority to require labeling changes based on new safety information and to require post-market studies or clinical trials to evaluate serious safety risks related to the use of a drug. The FDA also has the authority to require, as part of an NDA or post-approval, the submission of a REMS. Any REMS required by the FDA may lead to increased costs to assure compliance with new post-approval regulatory requirements and potential requirements or restrictions on the sale of approved products, all of which could lead to lower sales volume and revenue.

Manufacturers of drug products and their facilities are subject to continual review and periodic inspections by the FDA and other regulatory authorities for compliance with cGMPs and other regulations. If we or a regulatory agency discover problems with beloranib, such as adverse events of unanticipated severity or frequency, or problems with the facility where beloranib is manufactured, a regulatory agency may impose restrictions on beloranib, the manufacturer or us, including requiring withdrawal of beloranib from the market or suspension of manufacturing. If we, or the manufacturing facilities for beloranib fail to comply with applicable regulatory requirements, a regulatory agency may, among other things:

- issue warning letters or untitled letters;
- seek an injunction or impose civil or criminal penalties or monetary fines;
- suspend or withdraw marketing approval;
- suspend any ongoing clinical trials;
- refuse to approve pending applications or supplements to applications submitted by us;

- suspend or impose restrictions on operations, including costly new manufacturing requirements; or
- seize or detain products, refuse to permit the import or export of products, or request that we initiate a product recall.

Competing technologies could emerge, including devices and surgical procedures, adversely affecting our opportunity to generate revenue from the sale of beloranib.

The biotechnology and pharmaceutical industries are intensely competitive and subject to rapid and significant technological change. We have competitors in a number of jurisdictions, many of which have substantially greater name recognition, commercial infrastructures and financial, technical and personnel resources than we have. Established competitors may invest heavily to quickly discover and develop novel compounds that could make beloranib obsolete or uneconomical. Any new product that competes with an approved product may need to demonstrate compelling advantages in efficacy, convenience, tolerability and safety to be commercially successful. Other competitive factors, including generic competition, could force us to lower prices or could result in reduced sales. In addition, new products developed by others could emerge as competitors to beloranib. If we are not able to compete effectively against our current and future competitors, our business will not grow and our financial condition and operations will suffer.

There are no current pharmacological treatments for regulating hunger and hyperphagia-related behaviors of patients with PWS or patients with hypothalamic injury-associated obesity, and bariatric surgery is contraindicated in patients with PWS and is not frequently employed in patients with hypothalamic injury-associated obesity. We are aware of a clinical trial currently recruiting patients by Ferring Pharmaceuticals, Inc. to evaluate the use of carbetocin, an analogue of a brain peptide hormone oxytocin hypothesized to increase trust, reduce anxiety and improve behavior in patients with PWS. We also are aware of a clinical trial being conducted by Essentialis, Inc. to evaluate the safety and tolerability of controlled-release diazoxide in patients with PWS and to explore the effects of diazoxide on hyperphagia-related behaviors and energy expenditure. We are aware of an exploratory trial being planned, or in its early phase of execution, by Rhythm Pharmaceuticals to evaluate the effect of treatment with RM-493, a melanocortin receptor agonist, in patients with PWS. In addition, any of our competitors may develop a drug to treat patients with PWS at any time. We are not aware of any clinical trials of drugs specifically targeting patients with hypothalamic injury-associated obesity.

Our potential competitors in the severe obesity market include bariatric surgery providers, and, in addition, other potential approaches which utilize various implantable devices or surgical tools are in development, by companies such as Allergan, Inc., Boston Scientific Corporation, Covidien Ltd., EnteroMedics, Inc., GI Dynamics, Inc., Johnson & Johnson and Medtronic, Inc. In addition, beloranib will compete with orlistat, phentermine/topiramate and lorcaserin, three recently approved and currently marketed pharmaceutical products in the United States for the treatment of obesity, and several older agents, indicated for short-term administration, including phentermine, phendimetrazine, benzphetamine and diethylpropion. Orlistat is marketed in the United States by the Roche Group under the brand name Xenical and over-the-counter in the United States at half the prescribed dose by GlaxoSmithKline under the brand name alli. In June 2013, Arena Pharmaceuticals, Inc. launched its lorcaserin product, which is marketed in the United States under the name Belviq and in September 2012, Vivus, Inc. commercially launched its combination product, phentermine/topiramate, under the trade name Qsymia. In October 2014 Takeda Pharmaceuticals U.S.A., Inc. and Orexigen® Therapeutics, Inc. launched Contrave® (naltrexone HCI and bupropion HCI) extended-release tablets for chronic weight management in obese adults. Despite the large market opportunity for anti-obesity agents, there are relatively few competitive products in late-stage clinical development. Other companies pursuing pharmaceutical treatments for obesity include Neurosearch A/S, Novo Nordisk A/S and Takeda Pharmaceutical Company Limited.

Our future growth depends, in part, on our ability to penetrate foreign markets, where we would be subject to additional regulatory burdens and other risks and uncertainties.

Our future profitability will depend, in part, on our ability to commercialize beloranib in foreign markets for which we may rely on collaborations with third parties. If we commercialize beloranib in foreign markets, we would be subject to additional risks and uncertainties, including:

- our customers' ability to obtain reimbursement for beloranib in foreign markets;
- our inability to directly control commercial activities because we are relying on third parties;
- the burden of complying with complex and changing foreign regulatory, tax, accounting and legal requirements;
- different medical practices and customs in foreign countries affecting acceptance in the marketplace;
- import or export licensing requirements;
- longer accounts receivable collection times;

- longer lead times for shipping;
- language barriers for technical training;
- reduced protection of intellectual property rights in some foreign countries;
- foreign currency exchange rate fluctuations; and
- the interpretation of contractual provisions governed by foreign laws in the event of a contract dispute.

Foreign sales of beloranib could also be adversely affected by the imposition of governmental controls, political and economic instability, trade restrictions and changes in tariffs.

We are subject to healthcare laws and regulations, which could expose us to criminal sanctions, civil penalties, contractual damages, reputational harm and diminished profits and future earnings.

Healthcare providers, physicians and others will play a primary role in the recommendation and prescription of beloranib, if approved. Our future arrangements with third-party payors will expose us to broadly applicable fraud and abuse and other healthcare laws and regulations that may constrain the business or financial arrangements and relationships through which we market, sell and distribute beloranib, if we obtain marketing approval. Restrictions under applicable federal and state healthcare laws and regulations include the following:

- The federal healthcare anti-kickback statute prohibits, among other things, persons from knowingly and willfully soliciting, offering, receiving or providing remuneration, directly or indirectly, in cash or in kind, to induce or reward either the referral of an individual for, or the purchase, order or recommendation of, any good or service, for which payment may be made under federal healthcare programs such as Medicare and Medicaid.
- The federal False Claims Act imposes criminal and civil penalties, including those from civil whistleblower or qui tam actions, against individuals or entities for knowingly presenting, or causing to be presented, to the federal government, claims for payment that are false or fraudulent or making a false statement to avoid, decrease, or conceal an obligation to pay money to the federal government.
- The federal Health Insurance Portability and Accountability Act of 1996, as amended by the Health Information Technology for Economic and Clinical Health Act, imposes criminal and civil liability for executing a scheme to defraud any healthcare benefit program and also imposes obligations, including mandatory contractual terms, with respect to safeguarding the privacy, security and transmission of individually identifiable health information.
- The federal false statements statute prohibits knowingly and willfully falsifying, concealing or covering up a material fact or making any materially false statement in connection with the delivery of or payment for healthcare benefits, items or services.
- The federal transparency requirements, sometimes referred to as the “Sunshine Act,” under the Patient Protection and Affordable Care Act, require manufacturers of drugs, devices, biologics and medical supplies to report to the Department of Health and Human Services information related to physician payments and other transfers of value and physician ownership and investment interests.
- Analogous state laws and regulations, such as state anti-kickback and false claims laws and transparency laws, may apply to sales or marketing arrangements and claims involving healthcare items or services reimbursed by non-governmental third-party payors, including private insurers, and some state laws require pharmaceutical companies to comply with the pharmaceutical industry’s voluntary compliance guidelines and the relevant compliance guidance promulgated by the federal government in addition to requiring drug manufacturers to report information related to payments to physicians and other healthcare providers or marketing expenditures and drug pricing.

Ensuring that our future business arrangements with third parties comply with applicable healthcare laws and regulations could be costly. It is possible that governmental authorities will conclude that our business practices do not comply with current or future statutes, regulations or case law involving applicable fraud and abuse or other healthcare laws and regulations. If our operations, including anticipated activities to be conducted by our sales team, were found to be in violation of any of these laws or any other governmental regulations that may apply to us, we may be subject to significant civil, criminal and administrative penalties, damages, fines and exclusion from government funded healthcare programs, such as Medicare and Medicaid, any of which could substantially disrupt our operations. If any of the physicians or other providers or entities with whom we expect to do business is found not to be in compliance with applicable laws, they may be subject to criminal, civil or administrative sanctions, including exclusions from government funded healthcare programs.

The FDA and other regulatory agencies actively enforce the laws and regulations prohibiting the promotion of off-label uses. If we are found to have improperly promoted off-label uses, we may become subject to significant liability.

The FDA and other regulatory agencies strictly regulate the promotional claims that may be made about prescription products, such as beloranib, if approved. In particular, a product may not be promoted for uses that are not approved by the FDA or such other regulatory agencies as reflected in the product's approved labeling. If we receive marketing approval for beloranib as a treatment for obesity and hyperphagia in patients with PWS or hypothalamic injury-associated obesity, physicians may nevertheless prescribe beloranib to their patients in a manner that is inconsistent with the approved label. If we are found to have promoted such off-label uses, we may become subject to significant liability. The federal government has levied large civil and criminal fines against companies for alleged improper promotion and has enjoined several companies from engaging in off-label promotion. The FDA has also requested that companies enter into consent decrees or permanent injunctions under which specified promotional conduct is changed or curtailed. If we cannot successfully manage the promotion of beloranib, if approved, we could become subject to significant liability, which would materially adversely affect our business and financial condition.

Even if approved, reimbursement policies could limit our ability to sell beloranib.

Market acceptance and sales of beloranib will depend on reimbursement policies and may be affected by healthcare reform measures. Government authorities and third-party payors, such as private health insurers and health maintenance organizations, decide which medications they will pay for and establish reimbursement levels for those medications. Cost containment is a primary concern in the U.S. healthcare industry and elsewhere. Government authorities and these third-party payors have attempted to control costs by limiting coverage and the amount of reimbursement for particular medications. We cannot be sure that reimbursement will be available for beloranib and, if reimbursement is available, the level of such reimbursement. Reimbursement may impact the demand for, or the price of, beloranib. If reimbursement is not available or is available only at limited levels, we may not be able to successfully commercialize beloranib.

In some foreign countries, particularly in Canada and European countries, the pricing of prescription pharmaceuticals is subject to strict governmental control. In these countries, pricing negotiations with governmental authorities can take six to 12 months or longer after the receipt of regulatory approval and product launch. To obtain favorable reimbursement for the indications sought or pricing approval in some countries, we may be required to conduct a clinical trial that compares the cost-effectiveness of beloranib with other available therapies. If reimbursement for beloranib is unavailable in any country in which we seek reimbursement, if it is limited in scope or amount, if it is conditioned upon our completion of additional clinical trials, or if pricing is set at unsatisfactory levels, our operating results could be materially adversely affected.

Even though we have received orphan drug designation for PWS, we may not receive orphan drug exclusivity for beloranib.

As part of our business strategy, we have obtained orphan drug designation in the United States and the European Union for beloranib for the treatment of patients with PWS. In the United States, the company that first obtains FDA approval for a designated orphan drug for the specified rare disease or condition receives orphan drug marketing exclusivity for that drug for a period of seven years. This orphan drug exclusivity prevents the FDA from approving another application, including a full NDA to market the same drug for the same orphan indication, except in very limited circumstances, including when the FDA concludes that the later drug is safer, more effective or makes a major contribution to patient care. For purposes of small molecule drugs, the FDA defines "same drug" as a drug that contains the same active chemical entity and is intended for the same use as the drug in question. To obtain orphan drug exclusivity for a drug that shares the same active chemical entity as an already orphan designated drug, it must be demonstrated to the FDA that the drug is safer or more effective than the approved orphan designated drug, or that it makes a major contribution to patient care. In addition, a designated orphan drug may not receive orphan drug exclusivity if it is approved for a use that is broader than the indication for which it received orphan designation. In addition, orphan drug exclusive marketing rights in the United States may be lost if the FDA later determines that the request for designation was materially defective or if the manufacturer is unable to assure sufficient quantity of the drug to meet the needs of patients with the rare disease or condition or if another drug with the same active moiety is determined to be safer, more effective, or represents a major contribution to patient care. In the European Union, orphan drug designation also entitles a party to financial incentives such as reduction of fees or fee waivers and 10 years of market exclusivity is granted following drug or biological product approval, meaning that another application for marketing authorization of a later similar medicinal product for the same therapeutic indication will generally not be approved in the European Union. This period may be reduced to 6 years if the orphan drug designation criteria are no longer met, including where it is shown that the product is not sufficiently profitable to justify maintenance of market exclusivity.

Our product development programs for candidates other than beloranib may require substantial financial resources and may ultimately be unsuccessful.

In addition to the development of beloranib, we may pursue development of our other early-stage development programs. None of our other potential product candidates has commenced any clinical trials, and there are a number of FDA and certain European regulatory requirements that we must satisfy before we can commence clinical trials. Satisfaction of these requirements will entail substantial time, effort and financial resources. We may never satisfy these requirements. Any time, effort and financial resources we expend on our other early-stage development programs may adversely affect our ability to continue development and commercialization of beloranib, and we may never commence clinical trials of such development programs despite expending significant resources in pursuit of their development. If we do commence clinical trials of our other potential product candidates, such product candidates may never be approved by the FDA or other regulatory authorities.

Risks Relating to Our Intellectual Property Rights

If we are unable to adequately protect our proprietary technology or maintain issued patents which are sufficient to protect beloranib, others could compete against us more directly, which would have a material adverse impact on our business, results of operations, financial condition and prospects.

Our commercial success will depend in part on our success in obtaining and maintaining issued patents and other intellectual property rights in the United States and elsewhere and protecting our proprietary technology. If we do not adequately protect our intellectual property and proprietary technology, competitors may be able to use our technologies and erode or negate any competitive advantage we may have, which could harm our business and ability to achieve profitability.

Our owned and licensed patents and patent applications relate to beloranib compositions of matter, formulations, polymorphs, methods of treating obesity using dosing regimens of beloranib, and methods of treating hypothalamic obesity. The issued U.S. and European patents generally directed to beloranib compositions of matter are exclusively licensed and will each expire in 2019. We own two issued U.S. patents relating to beloranib polymorph compositions of matter that will expire in 2031 and two issued U.S. patents to methods of treating obesity that will expire in 2029. We own pending patent applications in Europe to beloranib polymorph composition of matter and methods of treating obesity that we expect to expire, once issued, in 2031.

As of September 30, 2014, we own four issued U.S. patents, seven pending U.S. patent applications and foreign counterpart applications, and one Patent Cooperation Treaty, or PCT, application that will allow us to seek corresponding protection worldwide, all of which relate to beloranib. We have a license to two U.S. issued patents, one with corresponding issued foreign counterpart patents that also relate to beloranib. We also co-own one patent application relating to methods of using beloranib with an option to exclusively license the co-owner rights.

As of September 30, 2014, we own nine pending U.S. patent applications with pending foreign counterpart applications and three PCT patent applications, all of which relate to our internal efforts to discover novel MetAP2 inhibitor program. Of these, one pending U.S. patent application with pending foreign counterpart patent applications and one PCT patent application relate to our early-stage product candidate ZGN-839.

As of September 30, 2014, we own one issued U.S. patent, two pending U.S. patent applications with pending foreign counterpart patent applications, one pending PCT patent application and two U.S. provisional patent applications that relate to our second-generation injectable MetAP2 inhibitor program.

We cannot provide any assurances that any of our patents have, or that any of our pending patent applications that mature into issued patents will include, claims with a scope sufficient to protect beloranib or our other product candidates. Other parties have developed technologies that may be related or competitive to our approach, and may have filed or may file patent applications and may have received or may receive patents that may overlap or conflict with our patent applications, either by claiming the same methods or formulations or by claiming subject matter that could dominate our patent position. The patent positions of biotechnology and pharmaceutical companies, including our patent position, involve complex legal and factual questions, and, therefore, the issuance, scope, validity and enforceability of any patent claims that we may obtain cannot be predicted with certainty. Patents, if issued, may be challenged, deemed unenforceable, invalidated, or circumvented. U.S. patents and patent applications may also be subject to interference proceedings, *ex parte* reexamination, or *inter partes* review proceedings, supplemental examination and challenges in district court. Patents may be subjected to opposition, post-grant review, or comparable proceedings lodged in various foreign, both national and regional, patent offices. These proceedings could result in either loss of the patent or denial of the patent application or loss or reduction in

the scope of one or more of the claims of the patent or patent application. In addition, such proceedings may be costly. Thus, any patents that we may own or exclusively license may not provide any protection against competitors. Furthermore, an adverse decision in an interference proceeding can result in a third party receiving the patent right sought by us, which in turn could affect our ability to develop, market or otherwise commercialize beloranib.

Furthermore, though an issued patent is presumed valid and enforceable, its issuance is not conclusive as to its validity or its enforceability and it may not provide us with adequate proprietary protection or competitive advantages against competitors with similar products. Competitors may also be able to design around our patents. Other parties may develop and obtain patent protection for more effective technologies, designs or methods. We may not be able to prevent the unauthorized disclosure or use of our technical knowledge or trade secrets by consultants, vendors, former employees and current employees. The laws of some foreign countries do not protect our proprietary rights to the same extent as the laws of the United States, and we may encounter significant problems in protecting our proprietary rights in these countries. If these developments were to occur, they could have a material adverse effect on our sales.

Our ability to enforce our patent rights depends on our ability to detect infringement. It is difficult to detect infringers who do not advertise the components that are used in their products. Moreover, it may be difficult or impossible to obtain evidence of infringement in a competitor's or potential competitor's product. Any litigation to enforce or defend our patent rights, even if we were to prevail, could be costly and time-consuming and would divert the attention of our management and key personnel from our business operations. We may not prevail in any lawsuits that we initiate and the damages or other remedies awarded if we were to prevail may not be commercially meaningful.

In addition, proceedings to enforce or defend our patents could put our patents at risk of being invalidated, held unenforceable, or interpreted narrowly. Such proceedings could also provoke third parties to assert claims against us, including that some or all of the claims in one or more of our patents are invalid or otherwise unenforceable. If any of our patents covering beloranib are invalidated or found unenforceable, our financial position and results of operations would be materially and adversely impacted. In addition, if a court found that valid, enforceable patents held by third parties covered beloranib, our financial position and results of operations would also be materially and adversely impacted.

The degree of future protection for our proprietary rights is uncertain, and we cannot ensure that:

- any of our patents, or any of our pending patent applications, if issued, will include claims having a scope sufficient to protect beloranib or any other products or product candidates;
- any of our pending patent applications will issue as patents;
- we will be able to successfully commercialize beloranib, if approved, before our relevant patents expire;
- we were the first to make the inventions covered by each of our patents and pending patent applications;
- we were the first to file patent applications for these inventions;
- others will not develop similar or alternative technologies that do not infringe our patents;
- any of our patents will be found to ultimately be valid and enforceable;
- any patents issued to us will provide a basis for an exclusive market for our commercially viable products, will provide us with any competitive advantages or will not be challenged by third parties;
- we will develop additional proprietary technologies or product candidates that are separately patentable; or
- that our commercial activities or products will not infringe upon the patents of others.

We rely upon unpatented trade secrets, unpatented know-how and continuing technological innovation to develop and maintain our competitive position, which we seek to protect, in part, by confidentiality agreements with our employees and our collaborators and consultants. We also have agreements with our employees and selected consultants that obligate them to assign their inventions to us and have non-compete agreements with some, but not all, of our consultants. It is possible that technology relevant to our business will be independently developed by a person that is not a party to such an agreement. Furthermore, if the employees and consultants who are parties to these agreements breach or violate the terms of these agreements, we may not have adequate remedies for any such breach or violation, and we could lose our trade secrets through such breaches or violations. Further, our trade secrets could otherwise become known or be independently discovered by our competitors.

We may infringe the intellectual property rights of others, which may prevent or delay our product development efforts and stop us from commercializing or increase the costs of commercializing beloranib, if approved.

Our success will depend in part on our ability to operate without infringing the intellectual property and proprietary rights of third parties. We cannot assure you that our business, products and methods do not or will not infringe the patents or other intellectual property rights of third parties.

The pharmaceutical industry is characterized by extensive litigation regarding patents and other intellectual property rights. Other parties may allege that beloranib or the use of our technologies infringes patent claims or other intellectual property rights held by them or that we are employing their proprietary technology without authorization. Patent and other types of intellectual property litigation can involve complex factual and legal questions, and their outcome is uncertain. Any claim relating to intellectual property infringement that is successfully asserted against us may require us to pay substantial damages, including treble damages and attorney's fees if we are found to be willfully infringing another party's patents, for past use of the asserted intellectual property and royalties and other consideration going forward if we are forced to take a license. In addition, if any such claim were successfully asserted against us and we could not obtain such a license, we may be forced to stop or delay developing, manufacturing, selling or otherwise commercializing beloranib.

Even if we are successful in these proceedings, we may incur substantial costs and divert management time and attention in pursuing these proceedings, which could have a material adverse effect on us. If we are unable to avoid infringing the patent rights of others, we may be required to seek a license, defend an infringement action or challenge the validity of the patents in court, or redesign our products. Patent litigation is costly and time consuming. We may not have sufficient resources to bring these actions to a successful conclusion. In addition, intellectual property litigation or claims could force us to do one or more of the following:

- cease developing, selling or otherwise commercializing beloranib;
- pay substantial damages for past use of the asserted intellectual property;
- obtain a license from the holder of the asserted intellectual property, which license may not be available on reasonable terms, if at all; and
- in the case of trademark claims, redesign, or rename, beloranib to avoid infringing the intellectual property rights of third parties, which may not be possible and, even if possible, could be costly and time-consuming.

Any of these risks coming to fruition could have a material adverse effect on our business, results of operations, financial condition and prospects.

We may be subject to claims challenging the inventorship or ownership of our patents and other intellectual property.

We may also be subject to claims that former employees, collaborators or other third parties have an ownership interest in our patents or other intellectual property. Litigation may be necessary to defend against these and other claims challenging inventorship or ownership. If we fail in defending any such claims, in addition to paying monetary damages, we may lose valuable intellectual property rights, such as exclusive ownership of, or right to use, valuable intellectual property. Such an outcome could have a material adverse effect on our business. Even if we are successful in defending against such claims, litigation could result in substantial costs and be a distraction to management and other employees.

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

The U.S. Patent and Trademark Office, or U.S. PTO, and various foreign governmental patent agencies require compliance with a number of procedural, documentary, fee payment and other provisions during the patent process. There are situations in which noncompliance can result in abandonment or lapse of a patent or patent application, resulting in partial or complete loss of patent rights in the relevant jurisdiction. In such an event, competitors might be able to enter the market earlier than would otherwise have been the case.

We may be involved in lawsuits to protect or enforce our patents or the patents of our licensors, which could be expensive, time-consuming and unsuccessful.

Competitors may infringe our patents or the patents of our licensors. To counter infringement or unauthorized use, we may be required to file infringement claims, which can be expensive and time-consuming. In addition, in an infringement

proceeding, a court may decide that a patent of ours or our licensors is not valid, is unenforceable and/or is not infringed, or may refuse to stop the other party from using the technology at issue on the grounds that our patents do not cover the technology in question. An adverse result in any litigation or defense proceedings could put one or more of our patents at risk of being invalidated or interpreted narrowly and could put our patent applications at risk of not issuing.

Interference proceedings provoked by third parties or brought by us may be necessary to determine the priority of inventions with respect to our patents or patent applications or those of our licensors. An unfavorable outcome could require us to cease using the related technology or to attempt to license rights to it from the prevailing party. Our business could be harmed if the prevailing party does not offer us a license on commercially reasonable terms. Our defense of litigation or interference proceedings may fail and, even if successful, may result in substantial costs and distract our management and other employees. We may not be able to prevent, alone or with our licensors, misappropriation of our intellectual property rights, particularly in countries where the laws may not protect those rights as fully as in the United States.

Furthermore, because of the substantial amount of discovery required in connection with intellectual property litigation, there is a risk that some of our confidential information could be compromised by disclosure during this type of litigation. There could also be public announcements of the results of hearings, motions or other interim proceedings or developments. If securities analysts or investors perceive these results to be negative, it could have a material adverse effect on the price of our common stock.

Issued patents covering our product candidates could be found invalid or unenforceable if challenged in court.

If we or one of our licensing partners initiated legal proceedings against a third party to enforce a patent covering our product candidate, the defendant could counterclaim that the patent covering our product candidate is invalid and/or unenforceable. In patent litigation in the United States, defendant counterclaims alleging invalidity and/or unenforceability are commonplace. Grounds for a validity challenge include alleged failures to meet any of several statutory requirements, including lack of novelty, obviousness or non-enablement. Grounds for unenforceability assertions include allegations that someone connected with prosecution of the patent withheld relevant information from the U.S. PTO, or made a misleading statement, during prosecution. Third parties may also raise similar claims before administrative bodies in the United States or abroad, even outside the context of litigation. Such mechanisms include re-examination, post grant review and equivalent proceedings in foreign jurisdictions, e.g., opposition proceedings. Such proceedings could result in revocation or amendment of our patents in such a way that they no longer cover our product candidates or competitive products. The outcome following legal assertions of invalidity and unenforceability is unpredictable. With respect to validity, for example, we cannot be certain that there is no invalidating prior art, of which we and the patent examiner were unaware during prosecution. If a defendant were to prevail on a legal assertion of invalidity and/or unenforceability, we would lose at least part, and perhaps all, of the patent protection on our product candidates. Such a loss of patent protection would have a material adverse impact on our business.

We do not seek to protect our intellectual property rights in all jurisdictions throughout the world and we may not be able to adequately enforce our intellectual property rights even in the jurisdictions where we seek protection.

Filing, prosecuting and defending patents on product candidates in all countries and jurisdictions throughout the world would be prohibitively expensive, and our intellectual property rights in some countries outside the United States could be less extensive than those in the United States. In addition, the laws of some foreign countries do not protect intellectual property rights to the same extent as federal and state laws in the United States. Consequently, we may not be able to prevent third parties from practicing our inventions in all countries outside the United States, or from selling or importing products made using our inventions in and into the United States or other jurisdictions. Competitors may use our technologies in jurisdictions where we have not obtained patent protection to develop their own products and further, may export otherwise infringing products to territories where we have patent protection, but enforcement is not as strong as that in the United States. These products may compete with our products and our patents or other intellectual property rights may not be effective or sufficient to prevent them from competing.

Many companies have encountered significant problems in protecting and defending intellectual property rights in foreign jurisdictions. The legal systems of certain countries, particularly certain developing countries, do not favor the enforcement of patents and other intellectual property protection, particularly those relating to biopharmaceuticals, which could make it difficult for us to stop the infringement of our patents or marketing of competing products in violation of our proprietary rights generally. Proceedings to enforce our patent rights in foreign jurisdictions could result in substantial costs and divert our efforts and attention from other aspects of our business, could put our patents at risk of being invalidated or interpreted narrowly, could put our patent applications at risk of not issuing and could provoke third parties to assert claims against us. We may not prevail in any lawsuits that we initiate and the damages or other remedies awarded, if any, may not be commercially meaningful. Accordingly, our efforts to enforce our intellectual property rights around the world may be inadequate to obtain a significant commercial advantage from the intellectual property that we develop or license.

We are dependent on licensed intellectual property. If we were to lose our rights to licensed intellectual property, we may not be able to continue developing or commercializing beloranib or our other product candidates, if approved.

We have licensed our rights to beloranib from Chong Kun Dang Pharmaceutical Corp. of South Korea, or CKD. Our license with CKD imposes various obligations on us, including a requirement to use commercially reasonable efforts to develop beloranib and provides CKD the right to terminate the license thereunder in the event of a material breach. For example, CKD may allege that we have breached our license agreement and may accordingly seek to terminate our license with them. Termination of our license from CKD could result in our loss of the right to use the licensed intellectual property, which could materially adversely affect our ability to develop and commercialize beloranib, if approved, as well as harm our competitive business position and our business prospects. We also have an exclusive license with Children's Medical Center Corporation, or Children's, pursuant to which we exclusively licensed certain patient rights relating to decreasing the growth of fat tissue from Children's on a worldwide basis.

We may enter into additional license(s) to third-party intellectual property that are necessary or useful to our business. Future licensor(s) may also allege that we have breached our license agreement and may accordingly seek to terminate our license with them. In addition, future licensor(s) may decide to terminate our license at will. If successful, this could result in our loss of the right to use the licensed intellectual property, which could materially adversely affect our ability to develop and commercialize a product candidate or product, if approved, as well as harm our competitive business position and our business prospects.

We have not yet registered trademarks for a commercial trade name for beloranib and failure to secure such registrations could adversely affect our business.

We have not yet registered trademarks for a commercial trade name for beloranib. Any future trademark applications may be rejected during trademark registration proceedings. Although we would be given an opportunity to respond to those rejections, we may be unable to overcome such rejections. In addition, in the U.S. PTO and in comparable agencies in many foreign jurisdictions, third parties are given an opportunity to oppose pending trademark applications and to seek to cancel registered trademarks. Opposition or cancellation proceedings may be filed against our trademarks, and our trademarks may not survive such proceedings. Moreover, any name we propose to use with our product candidates in the United States must be approved by the FDA, regardless of whether we have registered it, or applied to register it, as a trademark. The FDA typically conducts a review of proposed product names, including an evaluation of potential for confusion with other product names. If the FDA objects to any of our proposed proprietary product names, we may be required to expend significant additional resources in an effort to identify a suitable substitute name that would qualify under applicable trademark laws, not infringe the existing rights of third parties and be acceptable to the FDA.

If we do not obtain additional protection under the Hatch-Waxman Amendments and similar foreign legislation by extending the patent terms and obtaining data exclusivity for beloranib, our business may be materially harmed.

Depending upon the timing, duration and specifics of FDA marketing approval of beloranib, one or more of the U.S. patents we license may be eligible for limited patent term restoration under the Drug Price Competition and Patent Term Restoration Act of 1984, referred to as the Hatch-Waxman Amendments. The Hatch-Waxman Amendments permit a patent restoration term of up to five years as compensation for patent term lost during product development and the FDA regulatory review process. However, we may not be granted an extension because of, for example, failing to apply within applicable deadlines, failing to apply prior to expiration of relevant patents or otherwise failing to satisfy applicable requirements. Moreover, the applicable time period or the scope of patent protection afforded could be less than we request. If we are unable to obtain patent term extension or restoration or the term of any such extension is less than we request, our competitors may obtain approval of competing products following our patent expiration, and our ability to generate revenues could be materially adversely affected.

Changes in U.S. patent law could diminish the value of patents in general, thereby impairing our ability to protect our products.

The United States has recently enacted and is currently implementing the America Invents Act of 2011, which is wide-ranging patent reform legislation. Further, the U.S. Supreme Court has ruled on several patent cases in recent years, either narrowing the scope of patent protection available in certain circumstances or weakening the rights of patent owners in certain situations. In addition to increasing uncertainty with regard to our ability to obtain future patents, this combination of

events has created uncertainty with respect to the value of patents, once obtained. Depending on decisions by the U.S. Congress, the federal courts and the U.S. PTO, the laws and regulations governing patents could change in unpredictable ways that would weaken our ability to obtain new patents or to enforce our existing patents or future patents.

We may be subject to damages resulting from claims that we or our employees have wrongfully used or disclosed alleged trade secrets of their former employers.

Our employees have been previously employed at other biotechnology or pharmaceutical companies, including our competitors or potential competitors. Although we are not aware of any claims currently pending against us, we may be subject to claims that these employees or we have inadvertently or otherwise used or disclosed trade secrets or other proprietary information of the former employers of our employees. Litigation may be necessary to defend against these claims. Even if we are successful in defending against these claims, litigation could result in substantial costs and be a distraction to management. If we fail in defending such claims, in addition to paying money claims, we may lose valuable intellectual property rights or personnel. A loss of key personnel or their work product could hamper or prevent our ability to commercialize beloranib, which would materially adversely affect our commercial development efforts.

General Company-Related Risks

We will need to develop and expand our company, and we may encounter difficulties in managing this development and expansion, which could disrupt our operations.

As of September 30, 2014, we had 18 full-time employees and one part-time employee, and we expect to increase our number of employees and the scope of our operations as we advance beloranib into later clinical trials. To manage our anticipated development and expansion, we must continue to implement and improve our managerial, operational and financial systems, expand our facilities and continue to recruit and train additional qualified personnel. Also, our management may need to divert a disproportionate amount of its attention away from its day-to-day activities and devote a substantial amount of time to managing these development activities. Due to our limited resources, we may not be able to effectively manage the expansion of our operations or recruit and train additional qualified personnel. This may result in weaknesses in our infrastructure, give rise to operational mistakes, loss of business opportunities, loss of employees and reduced productivity among remaining employees. The physical expansion of our operations may lead to significant costs and may divert financial resources from other projects, such as the development of beloranib. If our management is unable to effectively manage our expected development and expansion, our expenses may increase more than expected, our ability to generate or increase our revenue could be reduced and we may not be able to implement our business strategy. Our future financial performance and our ability to commercialize beloranib, if approved, and compete effectively will depend, in part, on our ability to effectively manage the future development and expansion of our company.

Our future success depends on our ability to retain our Chief Executive Officer, and to attract, retain and motivate qualified personnel.

We are highly dependent on Dr. Thomas E. Hughes, our Chief Executive Officer. We have entered into an employment agreement with Dr. Hughes, but he may terminate his employment with us at any time. Although we do not have any reason to believe that we will lose the services of Dr. Hughes in the foreseeable future, the loss of his services might impede the achievement of our research, development and commercialization objectives. We also do not have any key-man life insurance on Dr. Hughes. We rely on consultants and advisors, including scientific and clinical advisors, to assist us in formulating our development and commercialization strategy. Our consultants and advisors may be employed by employers other than us and may have commitments under consulting or advisory contracts with other entities that may limit their availability to us and may not be subject to our standard non-compete agreements. Recruiting and retaining qualified scientific personnel and sales and marketing personnel will also be critical to our success. We may not be able to attract and retain these personnel on acceptable terms given the competition among numerous pharmaceutical and biotechnology companies for similar personnel. We also experience competition for the hiring of scientific personnel from universities and research institutions. Failure to succeed in clinical trials may make it more challenging to recruit and retain qualified scientific personnel.

Our employees may engage in misconduct or other improper activities, including violating applicable regulatory standards and requirements or engaging in insider trading, which could significantly harm our business.

We are exposed to the risk of employee fraud or other misconduct. Misconduct by employees could include intentional failures to comply with the regulations of the FDA and applicable non-U.S. regulators, provide accurate information to the FDA and applicable non-U.S. regulators, comply with healthcare fraud and abuse laws and regulations in the United States

and abroad, report financial information or data accurately or disclose unauthorized activities to us. In particular, sales, marketing and business arrangements in the healthcare industry are subject to extensive laws and regulations intended to prevent fraud, misconduct, kickbacks, self-dealing and other abusive practices. These laws and regulations restrict or prohibit a wide range of pricing, discounting, marketing and promotion, sales commission, customer incentive programs and other business arrangements. Employee misconduct could also involve the improper use of, including trading on, information obtained in the course of clinical trials, which could result in regulatory sanctions and serious harm to our reputation. We have adopted a code of conduct, but it is not always possible to identify and deter employee misconduct, and the precautions we take to detect and prevent this activity may be ineffective in controlling unknown or unmanaged risks or losses or in protecting us from governmental investigations or other actions or lawsuits stemming from a failure to comply with these laws or regulations. If any such actions are instituted against us, and we are not successful in defending ourselves or asserting our rights, those actions could have a significant impact on our business, including the imposition of significant fines or other sanctions.

We face potential product liability exposure, and, if claims are brought against us, we may incur substantial liability.

The use of beloranib in clinical trials and the sale of beloranib, if approved, exposes us to the risk of product liability claims. Product liability claims might be brought against us by patients, healthcare providers or others selling or otherwise coming into contact with beloranib. For example, we may be sued if any product we develop allegedly causes injury or is found to be otherwise unsuitable during product testing, manufacturing, marketing or sale. Any such product liability claims may include allegations of defects in manufacturing, defects in design, a failure to warn of dangers inherent in the product, including as a result of interactions with alcohol or other drugs, negligence, strict liability and a breach of warranties. Claims could also be asserted under state consumer protection acts. If we become subject to product liability claims and cannot successfully defend ourselves against them, we could incur substantial liabilities. In addition, regardless of merit or eventual outcome, product liability claims may result in, among other things:

- withdrawal of patients from our clinical trials;
- substantial monetary awards to patients or other claimants;
- decreased demand for beloranib or any future product candidates following marketing approval, if obtained;
- damage to our reputation and exposure to adverse publicity;
- increased FDA warnings on product labels;
- litigation costs;
- distraction of management's attention from our primary business;
- loss of revenue; and
- the inability to successfully commercialize beloranib or any future product candidates, if approved.

We maintain product liability insurance coverage for our clinical trials with a $10.0 million annual aggregate coverage limit. Nevertheless, our insurance coverage may be insufficient to reimburse us for any expenses or losses we may suffer. Moreover, in the future, we may not be able to maintain insurance coverage at a reasonable cost or in sufficient amounts to protect us against losses, including if insurance coverage becomes increasingly expensive. If and when we obtain marketing approval for beloranib, we intend to expand our insurance coverage to include the sale of commercial products; however, we may not be able to obtain this product liability insurance on commercially reasonable terms. Large judgments have been awarded in class action lawsuits based on drugs that had unanticipated side effects. The cost of any product liability litigation or other proceedings, even if resolved in our favor, could be substantial, particularly in light of the size of our business and financial resources. A product liability claim or series of claims brought against us could cause our stock price to decline and, if we are unsuccessful in defending such a claim or claims and the resulting judgments exceed our insurance coverage, our financial condition, business and prospects could be materially adversely affected.

We must maintain effective internal control over financial reporting, and if we are unable to do so, the accuracy and timeliness of our financial reporting may be adversely affected, which could have a material adverse effect on our business and stock price.

We must maintain effective internal control over financial reporting in order to accurately and timely report our results of operations and financial condition. In addition, as a public company, the Sarbanes-Oxley Act of 2002, or the Sarbanes-Oxley Act, requires, among other things, that we assess the effectiveness of our disclosure controls and procedures quarterly and the effectiveness of our internal control over financial reporting at the end of each fiscal year. We anticipate being first

required to issue management's annual report on internal control over financial reporting, pursuant to Section 404 of the Sarbanes-Oxley Act, in connection with issuing our consolidated financial statements as of and for the year ending December 31, 2015.

During the course of preparing for our initial public offering, or IPO, we determined that material adjustments to various accounts were necessary, which required us to restate our consolidated financial statements as of and for the year ended December 31, 2011 and for the period from inception (November 22, 2005) through December 31, 2011 that had been previously audited by another independent audit firm. These adjustments leading to a restatement of those consolidated financial statements led us to conclude that we had material weaknesses in internal control over financial reporting as of December 31, 2011 as follows: (i) we did not maintain effective controls over accounting policies and application of GAAP—specifically, we did not maintain and communicate sufficient accounting policies, which limited our ability to make accounting decisions and to detect and correct accounting errors; and (ii) we did not maintain a sufficient complement of resources with an appropriate level of accounting knowledge, experience and training commensurate with our structure and financial reporting requirements.

During 2013, we executed on various remediation efforts, including ensuring we had sufficient resources with the appropriate technical accounting expertise and putting in place formalized policies and procedures to ensure complete and accurate consolidated financial statements are prepared. In that year, we hired additional senior accounting and finance employees, including a Chief Financial Officer with significant biotechnology industry experience, and engaged external consultants with significant financial and accounting technical experience. These additional resources have enabled us to (i) implement standardized financial reporting policies and procedures and a more structured close process and (ii) implement financial data reviews that involve separate preparation and review of the monthly, quarterly and annual financial data, reconciliations, analyses and information. Based on our assessment of the additional resources and our enhanced controls and procedures, our management concluded that, as of December 31, 2013, we had remediated the material weaknesses in our internal control over financial reporting described above.

The rules governing the standards that must be met for our management to assess our internal control over financial reporting pursuant to Section 404 of the Sarbanes-Oxley Act are complex and require significant documentation, testing and possible remediation. These stringent standards require that our audit committee be advised and regularly updated on management's review of internal control over financial reporting. Our management may not be able to effectively and timely implement controls and procedures that adequately respond to the increased regulatory compliance and reporting requirements that are applicable to us as a public company. If we fail to staff our accounting and finance function adequately or maintain internal control over financial reporting adequate to meet the demands that will be placed upon us as a public company, including the requirements of the Sarbanes-Oxley Act, our business and reputation may be harmed and our stock price may decline. Furthermore, investor perceptions of us may be adversely affected, which could cause a decline in the market price of our common stock.

In order to satisfy our obligations as a public company, we will need to hire additional qualified accounting and financial personnel with appropriate public company experience.

As a newly public company, we will need to establish and maintain effective disclosure and financial controls and make changes in our corporate governance practices. We will need to hire additional accounting and financial personnel with appropriate public company experience and technical accounting knowledge, and it may be difficult to recruit and maintain such personnel. Even if we are able to hire appropriate personnel, our existing operating expenses and operations will be impacted by the direct costs of their employment and the indirect consequences related to the diversion of management resources from product development efforts.

Our ability to use our net operating loss carryforwards and certain tax credit carryforwards may be subject to limitation.

As of December 31, 2013, we had federal and state net operating loss carryforwards of $10.5 million and $8.2 million, respectively. Our federal net operating loss carryforwards begin to expire in 2026, and our state net operating loss carryforwards began to expire in 2014. As of December 31, 2013, we also had federal and state research and development tax credit carryforwards of $4.7 million and $1.3 million, respectively, which begin to expire in 2026 and 2021, respectively. Under Section 382 of the Internal Revenue Code of 1986, as amended, or the Code, changes in our ownership may limit the amount of our net operating loss carryforwards and research and development tax credit carryforwards that could be utilized annually to offset our future taxable income, if any. This limitation would generally apply in the event of a cumulative change in ownership of our company of more than 50% within a three-year period. Any such limitation may significantly reduce our ability to utilize our net operating loss carryforwards and research and development tax credit carryforwards before they expire. The completion of our IPO, together with private placements and other transactions that

have occurred since our inception, may trigger such an ownership change pursuant to Section 382. Any such limitation, whether as the result of our IPO, prior private placements, sales of our common stock by our existing stockholders or additional sales of our common stock by us, could have a material adverse effect on our results of operations in future years. We have not completed a study to assess whether an ownership change for purposes of Section 382 has occurred, or whether there have been multiple ownership changes since our inception, due to the significant costs and complexities associated with such study.

Unfavorable global economic conditions could adversely affect our business, financial condition or results of operations.

Our results of operations could be adversely affected by general conditions in the global economy and in the global financial markets. The recent global financial crisis caused extreme volatility and disruptions in the capital and credit markets. A severe or prolonged economic downturn, such as the recent global financial crisis, could result in a variety of risks to our business, including, weakened demand for our product candidates and our ability to raise additional capital when needed on acceptable terms, if at all. A weak or declining economy could also strain our suppliers, possibly resulting in supply disruption, or cause our customers to delay making payments for our services. Any of the foregoing could harm our business and we cannot anticipate all of the ways in which the current economic climate and financial market conditions could adversely impact our business.

Our internal computer systems, or those of our third-party CROs or other contractors or consultants, may fail or suffer security breaches, which could result in a material disruption of our beloranib development programs.

Despite the implementation of security measures, our internal computer systems and those of our third-party CROs and other contractors and consultants are vulnerable to damage from computer viruses, unauthorized access, natural disasters, terrorism, war and telecommunication and electrical failures. While we have not experienced any such system failure, accident, or security breach to date, if such an event were to occur and cause interruptions in our operations, it could result in a material disruption of our programs. For example, the loss of clinical trial data for beloranib could result in delays in our regulatory approval efforts and significantly increase our costs to recover or reproduce the data. To the extent that any disruption or security breach results in a loss of or damage to our data or applications or other data or applications relating to our technology or product candidates, or inappropriate disclosure of confidential or proprietary information, we could incur liabilities and the further development of beloranib could be delayed.

We may not be successful in our efforts to identify or discover additional product candidates.

The success of our business depends primarily upon our ability to identify, develop and commercialize products based on our weight loss platform. Although beloranib is currently in clinical development, our research programs may fail to identify other potential product candidates for clinical development for a number of reasons. Our research methodology may be unsuccessful in identifying potential product candidates or our potential product candidates may be shown to have harmful side effects or may have other characteristics that may make the products unmarketable or unlikely to receive marketing approval.

If any of these events occur, we may be forced to abandon our development efforts for a program or programs, which would have a material adverse effect on our business and could potentially cause us to cease operations. Research programs to identify new product candidates require substantial technical, financial and human resources. We may focus our efforts and resources on potential programs or product candidates that ultimately prove to be unsuccessful.

We may acquire businesses or products, or form strategic alliances, in the future, and we may not realize the benefits of such acquisitions.

We may acquire additional businesses or products, form strategic alliances or create joint ventures with third parties that we believe will complement or augment our existing business. If we acquire businesses with promising markets or technologies, we may not be able to realize the benefit of acquiring such businesses if we are unable to successfully integrate them with our existing operations and company culture. We may encounter numerous difficulties in developing, manufacturing and marketing any new products resulting from a strategic alliance or acquisition that delay or prevent us from realizing their expected benefits or enhancing our business. We cannot assure you that, following any such acquisition, we will achieve the expected synergies to justify the transaction.

Risks Related to Our Financial Position and Need for Capital

We are an early stage biopharmaceutical company with a limited operating history and have not generated any revenue from product sales. We have incurred significant operating losses since our inception, and anticipate that we will incur continued losses for the foreseeable future.

We are an early stage company with a limited operating history. Biopharmaceutical product development is a highly speculative undertaking and involves a substantial degree of risk. We were incorporated in November 2005. Our operations to date have been limited primarily to organizing and staffing our company and conducting research and development activities for beloranib and ZGN-839. We have never generated any revenue from product sales. We have not obtained regulatory approvals for any of our product candidates.

Since our inception, we have focused substantially all of our efforts and financial resources on developing beloranib, which is currently in Phase 3 clinical development for one indication. We have funded our operations to date through proceeds from sales of redeemable convertible preferred stock, convertible debt and our IPO and have incurred losses in each year since our inception. Our net losses were $25.5 million for the nine months ended September 30, 2014. As of September 30, 2014, we had an accumulated deficit of $94.5 million. Substantially all of our operating losses resulted from costs incurred in connection with our development programs for beloranib and ZGN-839, licensing milestone fees and from general and administrative costs associated with our operations. We expect to incur increasing levels of operating losses over the next several years and for the foreseeable future. Our prior losses, combined with expected future losses, have had and will continue to have an adverse effect on our stockholders' deficit and working capital. We expect our research and development expenses to significantly increase in connection with our additional clinical trials of beloranib and development of ZGN-839 and of any other product candidates we may choose to pursue. In addition, if we obtain marketing approval for beloranib, we will incur significant sales, marketing and outsourced manufacturing expenses. Now that we are a public company, we will incur additional costs associated with operating as a public company. As a result, we expect to continue to incur significant and increasing operating losses for the foreseeable future. Because of the numerous risks and uncertainties associated with developing pharmaceutical products, we are unable to predict the extent of any future losses or when we will become profitable, if at all. Even if we do become profitable, we may not be able to sustain or increase our profitability on a quarterly or annual basis.

Our ability to become profitable depends upon our ability to generate revenue. To date, we have not generated any revenue from our lead product candidate, beloranib, and we do not know when, or if, we will generate any revenue. We do not expect to generate significant revenue unless and until we obtain marketing approval of, and begin to sell, beloranib. Our ability to generate revenue depends on a number of factors, including, but not limited to, our ability to:

- initiate and successfully complete later-stage clinical trials that meet their clinical endpoints;
- initiate and successfully complete all safety studies required to obtain U.S. and foreign marketing approval for beloranib as a treatment for obesity and hyperphagia in patients with PWS, hypothalamic injury-associated obesity and severely obese patients in the general population;
- commercialize beloranib, if approved, by developing a sales force or entering into collaborations with third parties; and
- achieve market acceptance of beloranib in the medical community and with third-party payors.

Absent our entering into a collaboration or partnership agreement, we expect to incur significant sales and marketing costs as we prepare to commercialize beloranib. Even if we initiate and successfully complete our pivotal clinical trials of beloranib, and beloranib is approved for commercial sale, and despite expending these costs, beloranib may not be a commercially successful drug. We may not achieve profitability soon after generating product sales, if ever. If we are unable to generate product revenue, we will not become profitable and may be unable to continue operations without continued funding.

We will need to raise additional funding, which may not be available on acceptable terms, or at all. Failure to obtain this necessary capital when needed may force us to delay, limit or terminate our product development efforts or other operations.

We are currently advancing our beloranib product candidate through clinical development. Developing small molecule products is expensive, and we expect our research and development expenses to increase substantially in connection with our ongoing activities, particularly as we advance beloranib in later, more costly, clinical trials. Depending on the status of regulatory approval or, if approved, commercialization of beloranib, as well as the progress we make in selling beloranib, we

may require additional capital to fund operating needs thereafter. We may also need to raise additional funds sooner if we choose to pursue additional indications and/or geographies for beloranib or otherwise expand more rapidly than we presently anticipate.

As of September 30, 2014, our cash and cash equivalents were $127.0 million, which will be sufficient to fund our current operations for at least the next 12 months. However, our operating plan may change as a result of many factors currently unknown to us, and we may need to seek additional funds sooner than planned, through public or private equity or debt financings, government or other third-party funding, marketing and distribution arrangements and other collaborations, strategic alliances and licensing arrangements or a combination of these approaches. In any event, we will require additional capital to obtain regulatory approval for, and to commercialize, our product candidates. Raising funds in the current economic environment may present additional challenges. Even if we believe we have sufficient funds for our current or future operating plans, we may seek additional capital if market conditions are favorable or if we have specific strategic considerations.

Any additional fundraising efforts may divert our management from their day-to-day activities, which may adversely affect our ability to develop and commercialize our product candidates. In addition, we cannot guarantee that future financing will be available in sufficient amounts or on terms acceptable to us, if at all. Moreover, the terms of any financing may adversely affect the holdings or the rights of our stockholders and the issuance of additional securities, whether equity or debt, by us, or the possibility of such issuance, may cause the market price of our shares to decline. The sale of additional equity or convertible securities would dilute all of our stockholders. In addition, the terms of any such securities may include liquidation or other preferences that materially adversely affect your rights as a stockholder. The incurrence of indebtedness would result in increased fixed payment obligations and we may be required to agree to certain restrictive covenants, such as limitations on our ability to incur additional debt, limitations on our ability to acquire, sell or license intellectual property rights and other operating restrictions that could adversely impact our ability to conduct our business. We could also be required to seek funds through arrangements with collaborative partners or otherwise at an earlier stage than otherwise would be desirable and we may be required to relinquish rights to some of our technologies or product candidate or otherwise agree to terms unfavorable to us, any of which may have a material adverse effect on our business, operating results and prospects.

If we are unable to obtain funding on a timely basis, we may be required to significantly curtail, delay or discontinue one or more of our research or development programs or the commercialization of any product candidate or be unable to expand our operations or otherwise capitalize on our business opportunities, as desired, which could materially affect our business, financial condition and results of operations.

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

We may seek additional capital through a combination of private and public equity offerings, debt financings, collaborations and strategic and licensing arrangements. To the extent that we raise additional capital through the sale of common stock or securities convertible or exchangeable into common stock, your ownership interest in our company will be diluted. In addition, the terms of any such securities may include liquidation or other preferences that materially adversely affect your rights as a stockholder. Debt financing, if available, would increase our fixed payment obligations and may involve agreements that include covenants limiting or restricting our ability to take specific actions, such as incurring additional debt, making capital expenditures or declaring dividends. If we raise additional funds through collaboration, strategic partnerships and licensing arrangements with third parties, we may have to relinquish valuable rights to beloranib, our intellectual property, future revenue streams or grant licenses on terms that are not favorable to us.

Risks Related to Our Common Stock

We expect that our stock price may fluctuate significantly.

Our IPO was completed on June 24, 2014 at a price of $16.00 per share. Therefore, there has been a public market for our common stock for only a short period of time. Although our common stock is listed on the NASDAQ Global Market, an active public market for our common stock may not develop or be sustained.

In addition, the market price of shares of our common stock could be subject to wide fluctuations in response to many risk factors listed in this section, and others beyond our control, including:

- plans for, progress of or results from pre-clinical studies and clinical trials of beloranib;
- the failure of the FDA or the EMA to approve beloranib;

- announcements of new products, technologies, commercial relationships, acquisitions or other events by us or our competitors;
- the success or failure of other weight loss therapies;
- regulatory or legal developments in the United States and other countries;
- failure of beloranib, if approved, to achieve commercial success;
- fluctuations in stock market prices and trading volumes of similar companies;
- general market conditions and overall fluctuations in U.S. equity markets;
- variations in our quarterly operating results;
- changes in our financial guidance or securities analysts' estimates of our financial performance;
- changes in accounting principles;
- our ability to raise additional capital and the terms on which we can raise it;
- sales of large blocks of our common stock, including sales by our executive officers, directors and significant stockholders;
- additions or departures of key personnel;
- discussion of us or our stock price by the press and by online investor communities; and
- other risks and uncertainties described in these risk factors.

These and other market and industry factors may cause the market price and demand for our common stock to fluctuate substantially, regardless of our actual operating performance, which may limit or prevent investors from readily selling their shares of common stock and may otherwise negatively affect the liquidity of our common stock. In addition, the stock market in general, and NASDAQ and biopharmaceutical companies in particular, have experienced extreme price and volume fluctuations that have often been unrelated or disproportionate to the operating performance of these companies. In the past, when the market price of a stock has been volatile, holders of that stock have instituted securities class action litigation against the company that issued the stock. If any of our stockholders brought a lawsuit against us, we could incur substantial costs defending the lawsuit. Such a lawsuit could also divert the time and attention of our management.

Our executive officers, directors and principal stockholders exercise significant control over our company.

Our executive officers, directors, principal stockholders and their affiliates, including investment funds affiliated with Atlas Ventures, or Atlas, investment funds affiliated with Third Rock Ventures, or TRV, investment funds affiliated with Alta Partners, or Alta, and entities affiliated with Fidelity Investment, or Fidelity, represent beneficial ownership, in the aggregate, of greater than 60% of our outstanding common stock as of September 30, 2014. As a result, these stockholders, if they act together, will be able to influence our management and affairs and control the outcome of matters submitted to our stockholders for approval, including the election of directors and any sale, merger, consolidation, or sale of all or substantially all of our assets. In addition, this concentration of ownership might adversely affect the market price of our common stock by:

- delaying, deferring or preventing a change of control of us;
- impeding a merger, consolidation, takeover or other business combination involving us; or
- discouraging a potential acquirer from making a tender offer or otherwise attempting to obtain control of us.

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

A significant number of our outstanding shares are subject to a180-day contractual lock-up and other legal restrictions on resale pursuant to lock-up agreements that our officers, directors and a significant majority of our stockholders executed in connection with our IPO. If these stockholders sell, or indicate an intent to sell, substantial amounts of our common stock in the public market after the expiration of the lock-up period, the trading price of our common stock could decline significantly and could decline below the IPO price.

After the lock-up agreements expire, based upon the number of shares of common stock outstanding as of September 30, 2014, up to an additional 15,799,051 shares of common stock will be eligible for sale in the public market, of which 11,625,778 shares are held by directors, executive officers and other affiliates and will be subject to certain limitations of Rule 144 under the Securities Act of 1933, as amended, or the Securities Act.

In addition, as of September 30, 2014, 3,946,574 shares of common stock that are either subject to outstanding options or reserved for future issuance under our equity incentive plans will become eligible for sale in the public market to the extent permitted by the provisions of various vesting schedules, the lock-up agreements and Rule 144 and Rule 701 under the Securities Act. If these additional shares of common stock are sold, or if it is perceived that they will be sold, in the public market, the market price of our common stock could decline.

Subsequent to our IPO, the holders of approximately 15,132,026 shares of our common stock are entitled to rights with respect to the registration of their shares under the Securities Act, subject to the lock-up agreements described above. Registration of these shares under the Securities Act would result in the shares becoming freely tradable without restriction under the Securities Act, except for shares purchased by affiliates. Any sales of securities by these stockholders could have a material adverse effect on the market our common stock.

We will have broad discretion in how we use the net proceeds from our IPO. We may not use these proceeds effectively, which could affect our results of operations and cause our stock price to decline.

We will have considerable discretion in the application of the net proceeds from our IPO. We intend to use the net proceeds from the IPO to advance clinical development of beloranib as a treatment for obesity and hyperphagia in patients with PWS and hypothalamic injury-associated obesity, to advance the clinical development of beloranib as a treatment for severe obesity in the general population, to continue the development of ZGN-839 and to fund new and ongoing research and development activities, working capital and other general corporate purposes, which may include funding for the hiring of additional personnel, capital expenditures and the costs of operating as a public company. We may use the net proceeds for purposes that do not yield a significant return or any return at all for our stockholders. In addition, we may invest the net proceeds from our IPO in a manner that does not produce income or that loses value.

We are an "emerging growth company" and will be able to avail ourselves of reduced disclosure requirements applicable to emerging growth companies, which could make our common stock less attractive to investors.

We are an "emerging growth company," as defined in the Jumpstart our Business Startups Act of 2012, or the JOBS Act, and we intend to take advantage of certain exemptions from various reporting requirements that are applicable to other public companies that are not "emerging growth companies" including not being required to comply with the auditor attestation requirements of Section 404(b) of the Sarbanes-Oxley Act, reduced disclosure obligations regarding executive compensation in our periodic reports and proxy statements, and exemptions from the requirements of holding a nonbinding advisory vote on executive compensation and shareholder approval of any golden parachute payments not previously approved. In addition, Section 107 of the JOBS Act also provides that an emerging growth company can take advantage of the extended transition period provided in Section 7(a)(2)(B) of the Securities Act, for complying with new or revised accounting standards. In other words, an emerging growth company can delay the adoption of certain accounting standards until those standards would otherwise apply to private companies. However, we are electing not to take advantage of such extended transition period, and as a result we will comply with new or revised accounting standards on the relevant dates on which adoption of such standards is required for non-emerging growth companies. Section 107 of the JOBS Act provides that our decision to not take advantage of the extended transition period for complying with new or revised accounting standards is irrevocable. We cannot predict if investors will find our common stock less attractive because we may rely on any of the exemptions available under the JOBS Act. If some investors find our common stock less attractive as a result, there may be a less active trading market for our common stock and our stock price may be more volatile. We may take advantage of these reporting exemptions until we are no longer an emerging growth company. We will remain an emerging growth company until the earliest of (i) the last day of the fiscal year in which we have total annual gross revenue of $1.0 billion or more; (ii) the last day of our fiscal year following the fifth anniversary of the date of the completion of our IPO; (iii) the date on which we have issued more than $1.0 billion in nonconvertible debt during the previous three years; and (iv) the date on which we are deemed to be a large accelerated filer under the rules of the SEC.

We have never paid dividends on our capital stock and we do not anticipate paying any dividends in the foreseeable future. Consequently, any gains from an investment in our common stock will likely depend on whether the price of our common stock increases.

We have not paid dividends on any of our classes of capital stock to date and we currently intend to retain all of our future earnings, if any, to fund the development and growth of our business. As a result, capital appreciation, if any, of our common stock will be your sole source of gain for the foreseeable future. Consequently, in the foreseeable future, you will likely only experience a gain from your investment in our common stock if the price of our common stock increases.

If equity research analysts do not publish research or reports about our business or if they issue unfavorable commentary or downgrade our common stock, the price of our common stock could decline.

The trading market for our common stock relies in part on the research and reports that equity research analysts publish about us and our business. We do not control these analysts. The price of our common stock could decline if one or more equity analysts downgrade our common stock or if analysts issue other unfavorable commentary or cease publishing reports about us or our business.

Anti-takeover provisions contained in our certificate of incorporation and bylaws, as well as provisions of Delaware law, could impair a takeover attempt.

Our certificate of incorporation, bylaws and Delaware law contain provisions which could have the effect of rendering more difficult, delaying or preventing an acquisition deemed undesirable by our board of directors. Our corporate governance documents include provisions:

- creating a classified board of directors whose members serve staggered three-year terms;
- authorizing "blank check" preferred stock, which could be issued by our board of directors without stockholder approval and may contain voting, liquidation, dividend, and other rights superior to our common stock;
- limiting the liability of, and providing indemnification to, our directors and officers;
- limiting the ability of our stockholders to call and bring business before special meetings;
- requiring advance notice of stockholder proposals for business to be conducted at meetings of our stockholders and for nominations of candidates for election to our board of directors;
- controlling the procedures for the conduct and scheduling of board of directors and stockholder meetings; and
- providing our board of directors with the express power to postpone previously scheduled annual meetings and to cancel previously scheduled special meetings.

These provisions, alone or together, could delay or prevent hostile takeovers and changes in control or changes in our management.

As a Delaware corporation, we are also subject to provisions of Delaware law, including Section 203 of the Delaware General Corporation law, which prevents some stockholders holding more than 15% of our outstanding common stock from engaging in certain business combinations without approval of the holders of substantially all of our outstanding common stock.

Any provision of our certificate of incorporation, bylaws or Delaware law that has the effect of delaying or deterring a change in control could limit the opportunity for our stockholders to receive a premium for their shares of our common stock, and could also affect the price that some investors are willing to pay for our common stock.

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

Unregistered Sales of Equity Securities

Upon the closing of our IPO, all of the outstanding shares of our redeemable convertible preferred stock were converted into 15,132,026 shares of common stock. The shares of common stock issued pursuant to such conversion were issued in reliance on the exemption from registration provided by Section 3(a)(9) of the Securities Act, which exemption is available for transactions involving securities exchanged by the issuer with its existing security holders exclusively where no commission or other remuneration is paid or given directly or indirectly for soliciting such exchange.

No underwriters were used in the foregoing transactions.

Use of Proceeds from IPO

On June 24, 2014, we closed the sale of 6,900,000 shares of common stock to the public (inclusive of 900,000 shares of common stock sold by us pursuant to the full exercise of an overallotment option granted to the underwriters) at a price of $16.00 per share, before underwriting discounts and commissions. The offer and sale of the shares in our IPO was registered under the Securities Act pursuant to registration statements on Form S-1 (File No. 333-195391), which was filed with the SEC on April 18, 2014 and amended subsequently and declared effective by the SEC on June 18, 2014, and Form S-1MEF (File No. 333-196891), which was filed with the SEC on June 18, 2014 and automatically effective upon filing. Following the sale of the shares in connection with the closing of our IPO, the offering terminated. The offering did not terminate before all the securities registered in the registration statements were sold. Leerink Partners LLC and Cowen and Company, LLC acted as joint book-running managers of the offering, and Canaccord Genuity Inc. and JMP Securities LLC acted as co-managers of the offering.

We raised approximately $102.7 million in net proceeds after deducting underwriting discounts and commissions of approximately $7.7 million. We also incurred other offering costs of approximately $2.5 million. None of these expenses consisted of direct or indirect payments made by us to directors, officers or persons owning 10% or more of our common stock or to their associates, or to our affiliates. There has been no material change in the planned use of proceeds from our IPO as described in our final prospectus filed with the SEC on June 19, 2014 pursuant to Rule 424(b)(4). We invested the funds received in cash equivalents and other short-term investments in accordance with our investment policy, and as of September 30, 2014, the entire amount of the net proceeds is included as cash and cash equivalents.

Item 6. Exhibits

The exhibits filed as part of this Quarterly Report on Form 10-Q are set forth on the Exhibit Index, which is incorporated herein by reference.

SIGNATURES

Pursuant to the requirements of the Securities Exchange Act of 1934, the registrant has duly caused this report to be signed on its behalf by the undersigned thereunto duly authorized.

ZAFGN, INC.

November 13, 2014 By: /s/ Thomas E. Hughes
Thomas E. Hughes
Chief Executive Officer
(Principal Executive Officer)

November 13, 2014 By: /s/ Patricia L. Allen
Patricia L. Allen
Chief Financial Officer
(Principal Financial Officer)

EXHIBIT INDEX

Exhibit No.	Description	Incorporated by Reference to: Form or Schedule	Exhibit No.	Filing Date with SEC	SEC File Number
31.1*	Certification of Principal Executive Officer pursuant to Exchange Act rules 13a-14 or 15d-14.				
31.2*	Certification of Principal Financial Officer pursuant to Exchange Act rules 13a-14 or 15d-14.				
32.1*	Certification of Principal Executive Officer and Principal Financial Officer pursuant to Exchange Act rules 13a-14(b) or 15d-14(b) and 18 U.S.C. Section 1350.				
101.INS**	XBRL Instance Document.				
101.SCH**	XBRL Taxonomy Extension Schema Document.				
101.CAL**	XBRL Taxonomy Extension Calculation Document.				
101.DEF**	XBRL Taxonomy Extension Definition Linkbase Document.				
101.LAB**	XBRL Taxonomy Extension Labels Linkbase Document.				
101.PRE**	XBRL Taxonomy Extension Presentation Link Document.				

* Filed herewith.

** Attached as Exhibit 101 to this report are documents formatted in XBRL (Extensible Business Reporting Language).

Exhibit 31.1

Certification

I, Thomas E. Hughes, certify that:

1. I have reviewed this quarterly report on Form 10-Q for the period ended September 30, 2014 of Zafgen, Inc.;

2. Based on my knowledge, this report does not contain any untrue statement of a material fact or omit to state a material fact necessary to make the statements made, in light of the circumstances under which such statements were made, not misleading with respect to the period covered by this report;

3. Based on my knowledge, the financial statements, and other financial information included in this report, fairly present in all material respects the financial condition, results of operations and cash flows of the registrant as of, and for, the periods presented in this report;

4. I am responsible for establishing and maintaining disclosure controls and procedures (as defined in Exchange Act Rules 13a-15(e) and 15d-15(e)) for the registrant and have:

   a. Designed such disclosure controls and procedures, or caused such disclosure controls and procedures to be designed under our supervision, to ensure that material information relating to the registrant, including its consolidated subsidiaries, is made known to us by others within those entities, particularly during the period in which this report is being prepared;

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

   c. Evaluated the effectiveness of the registrant's disclosure controls and procedures and presented in this report our conclusions about the effectiveness of the disclosure controls and procedures, as of the end of the period covered by this report based on such evaluation; and

   d. Disclosed in this report any change in the registrant's internal control over financial reporting that occurred during the registrant's most recent fiscal quarter (the registrant's fourth fiscal quarter in the case of an annual report) that has materially affected, or is reasonably likely to materially affect, the registrant's internal control over financial reporting; and

5. I have disclosed, based on my most recent evaluation of internal control over financial reporting, to the registrant's auditors and the audit committee of the registrant's board of directors (or persons performing the equivalent functions):

   a. All significant deficiencies and material weaknesses in the design or operation of internal control over financial reporting which are reasonably likely to adversely affect the registrant's ability to record, process, summarize and report financial information; and

   b. Any fraud, whether or not material, that involves management or other employees who have a significant role in the registrant's internal control over financial reporting.

Date: November 13, 2014

/s/ Thomas E. Hughes

Thomas E. Hughes
Chief Executive Officer
(Principal Executive Officer)

Exhibit 31.2

Certification

I, Patricia L. Allen, certify that:

1. I have reviewed this quarterly report on Form 10-Q for the period ended September 30, 2014 of Zafgen, Inc.;

2. Based on my knowledge, this report does not contain any untrue statement of a material fact or omit to state a material fact necessary to make the statements made, in light of the circumstances under which such statements were made, not misleading with respect to the period covered by this report;

3. Based on my knowledge, the financial statements, and other financial information included in this report, fairly present in all material respects the financial condition, results of operations and cash flows of the registrant as of, and for, the periods presented in this report;

4. I am responsible for establishing and maintaining disclosure controls and procedures (as defined in Exchange Act Rules 13a-15(e) and 15d-15(e)) for the registrant and have:

   a. Designed such disclosure controls and procedures, or caused such disclosure controls and procedures to be designed under our supervision, to ensure that material information relating to the registrant, including its consolidated subsidiaries, is made known to us by others within those entities, particularly during the period in which this report is being prepared;

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

   c. Evaluated the effectiveness of the registrant's disclosure controls and procedures and presented in this report our conclusions about the effectiveness of the disclosure controls and procedures, as of the end of the period covered by this report based on such evaluation; and

   d. Disclosed in this report any change in the registrant's internal control over financial reporting that occurred during the registrant's most recent fiscal quarter (the registrant's fourth fiscal quarter in the case of an annual report) that has materially affected, or is reasonably likely to materially affect, the registrant's internal control over financial reporting; and

5. I have disclosed, based on my most recent evaluation of internal control over financial reporting, to the registrant's auditors and the audit committee of the registrant's board of directors (or persons performing the equivalent functions):

   a. All significant deficiencies and material weaknesses in the design or operation of internal control over financial reporting which are reasonably likely to adversely affect the registrant's ability to record, process, summarize and report financial information; and

   b. Any fraud, whether or not material, that involves management or other employees who have a significant role in the registrant's internal control over financial reporting.

Date: November 13, 2014

/s/ Patricia L. Allen

Patricia L. Allen
Chief Financial Officer
(Principal Financial Officer)

Exhibit 32.1

CERTIFICATION PURSUANT TO
18 U.S.C. SECTION 1350,
AS ADOPTED PURSUANT TO
SECTION 906 OF THE SARBANES-OXLEY ACT OF 2002

In connection with the quarterly report on Form 10-Q of Zafgen, Inc. (the "Company") for the period ended September 30, 2014, as filed with the Securities and Exchange Commission on the date hereof (the "Report"), each of the undersigned officers hereby certify, pursuant to Section 906 of the Sarbanes-Oxley Act of 2002, 18 U.S.C. Section 1350, that to his or her knowledge:

1) the Report which this statement accompanies fully complies with the requirements of Section 13(a) or 15(d) of the Securities Exchange Act of 1934, as amended; and

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

Date: November 13, 2014

/s/ Thomas E. Hughes
Thomas E. Hughes
Chief Executive Officer
(Principal Executive Officer)

Date: November 13, 2014

/s/ Patricia L. Allen
Patricia L. Allen
Chief Financial Officer
(Principal Financial Officer)