

SANGAMO BIOSCIENCES INC  
Form 10-Q  
October 30, 2015

UNITED STATES

SECURITIES AND EXCHANGE COMMISSION

Washington, D.C. 20549

FORM 10-Q

(Mark One)

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

For the quarterly period ended September 30, 2015

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 000-30171

SANGAMO BIOSCIENCES, INC.

(exact name of registrant as specified in its charter)

Delaware 68-0359556  
(State or other jurisdiction of (IRS Employer  
incorporation or organization) Identification No.)

501 Canal Blvd

Richmond, California 94804

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(Address of principal executive offices)

(510) 970-6000

(Registrant's telephone number, including area code)

Indicate by check mark whether the registrant (1) has filed all reports required to be filed by section 13 or 15(d) of the Securities 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 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 October 26, 2015, 70,046,841 shares of the issuer's common stock, par value \$0.01 per share, were outstanding.

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SANGAMO BIOSCIENCES, INC.

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CERTIFICATIONS

SPECIAL NOTE REGARDING FORWARD-LOOKING STATEMENTS

Some statements contained in this report are forward-looking with respect to our operations, research, development and commercialization activities, clinical trials, operating results and financial condition. These statements involve known and unknown risks, uncertainties and other factors which may cause our actual results, performance or achievements to be materially different from any future results, performances or achievements expressed or implied by the forward-looking statements. Forward-looking statements include, but are not limited to, statements about:

- our strategy;
- product development and commercialization of our products;
- clinical trials;
- partnering;
- revenues from existing and new collaborations;
- our research and development and other expenses;
- sufficiency of our cash resources;
- our operational and legal risks; and
- our plans, objectives, expectations and intentions and any other statements that are not historical facts.

In some cases, you can identify forward-looking statements by terms such as: “anticipates,” “believes,” “continues,” “could,” “estimates,” “expects,” “intends,” “may,” “plans,” “seeks,” “should” and “will.” These statements reflect our current views with respect to future events and are based on assumptions and subject to risks and uncertainties. Many of these risks are discussed

in greater detail under the headings “Risk Factors” and “Management’s Discussion and Analysis of Financial Conditions and Results of Operations” in this Form 10-Q. Sangamo undertakes no obligation to publicly release any revisions to forward-looking statements to reflect events or circumstances arising after the date of this report. Readers are cautioned not to place undue reliance on the forward-looking statements, which speak only as of the date of this Quarterly Report on Form 10-Q.

ZFP Therapeutic® is a registered trademark of Sangamo BioSciences, Inc.

## PART I. FINANCIAL INFORMATION

ITEM 1. FINANCIAL STATEMENTS  
SANGAMO BIOSCIENCES, INC.

## CONDENSED CONSOLIDATED BALANCE SHEETS

(Unaudited; in thousands, except share and per share amounts)

	September 30, 2015	December 31, 2014
<b>ASSETS</b>		
Current assets:		
Cash and cash equivalents	\$34,287	\$6,030
Marketable securities	185,042	172,932
Interest receivable	399	423
Accounts receivable	7,004	10,368
Prepaid expenses	951	623
Restricted cash	—	320
Other current assets	—	183
Total current assets	227,683	190,879
Marketable securities, non-current	—	47,260
Property and equipment, net	3,063	1,479
Intangible assets, in-process research and development	—	1,870
Goodwill	1,585	1,585
Other assets	185	139
Total assets	\$232,516	\$243,212
<b>LIABILITIES AND STOCKHOLDERS' EQUITY</b>		
Current liabilities:		
Accounts payable and accrued liabilities	\$8,888	\$8,704
Accrued compensation and employee benefits	2,779	2,853
Escrow liability	—	275
Deferred revenues	10,461	9,050
Total current liabilities	22,128	20,882
Deferred revenues, non-current	6,830	13,149
Contingent consideration liability	—	1,800
Deferred tax liability	—	748
Total liabilities	28,958	36,579
Commitments and contingencies		
Stockholders' equity:		
Common stock, \$0.01 par value; 160,000,000 shares authorized, 70,042,758 and	700	690

69,062,394 shares issued and outstanding at September 30, 2015, and

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December 31, 2014, respectively

Additional paid-in capital	558,044	534,518
Accumulated deficit	(355,240)	(328,550)
Accumulated other comprehensive income (loss)	54	(25 )
Total stockholders' equity	203,558	206,633
Total liabilities and stockholders' equity	\$232,516	\$243,212

See accompanying notes.

SANGAMO BIOSCIENCES, INC.

## CONDENSED CONSOLIDATED STATEMENTS OF OPERATIONS

(Unaudited; in thousands, except per share amounts)

	Three months ended September 30,		Nine months ended September 30,	
	2015	2014	2015	2014
<b>Revenues:</b>				
Collaboration agreements	\$8,406	\$12,045	\$28,878	\$29,334
Research grants	163	372	1,540	1,584
Total revenues	8,569	12,417	30,418	30,918
<b>Operating expenses:</b>				
Research and development	16,694	16,340	47,292	41,883
General and administrative	4,560	3,731	14,309	11,347
Total operating expenses	21,254	20,071	61,601	53,230
Loss from operations	(12,685)	(7,654)	(31,183)	(22,312)
Interest and other income, net	101	109	406	214
Loss before income taxes	(12,584)	(7,545)	(30,777)	(22,098)
Benefit from income taxes	3,339	—	4,087	—
Net loss	\$(9,245)	\$(7,545)	\$(26,690)	\$(22,098)
Basic and diluted net loss per share	\$(0.13)	\$(0.11)	\$(0.38)	\$(0.33)
Shares used in computing basic and diluted net loss per share	69,892	68,230	69,622	66,488

See accompanying notes.

SANGAMO BIOSCIENCES, INC.

## CONDENSED CONSOLIDATED STATEMENTS OF COMPREHENSIVE LOSS

(Unaudited; in thousands)

	Three months ended		Nine months ended	
	September 30, 2015	2014	September 30, 2015	2014
Net loss	\$(9,245)	\$(7,545)	\$(26,690)	\$(22,098)
Change in unrealized gain on available-for-sale securities	57	20	79	41
Comprehensive loss	\$(9,188)	\$(7,525)	\$(26,611)	\$(22,057)

See accompanying notes.



SANGAMO BIOSCIENCES, INC.

## CONDENSED CONSOLIDATED STATEMENTS OF CASH FLOWS

(Unaudited: in thousands)

	Nine months ended September 30,	
	2015	2014
<b>Operating Activities:</b>		
Net loss	\$(26,690 )	\$(22,098 )
<b>Adjustments to reconcile net loss to net cash used in operating activities:</b>		
Depreciation and amortization	745	400
Amortization of premium on marketable securities	681	843
Stock-based compensation	8,664	6,165
Change in fair value of contingent consideration liability	(1,800 )	130
Intangible impairment	1,870	—
Benefit from income taxes	(4,087 )	—
<b>Net changes in operating assets and liabilities:</b>		
Interest receivable	24	25
Accounts receivable	3,364	(5,319 )
Prepaid expenses and other assets	129	(1,059 )
Accounts payable and accrued liabilities	(2,534 )	3,619
Accrued compensation and employee benefits	(74 )	(530 )
Deferred revenues	(4,908 )	14,861
Net cash used in operating activities	(24,616 )	(2,963 )
<b>Investing Activities:</b>		
Purchases of marketable securities	(168,627)	(176,385)
Maturities of marketable securities	203,175	76,605
Purchases of property and equipment	(2,327 )	(559 )
Net cash provided by / (used in) investing activities	32,221	(100,339)
<b>Financing Activities:</b>		
Proceeds from public offering of common stock, net of issuance costs	—	93,796
Taxes paid related to net share settlement of equity awards	(48 )	—
Proceeds from issuance of common stock	6,248	10,525
Claims settlement under Section 16(b)	14,452	—
Net cash provided by financing activities	20,652	104,321
Net increase in cash and cash equivalents	28,257	1,019
Cash and cash equivalents, beginning of period	6,030	10,186
Cash and cash equivalents, end of period	\$34,287	\$11,205

See accompanying notes.



SANGAMO BIOSCIENCES, INC.

NOTES TO CONDENSED CONSOLIDATED FINANCIAL STATEMENTS

September 30, 2015

(Unaudited)

NOTE 1—BASIS OF PRESENTATION AND SUMMARY OF SIGNIFICANT ACCOUNTING POLICIES

Basis of Presentation

The accompanying unaudited condensed consolidated financial statements of Sangamo BioSciences, Inc. (“Sangamo” or the “Company”) have been prepared in accordance with U.S. generally accepted accounting principles for interim financial information and pursuant to the rules and regulations of the Securities and Exchange Commission (SEC). Accordingly, they do not include all of the information and footnotes required by generally accepted accounting principles for complete financial statements. In the opinion of management, all adjustments (consisting of normal recurring adjustments) considered necessary for a fair presentation have been included. Operating results for the three and nine months ended September 30, 2015 are not necessarily indicative of the results that may be expected for the year ending December 31, 2015. The condensed consolidated balance sheet data at December 31, 2014 were derived from the audited consolidated financial statements included in Sangamo’s Form 10-K for the year ended December 31, 2014, as filed with the SEC. The accompanying condensed consolidated financial statements and related financial information should be read in conjunction with the audited financial statements and footnotes thereto for the year ended December 31, 2014, included in Sangamo’s Form 10-K, as filed with the SEC.

Use of Estimates

The preparation of financial statements in conformity with generally accepted accounting principles requires management to make estimates and assumptions that affect the amounts reported in the financial statements and the accompanying notes. On an ongoing basis, management evaluates its estimates, including critical accounting policies or estimates related to revenue recognition, clinical trial accruals, fair value measurements, business combinations including the fair value of the contingent consideration liability for payments to former Ceregene, Inc. (Ceregene) stockholders and intangible assets related to the acquisition of Ceregene, and stock-based compensation. Estimates are based on historical experience and on various other market specific and other relevant assumptions that the Company believes to be reasonable under the circumstances, the results of which form the basis for making judgments about the carrying values of assets and liabilities that are not readily apparent from other sources. Actual results could differ from those estimates.

Revenue Recognition

Revenues from research activities made under strategic partnering agreements and collaborations are recognized as the services are provided when there is persuasive evidence that an arrangement exists, delivery has occurred, the price is fixed or determinable, and collectability is reasonably assured. Revenue generated from research and licensing agreements typically includes upfront signing or license fees, cost reimbursements, research services, minimum sublicense fees, milestone payments and royalties on future licensee’s product sales.

Multiple Element Arrangements prior to the adoption of ASU No. 2009-13, Revenue Recognition—Multiple Deliverable Revenue Arrangements (ASU 2009-13). For revenue arrangements entered into before January 1, 2011, that include multiple deliverables, the elements of such agreements were divided into separate units of accounting if the

deliverables met certain criteria, including whether the fair value of the delivered items could be determined and whether there was evidence of fair value of the undelivered items. In addition, the consideration was allocated among the separate units of accounting based on their fair values, and the applicable revenue recognition criteria are considered separately for each of the separate units of accounting. Prior to the adoption of ASU 2009-13, the Company recognized nonrefundable signing, license or non-exclusive option fees as revenue when rights to use the intellectual property related to the license were delivered and over the period of performance obligations if the Company had continuing performance obligations. The Company estimated the performance period at the inception of the arrangement and reevaluated it each reporting period. Changes to these estimates were recorded on a prospective basis.

Multiple Element Arrangements after the adoption of ASU 2009-13. ASU 2009-13 amended the accounting standards for certain multiple element revenue arrangements to:

- provide updated guidance on whether multiple elements exist, how the elements in an arrangement should be separated, and how the arrangement consideration should be allocated to the separate elements;
- require an entity to allocate arrangement consideration to each element based on a selling price hierarchy where the selling price for an element is based on vendor-specific objective evidence (VSOE), if available; third-party evidence (TPE), if available and VSOE is not available; or the best estimate of selling price (ESP), if neither VSOE nor TPE is available; and
- eliminate the use of the residual method and require an entity to allocate arrangement consideration using the relative selling price method.

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For revenue agreements with multiple element arrangements, such as license and development agreements, entered into on or after January 1, 2011, the Company allocates revenue to each non-contingent element based on the relative selling price of each element. When applying the relative selling price method, the Company determines the selling price for each deliverable using VSOE of selling price or TPE of selling price. If neither exists, the Company uses ESP for that deliverable. Revenue allocated is then recognized when the basic four revenue recognition criteria are met for each element. The collaboration and license agreements entered into with Shire International GmbH, formerly Shire AG (Shire), in January 2012 and Biogen Inc., formerly Biogen Idec Inc. (Biogen) in January 2014 were evaluated under these amended accounting standards.

Additionally, the Company may be entitled to receive certain milestone payments which are contingent upon reaching specified objectives. These milestone payments are recognized as revenue in full upon achievement of the milestone if there is substantive uncertainty at the date the arrangement is entered into that the objectives will be achieved and if the achievement is based on the Company's performance.

Minimum annual sublicense fees are also recognized as revenue in the period in which such fees are due. Royalty revenues are generally recognized when earned and collectability of the related royalty payment is reasonably assured. The Company recognizes cost reimbursement revenue under collaborative agreements as the related research and development costs for services are rendered. Deferred revenue represents the portion of research or license payments received which have not been earned.

Sangamo's research grants are typically multi-year agreements and provide for the reimbursement of qualified expenses for research and development as defined under the terms of the grant agreement. Revenue under grant agreements is recognized when the related qualified research expenses are incurred to the extent such amounts have been agreed to with the respective collaboration partner.

#### Recent Accounting Standards

In May 2014 the Financial Accounting Standards Board issued ASU 2014-09, Revenue from Contracts with Customers (ASU 2014-09). This standard outlines a single comprehensive model for entities to use in accounting for revenue arising from contracts with customers and supersedes most current revenue recognition guidance, including industry-specific guidance. The main principle of ASU 2014-09 is to recognize revenues when promised goods or services are transferred to customers in an amount that reflects the consideration that is expected to be received for those goods or services. ASU 2014-09 provides companies with two implementation methods: (i) apply the standard retrospectively to each prior reporting period presented (full retrospective application); or (ii) apply the standard retrospectively with the cumulative effect of initially applying the standard as an adjustment to the opening balance of retained earnings of the annual reporting period that includes the date of initial application (modified retrospective application). This guidance is effective for annual reporting periods beginning after December 15, 2017, including interim periods within that reporting period. The Company is currently in the process of evaluating the impact of the pending adoption of ASU 2014-09 on its consolidated financial statements.

#### NOTE 2—FAIR VALUE MEASUREMENT

The Company measures certain financial assets and liabilities at fair value on a recurring basis, including cash equivalents, available-for-sale-securities and the contingent consideration liability. The fair value of these assets and contingent liability was determined based on a three-tier hierarchy under the authoritative guidance for fair value measurements and disclosures that prioritizes the inputs used in measuring fair value as follows:

Level 1: Unadjusted quoted prices in active markets that are accessible at the measurement date for identical, unrestricted assets or liabilities;

Level 2: Quoted prices in markets that are not active or inputs which are observable, either directly or indirectly, for substantially the full term of the asset or liability; and

Level 3: Prices or valuation techniques that require inputs that are both significant to the fair value measurement and unobservable (i.e., supported by little or no market activity).

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The fair value measurements of the Company's cash equivalents, available-for-sale marketable securities and contingent consideration liability are identified at the following levels within the fair value hierarchy (in thousands):

	September 30, 2015			
	Fair Value Measurements			
	Total	Level 1	Level 2	Level 3
<b>Assets:</b>				
Cash equivalents:				
Money market funds	\$12,747	\$12,747	\$—	\$—
Total	12,747	12,747	—	—
Marketable securities:				
Commercial paper securities	15,399	—	15,399	—
Corporate debt securities	29,217	—	29,217	—
U.S. government sponsored entity debt securities	140,426	—	140,426	—
Total	185,042	—	185,042	—
Total cash equivalents and marketable securities	\$197,789	\$12,747	\$185,042	\$—
	December 31, 2014			
	Fair Value Measurements			
	Total	Level 1	Level 2	Level 3
<b>Assets:</b>				
Cash equivalents:				
Money market funds	\$3,182	\$3,182	\$—	\$—
Total	3,182	3,182	—	—
Marketable securities:				
Commercial paper securities	33,748	—	33,748	—
Corporate debt securities	22,813	—	22,813	—
U.S. government sponsored entity debt securities	163,631	—	163,631	—
Total	220,192	—	220,192	—
Total cash equivalents and marketable securities	\$223,374	\$3,182	\$220,192	\$—
<b>Liabilities:</b>				
Contingent consideration liability	\$1,800	\$—	\$—	\$1,800
Total	\$1,800	\$—	\$—	\$1,800

## Investments

The Company generally classifies its marketable securities as Level 2. Instruments can be classified as Level 2 when observable market prices for identical securities that are traded in less active markets are used. When observable market prices for identical securities are not available, such instruments are priced using benchmark curves, benchmarking of like securities, sector groupings, matrix pricing and valuation models. These valuation models are proprietary to the pricing providers or brokers and incorporate a number of inputs, including, listed in approximate order of priority: benchmark yields, reported trades, broker/dealer quotes, issuer spreads, two-sided markets, benchmark securities, bids, offers and reference data including market research publications. For certain security types, additional inputs may be used, or some of the standard inputs may not be applicable. Evaluators may prioritize inputs differently on any given day for any security based on market conditions, and not all inputs listed are available

for use in the evaluation process for each security evaluation on any given day.

#### Contingent Consideration Liability

In October 2013, the Company acquired Ceregene and recorded a liability for the estimated fair value of contingent consideration payments to former Ceregene stockholders, as outlined under the terms of the merger agreement with Ceregene. These contingent payments are owed if the Company grants a third-party license to develop and commercialize certain product candidates acquired from Ceregene, or if the Company commercializes any of such product candidates itself. The fair value of this liability is estimated using a probability-weighted discounted cash flow analysis. Such valuations require significant estimates and assumptions including but not limited to: determining the timing and estimated costs to complete the in-process projects, projecting regulatory approvals, estimating future cash flows and developing appropriate discount rates. The Company has classified this liability as Level 3.



Subsequent changes in the fair value of this contingent consideration liability are recorded to the research and development (R&D) expense line item in the Condensed Consolidated Statements of Operations as operating expenses. During the nine months ended September 30, 2015, the recognized amount of the liability for contingent consideration decreased by \$1.8 million due to the decrease in the probability of incurring potential future royalty payments associated with the impairment of the in-process research and development (IPR&D) assets acquired from Ceregene (see Note 6).

Fair value as of December 31, 2014	\$ 1,800
Change in fair value	(1,800)
Fair value as of September 30, 2015	\$—

### NOTE 3—MARKETABLE SECURITIES

Sangamo generally classifies its marketable securities as available-for-sale and records its investments at fair value. Available-for-sale securities are carried at estimated fair value, with the unrealized holding gains and losses included in accumulated other comprehensive income (loss). Investments that have maturities beyond one year as of the end of the reporting period are classified as non-current. The Company's investments are subject to a periodic impairment review, and the Company recognizes an impairment charge when a decline in the fair value of its investments below the cost basis is judged to be other-than-temporary. The Company considers various factors in determining whether to recognize an impairment charge, including the length of time and extent to which the fair value has been less than the Company's cost basis, the financial condition and near-term prospects of the investee, and the Company's intent and ability to hold the investment for a period of time sufficient to allow for any anticipated recovery in the market value.

The table below summarizes the Company's investments (in thousands):

	Amortized Cost	Gross Unrealized Gains	Gross Unrealized (Losses)	Estimated Fair Value
<b>September 30, 2015</b>				
Cash equivalents:				
Money market funds	\$ 12,747	\$ —	\$ —	\$ 12,747
Total	12,747	—	—	12,747
Available-for-sale securities:				
Commercial paper securities	\$ 15,371	\$ 28	\$ —	\$ 15,399
Corporate debt securities	29,215	2	—	29,217
U.S. government sponsored entity debt securities	140,402	24	—	140,426
Total	184,988	54	—	185,042
Total cash equivalents and available-for-sale securities	\$ 197,735	\$ 54	\$ —	\$ 197,789
<b>December 31, 2014</b>				
Cash equivalents:				
Money market funds	\$ 3,182	\$ —	\$ —	\$ 3,182
Total	3,182	—	—	3,182
Available-for-sale securities:				

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Commercial paper securities	\$ 33,715	\$ 33	\$ —	\$ 33,748
Corporate debt securities	22,831	—	(18 )	22,813
U.S. government sponsored entity debt securities	163,671	—	(40 )	163,631
<b>Total</b>	<b>220,217</b>	<b>33</b>	<b>(58 )</b>	<b>220,192</b>
Total cash equivalents and available-for-sale securities	\$ 223,399	\$ 33	\$ (58 )	\$ 223,374

The Company had no other-than-temporary impairments of its investments for the nine months ended September 30, 2015 or the twelve months ended December 31, 2014.

NOTE 4—BASIC AND DILUTED NET LOSS PER SHARE

Basic net loss per share has been computed by dividing the net loss by the weighted-average number of shares of common stock outstanding during the period. Diluted net loss per share is calculated by dividing net loss by the weighted-average number of shares of common stock and potential dilutive securities outstanding during the period.

Because Sangamo is in a net loss position, diluted net loss per share excludes the effects of common stock equivalents consisting of stock options and unvested restricted stock units, which are anti-dilutive. The total number of shares subject to stock

options outstanding excluded from consideration in the calculation of diluted net loss per share for the three and nine months ended September 30, 2015 and 2014 were 7,868,033 and 8,263,998, respectively.

#### NOTE 5—MAJOR CUSTOMERS, PARTNERSHIPS AND STRATEGIC ALLIANCES

##### Collaboration Agreements

##### Collaboration and License Agreement with Biogen Inc. in Human Therapeutics

In January 2014 the Company entered into a Global Research, Development and Commercialization Collaboration and License Agreement (the “Biogen Agreement”) with Biogen, pursuant to which Sangamo and Biogen collaborate to discover, develop, seek regulatory approval for and commercialize therapeutics based on Sangamo’s zinc finger DNA-binding protein (ZFP) technology for hemoglobinopathies, including beta-thalassemia and sickle cell disease (SCD).

Under the Biogen Agreement, Sangamo and Biogen jointly conduct two research programs: the beta-thalassemia program and the SCD program. For the beta-thalassemia program, Sangamo is responsible for all discovery, research and development activities through the first human clinical trial for the first ZFP Therapeutic developed under the Biogen Agreement for the treatment of beta-thalassemia. For the SCD program, both parties are responsible for research and development activities through the submission of an Investigational New Drug (IND) application for ZFP Therapeutics intended to treat SCD. For both programs, Biogen is responsible for subsequent world-wide clinical development, manufacturing and commercialization of licensed products developed under the Biogen Agreement. At the end of specified research terms for each program or under certain specified circumstances, Biogen retains the right to step in and take over any remaining activities of Sangamo. Furthermore, Sangamo has an option to co-promote in the United States any licensed product to treat beta-thalassemia and SCD developed under the Biogen Agreement, and Biogen agrees to compensate Sangamo for such co-promotion activities.

Sangamo received an upfront license fee of \$20.0 million upon entering into the Biogen Agreement. In addition, the Company will also be eligible to receive \$126.3 million in payments upon the achievement of specified research, regulatory, clinical development milestones, as well as \$167.5 million in payments upon the achievement of specified commercialization and sales milestones. Biogen reimburses Sangamo for agreed upon costs incurred in connection with research and development activities conducted by Sangamo. In addition, Sangamo is eligible to receive contingent payments upon the achievement of specified regulatory, clinical development, commercialization and sales milestones. The total amount of potential regulatory, clinical development, commercialization and sales contingent payments, assuming the achievement of all specified milestone events in the Biogen Agreement, is \$293.8 million, including Phase 1 contingent payments of \$7.5 million for each of the beta-thalassemia and SCD programs. In addition, if products are commercialized under the Biogen Agreement, Biogen will pay Sangamo incremental royalties for each licensed product that are a tiered double-digit percentage of annual net sales of such product. To date, no milestone payments have been received and no products have been approved and therefore no royalty fees have been earned under the Biogen Agreement.

All contingent payments under the Biogen Agreement, when earned, will be non-refundable and non-creditable. The Company has evaluated the contingent payments under the Biogen Agreement based on the authoritative guidance for research and development milestones and determined that certain of these payments meet the definition of a milestone and that all such milestones are evaluated to determine if they are considered substantive milestones. Milestones are considered substantive if they are related to events (i) that can be achieved based in whole or in part on either the Company’s performance or on the occurrence of a specific outcome resulting from the Company’s performance, (ii) for which there was substantive uncertainty at the date the agreement was entered into that the event would be achieved and (iii) that would result in additional payments being due to the Company. Accordingly, consideration received for

the achievement of milestones that are determined to be substantive will be recognized as revenue in their entirety in the period when the milestones are achieved and collectability is reasonably assured. Revenue for the achievement of milestones that are not substantive will be recognized over the remaining period of the Biogen Agreement, assuming all other applicable revenue recognition criteria have been met.

Subject to the terms of the Biogen Agreement, Sangamo grants Biogen an exclusive, royalty-bearing license, with the right to grant sublicenses, to use certain ZFP and other technology controlled by Sangamo for the purpose of researching, developing, manufacturing and commercializing licensed products developed under the Biogen Agreement. Sangamo also grants Biogen a non-exclusive, world-wide, royalty free, fully paid license, with the right to grant sublicenses, of Sangamo's interest in certain other intellectual property developed pursuant to the Biogen Agreement.

The Company has identified the deliverables within the arrangement as a license to the technology and on-going research services activities. The Company has concluded that the license is not a separate unit of accounting as it does not have stand-alone value to Biogen apart from the research services to be performed pursuant to the Biogen Agreement. As a result, the Company will recognize revenue from the upfront payment on a straight-line basis over a forty-month estimated initial research term during which the Company will perform research services. As of September 30, 2015, the Company has deferred revenue of \$10.2 million related to the Biogen Agreement.

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Revenues recognized under the agreement with Biogen for the three and nine months ended September 30, 2015 and 2014 are as follows (in thousands):

	Three months ended September 30, 2015		Nine months ended September 30, 2014	
Revenue related to Biogen Collaboration:				
Recognition of upfront fee	\$ 1,556	\$ 1,557	\$ 4,619	\$ 3,756
Research services	2,594	3,508	5,827	5,270
Total	\$ 4,150	\$ 5,065	\$ 10,446	\$ 9,026

Related costs and expenses incurred under the Biogen agreement related to the beta-thalassemia project, which was co-funded with California Institute for Regenerative Medicine (CIRM), were \$2.3 million and \$2.0 million during the three months ended September 30, 2015 and, 2014, respectively and \$4.7 million and \$4.5 million during the nine months ended September 30, 2015 and, 2014, respectively. Related costs and expenses for other projects including sickle cell disease under the Biogen agreement were \$0.7 million and \$0.5 million during the three months ended September 30, 2015 and, 2014, respectively and \$2.7 million and \$0.8 million during the nine months ended September 30, 2015 and 2014, respectively.

#### Collaboration and License Agreement with Shire International GmbH in Human Therapeutics and Diagnostics

In January 2012 the Company entered into a collaboration and license agreement (the “Shire Agreement”) with Shire, pursuant to which the Company and Shire collaborate to research, develop and commercialize human therapeutics and diagnostics for monogenic diseases based on Sangamo’s novel ZFP technology. This agreement was amended on September 1, 2015.

Under the original Shire Agreement, the Company and Shire agreed to develop potential human therapeutic or diagnostic products for seven gene targets. The initial four gene targets selected were blood clotting Factors VII, VIII, IX and X, and products developed for such initial gene targets will be used for treating or diagnosing hemophilia A and B. In June 2012, Shire selected a fifth gene target for the development of a ZFP Therapeutic for Huntington’s disease. Shire had the right, subject to certain limitations, to designate two additional gene targets. Pursuant to the Shire Agreement, the Company granted Shire an exclusive, world-wide, royalty-bearing license, with the right to grant sublicenses, to use Sangamo’s ZFP technology for the purpose of developing and commercializing human therapeutic and diagnostic products for the gene targets.

Under the terms of the Shire Agreement, the Company was responsible for all research activities through the submission of an IND or European Clinical Trial Application (CTA), while Shire was responsible for clinical development and commercialization of products generated from the research program from and after the acceptance of an IND or CTA for the product. Shire reimbursed Sangamo for agreed upon internal and external program-related research costs. The Company received an upfront license fee of \$13.0 million upon entering into the Shire Agreement in 2012. In 2014 Sangamo recognized a \$1.0 million milestone payment related to the hemophilia program.

On September 1, 2015, the Shire Agreement was amended such that Shire agreed to return to Sangamo the exclusive, world-wide rights to gene targets for the development and commercialization of ZFP Therapeutics for hemophilia A and B. Shire retains the rights and will continue to develop a ZFP Therapeutic for Huntington’s disease and a ZFP Therapeutic for one additional gene target yet to be named. Sangamo will provide certain target feasibility services, and upon Shire’s request, certain research activities according to a research plan as agreed upon by both companies.

Such research activities performed by Sangamo will be reimbursed by Shire. Shire's rights with respect to other targets contemplated in the original agreement revert to Sangamo. Under the revised agreement, each company is responsible for expenses associated with its own programs and will reimburse the other for any ongoing services provided. Shire is responsible for reimbursement of \$4.0 million related to obligations prior to the amendment date which will be recognized in revenue as expenses are incurred. During the 3 months ended September 30, 2015, \$2.3 million was incurred and recognized related to prior obligations. Sangamo has granted Shire a right of first negotiation to license the hemophilia A and B programs. Under the amended agreement, Shire does not have any milestone payment obligations to us with respect to the retained programs, but it is required to pay single digit percentage royalties to us, up to a specified maximum cap, on the commercial sales of ZFP therapeutic products from such programs. Under the Agreement, Sangamo has full control over, and full responsibility for the costs of, the hemophilia programs returned to us, subject to certain diligence obligations and Shire's right of first negotiation to obtain a license to such programs under certain circumstances. The Company is required to pay single digit percentage royalties to Shire, up to a specified maximum cap, on commercial sales of ZFP therapeutic products from such returned programs.

The Company has identified the deliverables within the amended arrangement as a license to the technology and on-going research services activities. The Company has concluded that the license is not a separate unit of accounting as it does not have stand-alone value to Shire apart from the research services to be performed pursuant to the Shire amendment. Sangamo continues to be responsible for research activities related to our licensed technology with Shire under the amendment. As a result, the Company will continue to recognize revenue from the upfront payment received upon entering into the original Shire agreement in 2012 on a

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straight-line basis over the six-year initial research term during which the Company expects to perform research services. As of September 30, 2015, the Company has deferred revenue of \$7.1 million related to the Shire Agreement.

Revenues recognized under the agreement with Shire for the three and nine months ended September 30, 2015 and 2014, were as follows (in thousands):

	Three months ended		Nine months ended	
	September 30,		September 30,	
	2015	2014	2015	2014
<b>Revenue related to Shire Collaboration:</b>				
Recognition of upfront fee	\$542	\$542	\$1,625	\$1,625
Recognition of milestone	—	1,000	—	1,000
Research services	3,313	5,002	11,729	15,912
<b>Total</b>	<b>\$3,855</b>	<b>\$6,544</b>	<b>\$13,354</b>	<b>\$18,537</b>

Related costs and expenses incurred under the Shire agreement were \$3.2 million and \$5.1 million during the three months ended September 30, 2015 and 2014, respectively and \$11.9 million and \$15.5 million during the nine months ended September 30, 2015 and 2014, respectively.

**Agreement with Sigma-Aldrich Corporation in Laboratory Research Reagents, Transgenic Animal and Commercial Protein Production Cell-line Engineering**

In July 2007 the Company entered into a license agreement (the “Sigma Agreement”) with Sigma-Aldrich Corporation (Sigma). Under the Sigma Agreement, Sangamo agreed to provide Sigma with access to Sangamo’s proprietary ZFP technology and the exclusive right to use the technology to develop and commercialize research reagent products and services in the research field, excluding certain agricultural research uses that Sangamo previously licensed to Dow AgroSciences LLC (DAS). Under the Sigma Agreement, Sangamo and Sigma agreed to conduct a three-year research program to develop laboratory research reagents using Sangamo’s ZFP technology during which time Sangamo agreed to assist Sigma in connection with its efforts to market and sell services employing the Company’s ZFP technology in the research field. Sangamo has transferred its ZFP manufacturing technology to Sigma.

In October 2009 the Company expanded the Sigma Agreement. In addition to the original terms of the Sigma Agreement, Sigma received exclusive rights to develop and distribute ZFP-modified cell lines for commercial production of protein pharmaceuticals and certain ZFP-engineered transgenic animals for commercial applications. Under the terms of the Sigma Agreement as expanded in 2009, Sigma made an upfront cash payment of \$20.0 million consisting of a \$4.9 million purchase of 636,133 shares of Sangamo common stock and a \$15.1 million upfront license fee. The upfront license fee was recognized on a straight-line basis from the effective date of the expanded license through July 2010, which represents the period over which Sangamo was obligated to perform research services for Sigma. Sangamo is also eligible to receive commercial license fees of \$5.0 million based upon a percentage of net sales. As of September 30, 2015 Sangamo has received the entire \$5.0 million of commercial license fees and is eligible to receive royalty payments of 5.25% of net sales and sublicensing revenue. In addition, upon the achievement of certain cumulative commercial milestones Sigma will make milestone payments to Sangamo up to an aggregate of \$25.0 million.

Revenues recognized under the agreement with Sigma for the three and nine months ended September 30, 2015 and 2014, were as follows (in thousands):

	Three months ended September 30, 2015		2014		Nine months ended September 30, 2015		2014	
<b>Revenue related to Sigma Collaboration:</b>								
Royalty revenues	\$97	\$67	\$390	\$270				
License fee revenues	172	269	4,449	447				
Total	\$269	\$336	\$4,839	\$717				

Related costs and expenses incurred under the Sigma agreement were both \$0.0 million during the three months ended September 30, 2015 and 2014, respectively. Related costs and expenses incurred under the Sigma agreement were \$0.3 million and \$0.1 million during the nine months ended September 30, 2015 and 2014, respectively.

#### Agreement with Dow AgroSciences in Plant Agriculture

In October 2005 the Company entered into an exclusive commercial license agreement with DAS (the "DAS Agreement"). Under the DAS Agreement, Sangamo provides DAS with access to Sangamo's proprietary ZFP technology and the exclusive right to



use the technology to modify the genomes or alter the nucleic acid or protein expression of plant cells, plants, or plant cell cultures. Sangamo has retained rights to use plants or plant-derived products to deliver ZFP transcription factors (ZFP TFs) or ZFP nucleases (ZFNs) into humans or animals for diagnostic, therapeutic or prophylactic purposes. The DAS Agreement provided for an initial three-year research term. In June 2008, DAS exercised its option under the agreement to obtain a commercial license to sell products incorporating or derived from plant cells generated using the Company's ZFP technology, including agricultural crops, industrial products and plant-derived biopharmaceuticals. The exercise of the option triggered a one-time commercial license fee of \$6.0 million, payment of the remaining \$2.3 million of the previously agreed \$4.0 million in research milestones, development and commercialization milestone payments for each product, and royalties on sales of products. Furthermore, DAS has the right to sublicense Sangamo's ZFP technology to third parties for use in plant cells, plants or plant cell cultures. Sangamo will be entitled to 25% of any cash consideration received by DAS under such sublicenses. In December 2010, the Company amended the DAS Agreement to extend the period of reagent manufacturing services and research services through December 31, 2012.

The DAS Agreement also provides for minimum license fees each year due to Sangamo every October, provided the Agreement is not terminated by DAS. Annual fees range from \$250,000 to \$3.0 million and total \$25.3 million over 11 years. The Company does not have any ongoing performance obligations under the agreement with DAS. DAS has the right to terminate the agreement at any time; accordingly, the Company's actual license fees over the term of the DAS Agreement could be lower than \$25.3 million. In addition, each party may terminate the DAS Agreement upon an uncured material breach by the other party. In the event of any termination of the DAS Agreement, all rights to use the Company's ZFP technology will revert to Sangamo, and DAS will no longer be permitted access to Sangamo's ZFP technology or to develop or, except in limited circumstances, commercialize any products derived from the Company's ZFP technology.

There were no revenues or related costs and expenses recognized under the DAS Agreement during the three and nine months ended September 30, 2015 and 2014, respectively.

#### Funding from Research Foundations

##### California Institute for Regenerative Medicine - HIV

In May 2014 CIRM agreed to fund a \$5.6 million Strategic Partnership Award to fund the clinical studies of this potentially curative ZFP Therapeutic for HIV/AIDS based on the application of its ZFN genome editing technology in hematopoietic stem and progenitor cells (HSPCs). The four year grant provides matching funds to support evaluation of the Company's stem cell-based ZFP Therapeutic in a clinical trial in HIV-infected individuals conducted at City of Hope.

There were no revenues attributable to research and development performed under the Strategic Partnership Award during the three and nine months ended September 30, 2015 and 2014, respectively. Related costs and expenses incurred under the CIRM Strategic Partnership Award were \$0.6 million and \$0.0 million during the three months ended September 30, 2015 and 2014, respectively and \$1.3 million and \$0.0 million during the nine months ended September 30, 2015 and 2014, respectively.

##### California Institute for Regenerative Medicine - Beta-Thalassemia

In May 2013 CIRM granted Sangamo a \$6.4 million Strategic Partnership Award to develop a potentially curative ZFP Therapeutic for beta-thalassemia based on the application of its ZFN gene editing technology in HSCs. The four-year grant was intended to provide matching funds for preclinical work to support an IND application and a Phase 1 clinical trial in transfusion-dependent beta-thalassemia patients using the BCL11A knockout strategy. In May 2015 Sangamo announced a consolidated development path for its beta-thalassemia and SCD programs using the "BCL11A Enhancer" target. Due to the switch to the BCL11A Enhancer strategy, CIRM and Sangamo terminated the Strategic Partnership Award as of June 30, 2015. Sangamo returned \$3.0 million in unused funds received from

CIRM under the award during the three months ended September 30, 2015.

Revenue attributable to research and development performed under the CIRM grant agreement for beta-thalassemia was \$0.0 million and \$0.4 million during the three months ended September 30, 2015 and 2014, respectively and \$1.2 million and \$1.1 million during the nine months ended September 30, 2015 and 2014, respectively. Related costs and expenses incurred under the CIRM grant agreement were \$0.0 million and \$0.4 million during the three months ended September 30, 2015 and 2014, respectively and \$1.2 million and \$1.1 million during the nine months ended September 30, 2015 and 2014, respectively.

## NOTE 6—INTANGIBLE ASSETS

Intangible assets for IPR&D consisted of two clinical product candidates from our 2013 acquisition of Ceregene. IPR&D is an intangible asset classified as indefinite-lived until the completion or abandonment of the associated research and development effort, and is amortized over an estimated useful life to be determined at the date the project is completed.

The carrying values of these intangibles assets are as follows (in thousands):

	As of September 30, 2015	As of December 31, 2014
CERE-110 for the treatment of Alzheimer's disease	\$ —	\$ 1,640
CERE-120 for the treatment of Parkinson's disease	—	230
<b>Total identifiable intangible assets</b>	<b>\$ —</b>	<b>\$ 1,870</b>

In the first quarter of 2015, the Company decided to discontinue the CERE-110 and CERE-120 clinical trial programs. As such, the probability of achieving projected revenues and cash flows associated with these programs were adversely affected. The Company does not believe the programs have an alternative future use for itself or other market participants. Accordingly, during the nine months ended September 30, 2015, the Company recognized a \$1.9 million impairment charge related to these assets. The impairment is recorded in research and development expense in the accompanying condensed consolidated statements of operations.

## NOTE 7—INCOME TAXES

The Company maintains deferred tax assets that reflect the net tax effects of temporary differences between the carrying amounts of assets and liabilities for financial reporting purposes and the amounts used for income tax purposes. These deferred tax assets include net operating loss carryforwards, research credits and capitalized research and development costs. Realization of deferred tax assets is dependent upon future earnings, if any, the timing and amount of which are uncertain based on Sangamo's history of losses. Accordingly, the Company's net deferred tax assets have been fully offset by a valuation allowance. Utilization of operating losses and credits may be subject to substantial annual limitation due to ownership change provisions of the Internal Revenue Code of 1986, as amended and similar state provisions. The annual limitation may result in the expiration of net operating losses and credits before utilization.

During the nine months ended September 30, 2015, the Company received a \$14.5 million disgorgement settlement that was recognized as additional paid-in capital. The disgorgement settlement was recognized net of taxes of \$5.8 million, which under the intraperiod tax allocation rules resulted in an income tax benefit of \$3.4 being recognized in the accompanying condensed consolidated statements of operations for the three and nine months ended September 30, 2015.

## NOTE 8—STOCK-BASED COMPENSATION

The following table shows total stock-based compensation expense included in the condensed consolidated statements of operations for the three and nine months ended September 30, 2015 and 2014 (in thousands):

	Three months ended September 30, 2015		Nine months ended September 30, 2014	
Research and development	\$1,595	\$1,182	\$5,020	\$3,418
General and administrative	1,233	947	3,644	2,747
Total stock-based compensation expense	\$2,828	\$2,129	\$8,664	\$6,165

NOTE 9—CLAIMS SETTLEMENT

In September 2015, the Company received \$14.5 million as a settlement with certain investors who were beneficial owners of our common stock related to the disgorgement of short-swing profits pursuant to Section 16 of the Securities Exchange Act of 1934, as amended. The settlement of \$8.7 million, net of a \$5.8 million income tax benefit and certain expenses, was recognized as additional paid-in capital.

ITEM MANAGEMENT'S DISCUSSION AND ANALYSIS OF FINANCIAL CONDITION AND RESULTS OF  
2. OPERATIONS

The discussion in "Management's Discussion and Analysis of Financial Condition and Results of Operations" contains trend analysis, estimates and other forward-looking statements within the meaning of Section 27A of the Securities Act of 1933, as amended, and Section 21E of the Securities Exchange Act of 1934, as amended. These forward-looking statements include, without limitation, statements containing the words "believes," "anticipates," "expects," "continue," and other words of similar import or the negative of those terms or expressions. Such forward-looking statements are subject to known and unknown risks, uncertainties, estimates and other factors that may cause the actual results, performance or achievements of the Company, or industry results, to be materially different from any future results, performance or achievements expressed or implied by such forward-looking statements. You should read the following discussion and analysis along with the financial statements and notes attached to those statements included elsewhere in this report and in our annual report on Form 10-K for the year ended December 31, 2014 as filed with the SEC.

Overview

We are a clinical stage biopharmaceutical company focused on the research, development and commercialization of engineered DNA-binding proteins for the development of novel therapeutic strategies for unmet medical needs. Our current mission is to develop ZFP Therapeutics®, or human therapeutics based on our proprietary zinc finger DNA-binding protein (ZFP) technology, through early stage clinical testing, strategically partner with biopharmaceutical companies at points of value inflection and have the partners execute late-stage clinical trials and commercial development. In the long-term, our goal is to integrate marketing and development operations and to capture the value of late-stage and commercial ZFP Therapeutic products for ourselves.

We and our licensed partners are the leaders in the research, development and commercialization of ZFPs, a naturally occurring class of proteins. We have used our knowledge and expertise to develop a proprietary technology platform. ZFPs can be engineered to make ZFP nucleases (ZFNs), proteins that can be used to modify DNA sequences in a variety of ways and ZFP transcription factors (ZFP TFs), proteins that can be used to turn genes on or off. As ZFPs act at the DNA level, they have broad potential applications in several areas, including human therapeutics, plant agriculture and research reagents, as well as production of transgenic animals and cell-line engineering.

The main focus for our company is the development of novel human therapeutics and we are building a pipeline of ZFP Therapeutics. Our lead ZFP Therapeutic, SB-728-T, a ZFN-modified autologous T-cell product for the treatment of HIV/AIDS, is the first therapeutic application of our ZFN technology and is being evaluated in a Phase 2 clinical trial in HIV-infected subjects. We also have a Phase 1 clinical trial of this same approach in hematopoietic stem cells.

In January 2014 we entered into a collaborative partnership with Biogen Inc., formerly Biogen Idec Inc. (Biogen) to research, develop and commercialize our preclinical ZFP Therapeutic development program in hemoglobinopathies, targeting sickle cell disease (SCD) and beta-thalassemia. In January 2012 we entered into a collaborative partnership with Shire International GmbH, formerly Shire AG (Shire), to research, develop and commercialize certain of our preclinical ZFP Therapeutic development programs, including programs in hemophilia A and B, Huntington's disease (HD) and other monogenic diseases. On September 1, 2015, the agreement with Shire was amended such that Shire agreed to return to us the exclusive world-wide rights to gene targets for the development, clinical testing and commercialization of ZFP Therapeutics for hemophilia A and B. Shire will retain rights and will continue to develop ZFP Therapeutic clinical leads for HD disease and a ZFP Therapeutic for one additional gene target yet to be named. Sangamo will provide certain target feasibility services, and upon Shire's request, certain research activities according to a research plan as agreed upon by both companies. Such research activities performed by Sangamo will be reimbursed by Shire. Shire's rights with respect to other targets contemplated in the original agreement revert to Sangamo. Under the revised agreement, each company is responsible for expenses associated with its own programs and will reimburse the other for any ongoing services provided. We also have proprietary preclinical programs in several lysosomal storage disorders (LSDs). In addition, we have research stage programs in other monogenic

diseases, including certain immunodeficiencies, as well as central nervous system (CNS) disorders and cancer immunotherapy.

We believe the potential commercial applications of ZFPs are broad-based and we have entered into strategic partnerships in fields outside human therapeutics to facilitate the sale or licensing of our ZFP platform as follows:

- We have a license agreement with the research reagent company Sigma-Aldrich Corporation (Sigma). Sigma has the exclusive rights to develop and market high value laboratory research reagents based upon our ZFP technology as well as ZFP-modified cell lines for commercial production of protein pharmaceuticals and ZFP-engineered transgenic animals. Sigma is marketing ZFN-derived gene editing tools under the trademark CompoZr®.
- We have a license agreement with Dow AgroSciences, LLC (DAS), a wholly owned subsidiary of Dow Chemical Corporation. Under the agreement, we have provided DAS with access to our ZFP technology and the exclusive rights to use it to modify the genomes or alter protein expression of plant cells, plants or plant cell cultures. DAS markets our ZFN

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technology under the trademark EXZACT™ Precision Technology. We have retained rights to use plants or plant-derived products to deliver ZFP TFs or ZFNs into human or animals for diagnostic, therapeutic or prophylactic purposes.

We have incurred net losses since inception and expect to incur losses in the future as we continue our research and development activities. To date, we have funded our operations primarily through the issuance of equity securities, payments from corporate collaborations and research grants.

For the three months ended September 30, 2015, we incurred a consolidated net loss of \$9.2 million, or \$0.13 per share, compared to a net loss of \$7.5 million, or \$0.11 per share, for the same period in 2014. For the nine months ended September 30, 2015, we incurred a consolidated net loss of \$26.7 million, or \$0.38 per share, compared to a net loss of \$22.1 million, or \$0.33 per share, for the same period in 2014. As of September 30, 2015, we had cash, cash equivalents, marketable securities and interest receivable totaling \$219.7 million compared to \$226.6 million as of December 31, 2014. As of September 30, 2015, we had an accumulated deficit of \$355.2 million.

Our revenues have consisted primarily of revenues from partnerships of our ZFP technology platform in both therapeutic and non-therapeutic applications, including license fees, research reimbursement and milestones, royalties, as well as revenues from research grant funding. We expect revenues will continue to fluctuate from period to period, and there can be no assurance that new collaborations or partner funding will continue beyond their current terms.

In the development of our ZFP technology platform, we are focusing our resources on higher-value ZFP Therapeutic product development and less on our non-therapeutic applications. Development of novel therapeutic products is costly and is subject to a lengthy and uncertain regulatory process at the FDA. Our future products will be gene-based therapeutics. Adverse events in both our own clinical program and other programs may have a negative impact on regulatory approval, the willingness of potential commercial partners to enter into agreements and public perception.

#### Critical Accounting Estimates

The accompanying discussion and analysis of our financial condition and results of operations are based upon our condensed consolidated financial statements and the related disclosures, which have been prepared in accordance with generally accepted accounting principles in the United States. The preparation of these financial statements requires us to make estimates, assumptions and judgments that affect the reported amounts in our consolidated financial statements and accompanying notes. We base our estimates on historical experience and on various other assumptions that we believe to be reasonable under the circumstances, the results of which form the basis for making judgments about the carrying values of assets and liabilities that are not readily apparent from other sources. Actual results may differ from these estimates under different assumptions or conditions. We believe that there have been no significant changes in our critical accounting policies and estimates disclosed in our Annual Report on Form 10-K for the year ended December 31, 2014, as filed with the SEC.

#### Results of Operations

Three and nine months ended September 30, 2015 and 2014

#### Revenues

Three months ended September 30, (in thousands, except percentage values)				Nine months ended September 30, (in thousands, except percentage values)			
2015	2014	Change	%	2015	2014	Change	%

Revenues:								
Collaboration agreements	\$8,406	\$12,045	\$(3,639)	(30%)	\$28,878	\$29,334	\$(456)	(2%)
Research Grants	163	372	(209)	(56%)	1,540	1,584	(44)	(3%)
Total revenues	\$8,569	\$12,417	\$(3,848)	(31%)	\$30,418	\$30,918	\$(500)	(2%)

Total revenues consist of revenues from collaboration agreements and research grants. We anticipate revenues over the next several years will be derived primarily from our collaboration agreements with Biogen, DAS and Sigma.

Revenues from our corporate collaboration agreements were \$8.4 million for the three months ended September 30, 2015, compared to \$12.0 million in the corresponding period in 2014. The \$3.6 million decrease in collaboration agreements revenues was primarily due to a decrease of \$2.7 million in revenues related to the amendment of our collaboration and license agreement with Shire and a \$0.9 million decrease in revenues related to our collaboration and license agreement with Biogen. The revenues from Shire included \$3.3 million from research services and \$0.5 million related to partial recognition of an upfront license fee of \$13.0 million. The revenues from Biogen included \$2.6 million from research services and \$1.6 million related to partial recognition of an upfront



payment of \$20.0 million. Research grant revenues were approximately \$0.2 million for the three months ended September 30, 2015, compared to \$0.4 million in the corresponding period in 2014.

Revenues from our corporate collaboration agreements were \$28.9 million for the nine months ended September 30, 2015, compared to \$29.3 million in the corresponding period in 2014. The decrease of \$0.4 million in collaboration agreement revenues was primarily attributable to a \$5.2 million decrease in revenues related to the amendment of our agreement with Shire and a \$0.8 million decrease under our license agreement with Open Monoclonal Technology, Inc., partially offset by a \$4.0 million increase in revenues related to our agreement with Sigma and a \$1.4 million increase in revenues related to our agreement with Biogen. Research grant revenues were \$1.5 million for the nine months ended September 30, 2015, compared to \$1.6 million in the corresponding period in 2014.

### Operating Expenses

	Three months ended September 30, (in thousands, except percentage values)				Nine months ended September 30, (in thousands, except percentage values)			
	2015	2014	Change	%	2015	2014	Change	%
<b>Operating expenses:</b>								
Research and development	\$ 16,694	\$ 16,340	\$ 354	2 %	\$ 47,292	\$ 41,883	\$ 5,409	13 %
General and administrative	4,560	3,731	829	22 %	14,309	11,347	2,962	26 %
Total expenses	\$ 21,254	\$ 20,071	\$ 1,183	6 %	\$ 61,601	\$ 53,230	\$ 8,371	16 %

### Research and development

Research and development expenses consist primarily of salaries and personnel related expenses, including stock-based compensation, laboratory supplies, preclinical and clinical studies, manufacturing expenses, allocated facilities expenses, subcontracted research expenses and expenses for technology licenses. In 2015, we established a Technical Operations group to manage the relationships with third-party vendors used in our manufacturing processes as well as to improve our process development and increase our overall manufacturing capabilities. We expect to continue to devote substantial resources to research and development in the future and expect research and development expenses to increase in the next several years if we are successful in advancing our HIV/AIDS program in the clinic and if we are able to move our earlier stage ZFP Therapeutic product candidates into clinical trials. We also expect that expenses related to research performed under our collaboration and license agreement with Biogen will increase our research and development expenses during the terms of the agreements. Pursuant to the terms of the agreement with Biogen, future expenses for research activities under the collaboration will be reimbursed, including internal employee and external research costs related to the programs. The reimbursement for these services will be recognized as revenue as the expenses are incurred and collection is reasonably assured.

Research and development expenses were \$16.7 million for the three months ended September 30, 2015, compared to \$16.3 million in the corresponding period in 2014. The increase of \$0.4 million in research and development expenses was primarily due to increases of \$0.7 million increase in facilities and lab supply expenses, \$0.6 million in salaries and benefits and \$0.4 million in stock-based compensation expense attributable to the establishment of a Technical Operations group in 2015, partially offset by decrease of \$0.6 million in external research expenses and \$0.7 million in clinical trial and manufacturing expenses related to our HIV/AIDS program.

Research and development expenses were \$47.3 million for the nine months ended September 30, 2015, compared to \$41.9 million in the corresponding period in 2014. The increase of \$5.4 million in research and development expenses

was primarily due to an increase of \$2.2 million in salaries and benefits, \$2.0 million increase in facilities expense related to increased headcount in the Technical Operations group and \$1.6 million in stock-based compensation expense. The increase was partially offset by a decrease of \$1.1 million in external research expenses.

#### General and administrative

General and administrative expenses consist primarily of salaries and personnel related expenses, including stock-based compensation, other expenses for executive, finance and administrative personnel, professional fees, allocated facilities expenses, patent prosecution expenses and other general corporate expenses. As we continue to pursue clinical and commercial development of our therapeutic programs, we expect the business aspects of the Company to become more complex. In the future we may be required to add personnel and incur additional expenses related to the maturity of our business.

General and administrative expenses were \$4.6 million for the three months ended September 30, 2015 compared to \$3.7 million for the corresponding period in 2014. The increase was primarily related to an increase of \$0.3 million in salaries and benefits, \$0.3 million in stock-based compensation expense and \$0.2 million in professional fees and consulting expense.

General and administrative expenses were \$14.3 million for the nine month period ended September 30, 2015 and \$11.3 million for the corresponding period in 2014. The increase was primarily related to an increase of \$1.0 million in professional fees and consulting expense, \$0.9 million in stock-based compensation expense and \$0.6 million in salaries and benefits.

#### Benefit from income taxes

During the nine months ended September 30, 2015, the Company recognized an income tax benefit of \$4.1 million, including a \$3.4 million benefit from income taxes related to the \$14.5 million settlement with certain investors who were beneficial owners. Under the intraperiod tax allocations rules, the income taxes related to the settlement recognized in additional paid-in capital requires an income tax benefit to be recognized.

#### Liquidity and Capital Resources

##### Liquidity

Since inception, we have incurred significant net losses and we have funded our operations primarily through the issuance of equity securities, payments from corporate collaborators and strategic partners and research grants.

As of September 30, 2015, we had cash, cash equivalents, marketable securities and interest receivable totaling \$219.7 million compared to \$226.6 million as of December 31, 2014.

Our most significant use of capital pertains to salaries and benefits for our employees and external development expenses, such as manufacturing and clinical trial activities, related to our ZFP Therapeutic programs. Our cash and investment balances are held in a variety of interest bearing instruments, which can include obligations of U.S. government agencies, U.S. treasury debt securities, corporate debt securities, commercial paper securities and money market funds. Cash in excess of immediate requirements is invested in accordance with our investment policy with a view toward capital preservation and liquidity.

Under the agreement with Biogen, we received an upfront license fee of \$20.0 million in 2014. Biogen will reimburse us for agreed upon costs incurred in connection with research and development activities conducted by us. In addition, we are eligible to receive development milestone payments upon the achievement of specified regulatory, clinical development and commercialization milestones. We will also be eligible to receive incremental royalties for each licensed product that are a tiered double-digit percentage of annual net sales of such product, if any.

In January 2012, we entered into a license and collaboration agreement with Shire, under which we received an upfront license fee of \$13.0 million. In addition, Shire agreed to reimburse us for agreed upon costs incurred in connection with research and development activities that we conducted and to pay us certain milestone payments based on our achievement of specified research, regulatory, clinical development, commercialization and sales milestones, which depended upon our ability with Shire to continue to progress our programs under collaboration. We were also eligible to receive royalty payments on net sales of products developed under the collaboration, if any. On September 1, 2015, we amended the Shire agreement such that going forward, each company is responsible for expenses associated with its own programs and will reimburse the other for any ongoing services provided. Under the amended agreement, Shire does not have any milestone payment obligations to us with respect to the retained programs, but it is required to pay single digit percentage royalties to us, up to a specified maximum cap, on the commercial sales of ZFP therapeutic products from such programs. Under the Agreement, we have full control over, and full responsibility for the costs of, the hemophilia programs returned to us, subject to certain diligence obligations and Shire's right of first negotiation to obtain a license to such programs under certain circumstances. We are required to pay single digit percentage royalties to Shire, up to a specified maximum cap, on commercial sales of ZFP therapeutic products from such returned programs.

## Cash Flow

Operating activities. Net cash used in operating activities for the nine months ended September 30, 2015 and 2014 was \$24.6 million and \$3.0 million, respectively. Net cash used in operating activities for the nine months ended September 30, 2015 primarily reflected the increases in net loss for the period as well as a decrease in deferred revenue, partially offset by the increases in stock-based compensation and a decrease in accounts receivable. Net cash used in operating activities for the nine months ended September 30, 2014 primarily reflected the increases in net loss for the period as well as an increase in accounts receivable and other current assets and a decrease in accrued compensation, partially offset by the increases in deferred revenues related to our collaboration agreement with Biogen, accounts payable and stock-based compensation.

Investing activities. Net cash provided in investing activities for the nine months ended September 30, 2015 was \$32.2 million, while cash used in investing activities was \$100.3 million for the nine months ended September 30, 2014. Cash flows from investing activities for both periods primarily related to purchases and maturities of investments.

Financing activities. Net cash provided by financing activities for the nine months ended September 30, 2015 and 2014 was \$20.7 million and \$104.3 million, respectively. Net cash provided by financing activities for the nine month period ended September 30, 2015 was primarily related to a \$14.5 million settlement with certain investors that were beneficial owners of our common stock related to the disgorgement of short-swing profits pursuant to Section 16 of the Securities Exchange Act of 1934, as amended as well as proceeds from the exercise of stock options. Net cash provided by financing activities for the nine month period ended September 30, 2014 was primarily attributable to \$93.8 million in net proceeds from the public offering of the Company's common stock completed in March 2014 as well as proceeds from the exercise of stock options.

#### Operating Capital and Capital Expenditure Requirements

We anticipate continuing to incur operating losses for at least the next several years. While our rate of cash usage may increase in the future, in particular to support our product development endeavors, we believe that the available cash resources as well as funds received from corporate collaborators, strategic partners and research grants will enable us to maintain our currently planned operations through 2016. Future capital requirements will be substantial, and if our capital resources are insufficient to meet future capital requirements, we will need to raise additional capital to fund our operations, including ZFP Therapeutic development activities, through equity or debt financing. We regularly consider fund raising opportunities and may decide, from time to time, to raise capital based on various factors, including market conditions and our plans of operation. Additional capital may not be available on terms acceptable to us, or at all. If adequate funds are not available, or if the terms of potential funding sources are unfavorable, our business and our ability to develop our technology and our ZFP Therapeutic products would be harmed. Furthermore, any sales of additional equity securities may result in dilution to our stockholders, and any debt financing may include covenants that restrict our business.

Our future capital requirements will depend on many factors and are not limited to the following:

- the initiation, progress, timing and completion of clinical trials for our product candidates;
- the outcome, timing and cost of regulatory approvals;
- the success of our collaborations with Biogen, Shire and other partners;
- delays that may be caused by changing regulatory requirements;
- the number of product candidates that we pursue;
- the costs involved in filing and prosecuting patent applications and enforcing and defending patent claims;
  - the timing and terms of future in-licensing and out-licensing transactions;
- the cost of procuring clinical and commercial supplies, including the cost to manufacture our ZFP therapeutic products;
- investment made in the development of internal process development and manufacturing capabilities;
- the extent to which we acquire or invest in businesses, products or technologies; and
- the costs of litigation.

#### ITEM 3. QUANTITATIVE AND QUALITATIVE DISCLOSURES ABOUT MARKET RISK

Our primary exposure to market risk is interest income sensitivity relating to our cash, cash equivalents and investments, which is affected by changes in the general level of U.S. interest rates. We do not have any foreign currency or other derivative financial instruments.

Our market risks at September 30, 2015 have not changed materially from those discussed in Item 7A of our Form 10-K for the year ended December 31, 2014 on file with the SEC.

ITEM 4. CONTROLS AND PROCEDURES

Disclosure Controls and Procedures

We maintain disclosure controls and procedures that are designed to provide reasonable assurance that information required to be disclosed in our Exchange Act reports is recorded, processed, summarized and reported within the time periods specified in the Securities and Exchange Commission's rules and forms and that such information is accumulated and communicated to our management, including our Chief Executive Officer and Chief Financial Officer, as appropriate, to allow timely decisions regarding required disclosure. In designing and evaluating the disclosure controls and procedures, management recognizes that any controls and procedures, no matter how well designed and operated, can provide only reasonable, and not absolute, assurance of achieving the desired control objectives. In reaching a reasonable level of assurance, management necessarily was required to apply its judgment in evaluating the cost benefit relationship of possible controls and procedures.

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As required by the Securities and Exchange Commission Rule 13a-15(b), we carried out an evaluation, under the supervision of and with the participation of our management, including our Chief Executive Officer and Chief Financial Officer, of the effectiveness of the design and operation of our disclosure controls and procedures as of the end of the period covered by this report. Based on the foregoing, our Chief Executive Officer and Chief Financial Officer concluded that our disclosure controls and procedures were effective at the reasonable assurance level.

#### Change in Internal Control over Financial Reporting

There has been no change in our internal controls over financial reporting during our most recent fiscal quarter that has materially affected, or is reasonably likely to materially affect, our internal controls over financial reporting.

## PART II. OTHER INFORMATION

### ITEM 1. LEGAL PROCEEDINGS

We are not party to any material pending legal proceedings.

### ITEM 1A. RISK FACTORS

An investment in our common stock involves significant risk. You should carefully consider the information described in the following risk factors, together with the other information appearing elsewhere in this report, before making an investment decision regarding our common stock. While the risk factors set forth below update and supplement the risk factors set forth in our Annual Report on Form 10-K for the year ended December 31, 2014 (2014 Annual Report), you should review our 2014 Annual Report, including the section under the caption "Item 1A. Risk Factors," together with the other information appearing elsewhere in this report, before making an investment decision regarding our common stock. If any of the risks described below or in our 2014 Annual Report actually occur, our business, financial conditions, results of operation and future growth prospects would likely be materially and adversely affected. In these circumstances, the market price of our common stock could decline, and you may lose all or a part of your investment in our common stock. Moreover, the risks described below and in our 2014 Annual Report are not the only ones that we face. Additional risks not presently known to us or that we currently deem immaterial may also affect our business, operating results, prospects or financial condition. You should carefully consider these risk factors, together with all of the other information included in this Form 10-Q as well as our other publicly available filings with the Securities and Exchange Commission.

#### Risks Relating to Development, Commercialization and Regulatory Approval of our Products and Technology

ZFP Therapeutics have undergone limited testing in humans and our ZFP Therapeutics may fail safety studies in clinical trials.

We are conducting an on-going Phase 2 clinical trial (SB-728-mR-1401) of our ZFP Therapeutics for the treatment of HIV/AIDS and a Phase 1 study of the same approach in HSPCs. Preliminary data from these studies demonstrates that treatment of aviremic HIV-infected subjects with SB-728-T has been well-tolerated. In addition, data from Phase 1 and several Phase 2 clinical trials of our ZFP Therapeutic, SB-509, for diabetic neuropathy and ALS demonstrated that the drug was well tolerated in these studies. However, if one of our ZFP Therapeutic fails one of its safety studies, it could reduce our ability to attract new investors and corporate partners.

All of these studies are designed primarily to evaluate the safety and tolerability of this ZFP Therapeutic approach. Our clinical studies are a highly visible test of our ZFP Therapeutics and our investors assess the value of our technology primarily based on the continued progress of ZFP Therapeutic products into and through clinical trials. If clinical trials of our ZFP Therapeutic products were halted due to safety concerns, this would negatively affect our operations and the value of shares in our common stock.

Our progress in early Phase 1 and Phase 2 trials may not be indicative of long-term efficacy in late stage clinical trials.

The results in early phases of clinical testing are based upon limited numbers of patients and a limited follow-up period. Typically, our Phase 1 clinical trials for indications of safety enroll less than 25 patients. Our Phase 2 and late-stage clinical trials generally enroll a larger number of patients. Accordingly, any positive data obtained in early Phase 1 and Phase 2 trials may not be indicative of long-term efficacy in late-stage clinical trials.



In September 2011, we announced preliminary data from our clinical program to develop SB-728-T for the treatment of HIV/AIDS which is now in Phase 2 clinical testing. The data demonstrated a statistically significant relationship between SB-728-T and the reduction of HIV viral load. In January 2012, we initiated a Phase 2 clinical study (SB-728-902, Cohort 5) and a Phase 1/2 clinical study (SB-728-1101) for the treatment of HIV/AIDS. In December 2013, we presented data from all cohorts of these two clinical trials. Three of seven evaluable subjects in Cohort 5 showed a decrease of greater than one log in their viral load during a sixteen week treatment interruption (TI) of their antiretroviral therapy (ART) with one subject achieving a transiently undetectable viral load during the TI period and one subject achieving control of their viral load during TI for a prolonged period (>70 weeks as of January 2015). Two of three additional subjects, enrolled in Cohort 3\* of this study, have demonstrated notable reductions in viral load during a TI from ART. Additional data were presented from the Company's Phase 1 study (SB-728-902, Cohorts 1-3) that demonstrated a long-term decrease in the peripheral blood mononuclear cell (PBMC) HIV reservoir using a sensitive test for integrated HIV DNA in nine of nine subjects over a 36 month period (median decrease 0.9 logs). Additional subjects were enrolled into the SB-728-1101 study to define the optimum dose of CytoXan required to safely enhance engraftment and an additional 12 subjects have been enrolled to further test this dose including nine subjects in an ongoing Phase 2 clinical trial (SB-728mR-T-1401) that is also testing repeat dosing of modified T-cells. However, there is no guarantee that these and other future studies of SB-728-T in later stage trials involving larger patient groups may produce positive or similar results as those obtained in earlier trials. In subjects in which viral load decreased, a measurable anti-HIV immune response was also observed.

A number of companies in the pharmaceutical and biotechnology industries have suffered significant setbacks in late stage clinical trials even after achieving promising results in earlier stage clinical trials. If a larger population of patients does not experience positive results, or if these results are not reproducible, our products may not receive approval from the FDA. Failure to confirm favorable results from earlier trials by demonstrating the safety and effectiveness of our ZFP Therapeutic products in late stage clinical trials with larger patient populations could have a material adverse effect on our business that would cause our stock price to decline significantly.

Our potential therapeutic products are subject to a lengthy and uncertain regulatory process, and we may encounter unanticipated toxicity or adverse events or fail to demonstrate efficacy, causing us to delay, suspend or terminate the development of a ZFP Therapeutic. If these potential products are not approved, we will not be able to commercialize those products.

The FDA must approve any human therapeutic product before it can be marketed in the United States. The process for receiving regulatory approval is long and uncertain, and a potential product may not withstand the rigors of testing under the regulatory approval processes.

Before commencing clinical trials in humans, we must submit an IND application to the FDA. The FDA has 30 days to comment on the application, and if the agency has no comments, we or our commercial partner may begin clinical trials. While we have stated our intention to file additional IND applications in the future, this is only a statement of intent, and we may not be able to do so because the associated product candidates may not meet the necessary preclinical requirements. In addition, there can be no assurance that, once filed, an IND application will result in the actual initiation of clinical trials. Clinical trials are subject to oversight by institutional review boards and the FDA. In addition, our proposed clinical studies require review from the Recombinant DNA Advisory Committee (RAC), which is the advisory board to the National Institutes of Health (NIH), focusing on clinical trials involving gene transfer. We will typically submit a proposed clinical protocol and other product-related information to the RAC three to six months prior to the expected IND application filing date.

#### Clinical trials:

- must be conducted in conformance with the FDA's good clinical practices, within the guidelines of the International Conference on Harmonisation of Technical Requirements for Registration of Pharmaceuticals for Human Use (ICH) and other applicable regulations;
- must meet requirements for Institutional Review Board (IRB) oversight;
- must follow Institutional Biosafety Committee (IBC) and NIH RAC guidelines where applicable;
- must meet requirements for informed consent;
- are subject to continuing FDA oversight;
- may require oversight by a Data Safety Monitoring Board (DSMB);
- may require large numbers of test subjects; and
- may be suspended by a commercial partner, the FDA, or us at any time if it is believed that the subjects participating in these trials are being exposed to unacceptable health risks or if the FDA finds deficiencies in the IND application or the conduct of these trials.

While we have stated our goal is to file IND applications for several ZFP Therapeutic programs in the future, we may encounter difficulties that may delay, suspend or scale back our efforts.

We have previously announced a strategy for our ZFP Therapeutic programs that enables the potential filing of two to four new IND applications per year in the foreseeable future. The preparation and submission of IND applications requires us to conduct rigorous and time-consuming preclinical testing, studies, and documentation relating to, among other things, the toxicity, safety, manufacturing, chemistry and clinical protocol of new ZFP Therapeutic products. We may experience unforeseen difficulties that could delay or otherwise prevent us from executing this strategy successfully. For example, we may encounter problems in the manufacturing of our ZFP Therapeutic products and fail to demonstrate consistency in the formulation of the drug. Our preclinical tests may produce negative or inconclusive

results, which may lead us to decide, or regulators may require us, to conduct additional preclinical testing. If we cannot obtain positive results in preclinical testing, we may decide to abandon the projects altogether. In addition, our ability to complete and file certain IND applications depends on the support of our partners and the timely performance of their obligations under relevant collaboration agreements. If our partners are not able to perform such obligations or if they choose to slow down or delay the progress, we may not be able to prepare and file the intended IND applications on a timely basis or at all. Furthermore, the filing of several IND applications involves significant cost and labor, and we may not have sufficient resources and personnel to complete the filing of all intended IND applications, which may force us to scale back the number of IND applications or forego potential IND applications that we believe are promising. Any delay, suspension or reduction of our efforts to pursue our preclinical and IND strategy could have a material adverse effect on our business and cause our stock price to decline.

We may not be able to find acceptable patients or may experience delays in enrolling patients for our clinical trials.

We may experience difficulties or delays in recruiting and enrolling a sufficient number of patients to participate in our clinical trials due to a variety of reasons, including competition from other clinical trial programs for the same indication, failure of patients to meet our enrollment criteria and premature withdrawals of patients prior to the completion of clinical trials. The FDA and institutional review boards may also require large numbers of patients, and the FDA may require that we repeat a clinical trial. Any delay resulting from our failure to enroll a sufficient number of patients on a timely basis may have a material adverse effect on our business.

As we cannot predict whether or when we will obtain regulatory approval to commercialize our product candidates, we cannot predict the timing of any future revenue from these product candidates.

We cannot commercialize any of our ZFP Therapeutics to generate revenue until the appropriate regulatory authorities have reviewed and approved the applications for the product candidates. We cannot ensure that the regulatory agencies will complete their review processes in a timely manner or that we will obtain regulatory approval for any product candidate that we or our collaborators develop. Satisfaction of regulatory requirements typically takes many years, is dependent upon the type, complexity and novelty of the product and requires the expenditure of substantial resources. Regulatory approval processes outside the United States include all of the risks associated with the FDA approval process. In addition, we may experience delays or rejections based upon additional government regulation from future legislation or administrative action or changes in FDA policy during the period of product development, clinical trials and FDA regulatory review.

We have limited experience in conducting clinical trials.

Our most advanced clinical program is our Phase 2 trial to evaluate the safety and efficacy of a ZFP Therapeutic for HIV/AIDS. However, the FDA will require additional clinical testing which involves significantly greater resources, commitments and expertise. As such, it is likely that we would need to enter into a collaborative relationship with a pharmaceutical company that could assume responsibility for late-stage development and commercialization.

We have limited experience in conducting advanced clinical trials and may not possess the necessary resources and expertise to complete such trials. We have entered into a collaborative agreement with Biogen to provide funding and assistance in the development of certain ZFP Therapeutics through the clinical trial process. Under the agreement with Biogen, we are responsible for all research and development through the first human clinical trial for the treatment of beta-thalassemia and both parties are responsible for research and development through the submission of IND for ZFP Therapeutics to treat sickle cell disease (SCD). However, there is no guarantee that we will be able to enter into future collaborative relationships with third parties that can provide us with the funding and expertise for later stage trials.

Regulatory approval, if granted, will be limited to specific uses or geographic areas, which could limit our ability to generate revenues.

Regulatory approval will be limited to the indicated use for which we can market a product. Further, once regulatory approval for a product is obtained, the product and its manufacturer are subject to continual review. Discovery of previously unknown problems with a product or manufacturer may result in restrictions on the product, manufacturer, and manufacturing facility, including withdrawal of the product from the market. In Japan and Europe, regulatory agencies also set or approve prices.

Even if regulatory clearance of a product is granted, this clearance is limited to those specific states and conditions for which the product is useful, as demonstrated through clinical trials. We cannot ensure that any ZFP Therapeutic product developed by us, alone or with others, will prove to be safe and effective in clinical trials and will meet all of the applicable regulatory requirements needed to receive marketing clearance in a given country.

Outside the United States, our ability to market a product is contingent upon receiving a marketing authorization from appropriate regulatory authorities; therefore we cannot predict whether or when we would be permitted to commercialize our product. These foreign regulatory approval processes include all of the risks associated with FDA clearance described above.

Commercialization of our technologies will depend, in part, on strategic partnering with other companies. If we are not able to find partners in the future or if our partners do not diligently pursue product development efforts, we may not be able to develop our technologies or products, which could slow our growth and decrease the value of our stock.

We expect to rely, to some extent, on our strategic partners to provide funding in support of our research and to perform independent research and preclinical and clinical testing. Our technology is broad-based, and we do not currently possess the resources necessary to fully develop and commercialize potential products that may result from our technologies or the resources or capabilities to complete the lengthy marketing approval processes that may be required for the products. Therefore, we plan to rely on strategic partnerships to help us develop and commercialize ZFP Therapeutic products. If we are unable to find partners or if the partners we

find, such as Shire and Biogen, are unable or unwilling to advance our programs, or if they do not diligently pursue product approval, this may slow our progress and adversely affect our ability to generate revenues. In addition, our partners may sublicense or abandon development programs or we may have disagreements or disputes with our partners, which would cause associated product development to slow or cease. In addition, the business or operations of our partners may change significantly through restructuring, acquisition or other strategic transactions or decisions that may negatively impact their ability to advance our programs. There can be no assurance that we will be able to establish further strategic collaborations for ZFP Therapeutic product development. We may require significant time to secure collaborations or partners because we need to effectively market the benefits of our technology to these future collaborators and partners, which may direct the attention and resources of our research and development personnel and management away from our primary business operations. Further, each collaboration or partnering arrangement will involve the negotiation of terms that may be unique to each collaborator or partner. These business development efforts may not result in a collaboration or partnership.

The loss of partnering agreements may delay or terminate the potential development or commercialization of products we may derive from our technologies, but it may also delay or terminate our ability to test ZFP Therapeutic candidates for specific genes. If any partner fails to conduct the collaborative activities successfully or in a timely manner, the preclinical or clinical development or commercialization of the affected product candidates or research programs could be delayed or terminated.

Under typical partnering agreements, we would expect to receive revenue for the research and development of a ZFP Therapeutic product based on achievement of specific milestones, as well as royalties based on a percentage of sales of the commercialized products. Achieving these milestones will depend, in part, on the efforts of our partner as well as our own. If we, or any partner, fail to meet specific milestones, then the partnership may be terminated, which could reduce our revenues. For more information on risks relating to our third party collaborative agreements, see “Risks Relating to our Collaborative Relationships.”

We may be unable to license gene transfer technologies that we may need to commercialize our ZFP technology.

In order to regulate or modify a gene in a cell, the ZFP must be efficiently delivered to the cell. We have licensed certain gene transfer technologies for our ZFP in research including AAV and mRNA technology. We are evaluating these systems and other technologies that may need to be used in the delivery of ZFP into cells for in vitro and in vivo applications, including ZFP Therapeutics. However, we may not be able to license the gene transfer technologies required to develop and commercialize our ZFP Therapeutics. We have not developed our own gene transfer technologies, and we rely on our ability to enter into license agreements to provide us with rights to the necessary gene transfer technology. Our approach has been to license appropriate technology as required. The inability to obtain a license to use gene transfer technologies with entities which own such technology on reasonable commercial terms, if at all, could delay or prevent the preclinical evaluation, drug development collaborations, clinical testing, and/or commercialization of our therapeutic product candidates.

Our gene regulation and genome editing technology is relatively new, and if we are unable to use this technology in all our intended applications, it would limit our revenue opportunities.

Our technology involves a relatively new approach to gene regulation and genome editing. Although we have generated ZFPs for thousands of gene sequences, we have not created ZFPs for all gene sequences and may not be able to do so, which could limit the usefulness of our technology. In addition, while we have demonstrated the function of engineered ZFNs and ZFP TFs in mammalian cells, yeast, insects, plants and animals, we have not yet demonstrated clinical efficacy of this technology in a controlled clinical trial in humans, and the failure to do so could restrict our ability to develop commercially viable products. If we, and our collaborators or strategic partners, are unable to extend our results to new commercially important genes, experimental animal models, and human clinical studies, we may be unable to use our technology in all its intended applications.

The expected value and utility of our ZFNs and ZFP TFs is in part based on our belief that the targeted editing of genes or specific regulation of gene expression may enable us to develop a new therapeutic approach as well as to help scientists better understand the role of genes in disease, and to aid their efforts in drug discovery and development. We also believe that ZFP-mediated targeted genome editing and gene regulation will have utility in agricultural applications. There is only a limited understanding of the role of specific genes in all these fields. Life sciences companies have developed or commercialized only a few products in any of these fields based on results from genomic research or the ability to regulate gene expression. We, our collaborators or our strategic partners, may not be able to use our technology to identify and validate drug targets or to develop commercial products in the intended markets.

Effective delivery of ZFNs and ZFP TFs into the appropriate target cells and tissues is critical to the success of the therapeutic applications of our ZFP technology. In order to have a meaningful therapeutic effect, the ZFP Therapeutic must be delivered to sufficient numbers of cells in the targeted tissue. The ZFN or ZFP TF must be present in that tissue for sufficient time to effect either modification of a therapeutically relevant gene or regulation of its expression. In our current clinical and preclinical programs, we administer our ZFP Therapeutics as a nucleic acid that encodes the ZFN or ZFP TF. We use different formulations to deliver the ZFP Therapeutic depending on the required duration of expression, the targeted tissue and the indication that we intend to treat. However, there can be no assurances that we will be able to effectively deliver our ZFNs and ZFP TFs to produce a beneficial therapeutic effect.

We are conducting proprietary research to discover ZFP Therapeutic product candidates. These programs increase our financial risk of product failure, may significantly increase our research expenditures, and may involve conflicts with future collaborators and strategic partners.

Our proprietary research programs consist of research that is funded solely by us or by grant funding and in which we retain exclusive rights to therapeutic products generated by such research. This is in contrast to certain of our research programs that may be funded by corporate partners in which we may share rights to any resulting products. Conducting proprietary research programs may not generate corresponding revenue and may create conflicts with our collaborators or strategic partners over rights to our intellectual property with respect to our proprietary research activities. Any conflict with our collaborators or strategic partners could reduce our ability to enter into future collaborations or partnering agreements and negatively impact our relationship with existing collaborators and partners that could reduce our revenue and delay or terminate our product development. As we continue to focus our strategy on proprietary research and therapeutic development, we expect to experience greater business risks, expend significantly greater funds and require substantial commitments of time from our management and staff.

Even if our technology proves to be effective, it still may not lead to commercially viable products.

Even if our collaborators or strategic partners are successful in using our ZFP technology in drug discovery, protein production, therapeutic development or plant agriculture, they may not be able to commercialize the resulting products or may decide to use other methods competitive with our technology. To date, no company has received marketing approval or has developed or commercialized any therapeutic or agricultural products based on our technology. Should our technology fail to provide safe, effective, useful or commercially viable approaches to the discovery and development of these products, this would significantly limit our business and future growth and would adversely affect our value.

Even if our product development efforts are successful and even if the requisite regulatory approvals are obtained, our ZFP Therapeutics may not gain market acceptance among physicians, patients, healthcare payers and the medical community.

A number of additional factors may limit the market acceptance of our ZFP Therapeutic products including the following:

- rate of adoption by healthcare practitioners;
- rate of a product's acceptance by the target population;
- timing of market entry relative to competitive products;
- availability of alternative therapies;
- price of our product relative to alternative therapies;
- availability of third-party reimbursement;
- extent of marketing efforts by us and third-party distributors or agents retained by us; and
- side effects or unfavorable publicity concerning our products or similar products.

Therefore, even after we have obtained the required regulatory approval for our ZFP Therapeutic products, we may not be able to commercialize these products successfully if we cannot achieve an adequate level of market acceptance.

We currently rely on third parties to conduct some or all aspects of manufacturing of our ZFP Therapeutic product candidates for preclinical and clinical development. If one of our third-party manufacturers fails to perform adequately or fulfill our needs, we may be required to incur significant costs and devote significant efforts, to find new suppliers or manufacturers.

We currently have limited experience in, and we do not own facilities for, clinical-scale manufacturing of our product candidates and we rely upon third-party contract manufacturing organizations to manufacture and supply drug product for our preclinical and clinical studies. The manufacture of pharmaceutical products in compliance with the FDA's



current good manufacturing practices (cGMP), requires significant expertise and capital investment, including the development of advanced manufacturing techniques and process controls. Manufacturers of pharmaceutical products often encounter difficulties in production, including difficulties with production costs and yields, quality control, including stability of the product candidate and quality assurance testing, shortages of qualified personnel, as well as compliance with strictly enforced cGMP requirements, other federal and state regulatory requirements and foreign regulations. If our manufacturers were to encounter any of these difficulties or otherwise fail to comply with their obligations to us or under applicable regulations, our ability to provide study drugs in our clinical studies would be jeopardized. Any delay or interruption in the supply of clinical study materials could delay the completion of our clinical studies, increase the costs associated with maintaining our clinical study programs and, depending upon the period of delay, require us to commence new studies at significant additional expense or terminate the studies completely.

All manufacturers of our product candidates must comply with cGMP requirements enforced by the FDA through its facilities inspection program. These requirements include, among other things, quality control, quality assurance and the maintenance of records and documentation. Manufacturers of our product candidates may be unable to comply with these cGMP requirements and with other FDA, state and foreign regulatory requirements. The FDA or similar foreign regulatory agencies may also implement new standards at any time, or change their interpretation and enforcement of existing standards for manufacture, packaging or testing of products. We have little control over our manufacturers' compliance with these regulations and standards. A failure to comply with these requirements may result in fines and civil penalties, suspension of production, suspension or delay in product approval, product seizure or recall or withdrawal of product approval. If the safety of any product supplied is compromised due to our manufacturers' failure to adhere to applicable laws or for other reasons, we may not be able to obtain regulatory approval for or successfully commercialize our products and we may be held liable for any injuries sustained as a result. Any of these factors could cause a delay of clinical studies, regulatory submissions, approvals or commercialization of our product candidates, entail higher costs or impair our reputation.

Our current agreements with our suppliers do not provide for the entire supply of the drug product necessary for all anticipated clinical studies or for full scale commercialization. If we and our suppliers cannot agree to the terms and conditions for them to provide the drug product necessary for our clinical and commercial supply needs, we may not be able to manufacture the product candidate until a qualified alternative supplier is identified, which could also delay the development of, and impair our ability to commercialize, our product candidates.

The number of third-party suppliers with the necessary manufacturing and regulatory expertise and facilities is limited, and it could be expensive and take a significant amount of time to arrange for alternative suppliers, which could have a material adverse effect on our business. New suppliers of any product candidate would be required to qualify under applicable regulatory requirements and would need to have sufficient rights under applicable intellectual property laws to the method of manufacturing the product candidate. Obtaining the necessary FDA approvals or other qualifications under applicable regulatory requirements and ensuring non-infringement of third-party intellectual property rights could result in a significant interruption of supply and could require the new manufacturer to bear significant additional costs which may be passed on to us.

We do not currently have the infrastructure or capability to manufacture, market and sell therapeutic products on a commercial scale.

In order for us to commercialize our therapeutic products directly, we would need to develop, or obtain through outsourcing arrangements, the capability to manufacture, market and sell our products on a commercial scale. Currently, we do not have the ability nor the financial resources to establish the infrastructure and organizations needed to execute these functions, including such infrastructure needed for the commercialization of any product from our HIV/AIDS, which can be complex and costly. If we are unable to establish adequate manufacturing, sales, marketing and distribution capabilities, we will not be able to directly commercialize our therapeutics products, which would limit our future growth.

#### Risks Relating to our Industry

If our competitors develop, acquire, or market technologies or products that are more effective than ours, this would reduce or eliminate our commercial opportunity.

Any products that we or our collaborators or strategic partners develop by using our ZFP technology platform will enter into highly competitive markets. Even if we are able to generate ZFP Therapeutics that are safe and effective for their intended use, competing technologies may prove to be more effective or less expensive, which, to the extent these competing technologies achieve market acceptance, will limit our revenue opportunities. In some cases, competing technologies have proven to be effective and less expensive. Competing technologies may include other methods of regulating gene expression or modifying genes. ZFNs and ZFP TFs have broad application in the life

sciences industry and compete with a broad array of new technologies and approaches being applied to genetic research by many companies. Competing proprietary technologies with our product development focus include but are not limited to:

- For ZFP Therapeutics:
- small molecule drugs;
- monoclonal antibodies;
- recombinant proteins;
- gene therapy/cDNAs;
- antisense;
- siRNA and microRNA approaches, exon skipping;

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- CRISPR/Cas9 technology;
- TALE proteins and MegaTALs; and
- meganucleases.
- For our Non-Therapeutic Applications:
  - For protein production: gene amplification, meganucleases, TALE technology, insulator technology, mini-chromosomes and CRISPR/Cas9 technology;
  - For target validation: antisense, siRNA, TALE technology and CRISPR/Cas9 technology;
  - For plant agriculture: recombination approaches, mutagenesis approaches, meganucleases, TALE technology, CRISPR/Cas9 technology, mini-chromosomes; and
  - For transgenic animals: somatic nuclear transfer, embryonic stem cell, TALE, CRISPR/Cas9 technology and transposase technologies.

In addition to possessing competing technologies, our competitors include pharmaceutical and biotechnology companies with:

- substantially greater capital resources than ours;
- larger research and development staffs and facilities than ours; and
- greater experience in product development and in obtaining regulatory approvals and patent protection.

These organizations also compete with us to:

- attract qualified personnel;
- attract parties for acquisitions, joint ventures or other collaborations; and
- license the proprietary technologies of academic and research institutions that are competitive with our technology, which may preclude us from pursuing similar opportunities.

Accordingly, our competitors may succeed in obtaining patent protection or commercializing products before us. In addition, any products that we develop may compete with existing products or services that are well established in the marketplace.

Adverse public perception in the field of gene therapy may negatively impact regulatory approval of, or demand for, our potential products.

Our potential therapeutic products are delivered to patients as gene-based drugs, or gene therapy. The clinical and commercial success of our potential products will depend in part on public acceptance of the use of gene therapy for the prevention or treatment of human diseases. Public attitudes may be influenced by claims that gene therapy is unsafe, and, consequently, our products may not gain the acceptance of the public or the medical community. Negative public reaction to gene therapy in general could result in greater government regulation and stricter labeling requirements of gene therapy products, including any of our products, and could cause a decrease in the demand for any products we may develop.

Laws or public sentiment may limit the production of genetically modified agricultural products, and these laws could reduce our partner's ability to sell such products.

Genetically modified products are currently subject to public debate and heightened regulatory scrutiny, either of which could prevent or delay production of agricultural products. We have a research license and commercial option agreement with DAS through which we provide DAS with access to our proprietary ZFP technology and the exclusive right to use our ZFP technology to modify the genomes or alter the nucleic acid or protein expression of plant cells, plants or plant cell cultures. The field-testing, production and marketing of genetically modified plants and plant products are subject to federal, state, local and foreign governmental regulation. Regulatory agencies administering existing or future regulations or legislation may not allow production and marketing of our genetically modified products in a timely manner or under technically or commercially feasible conditions. In addition, regulatory action or private litigation could result in expenses, delays or other impediments to our product development programs or the commercialization of resulting products.

The FDA currently applies the same regulatory standards to foods developed through genetic engineering as those applied to foods developed through traditional plant breeding. Genetically engineered food products, however, will be subject to pre-market review if these products raise safety questions or are deemed to be food additives. Governmental authorities could also, for social or other purposes, limit the use of genetically modified products created with our gene regulation technology.

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Even if the regulatory approval for genetically modified products developed under our agreement with DAS was obtained, our success will also depend on public acceptance of the use of genetically modified products including drugs, plants, and plant products. Claims that genetically modified products are unsafe for consumption or pose a danger to the environment may influence public attitudes. Our genetically modified products may not gain public acceptance. The subject of genetically modified organisms has received negative publicity in the United States and particularly in Europe, and such publicity has aroused public debate. The adverse publicity in Europe could lead to greater regulation and trade restrictions on imports of genetically altered products. Similar adverse public reaction or sentiment in the United States to genetic research and its resulting products could result in greater domestic regulation and could decrease the demand for our technology and products.

#### Risks Relating to our Finances

We have incurred significant operating losses since inception and anticipate that we will incur continued losses for the foreseeable future.

We have generated operating losses since we began operations in 1995. Our net losses for the years ended December 31, 2014, 2013 and 2012 were \$26.4 million, \$26.6 million and \$22.3 million, respectively. The extent of our future losses and the timing of profitability are uncertain, and we expect to incur losses for the foreseeable future. We have been engaged in developing our ZFP technology since inception, which has and will continue to require significant research and development expenditures. To date, we have generated our funding from issuance of equity securities, revenues derived from collaboration agreements, other strategic partnerships in non-therapeutic applications of our technology, federal government research grants and grants awarded by research foundations. As of September 30, 2015, we had an accumulated deficit of \$355.2 million. Since our IPO in 2000, we have generated an aggregate of approximately \$331.4 million in gross proceeds from the sale of our equity securities. We expect to continue to incur additional operating losses for the next several years as we continue to advance our ZFP Therapeutic product candidates. If the time required to generate significant product revenues and achieve profitability is longer than we currently anticipate or if we are unable to generate liquidity through equity financing or other sources of funding, we may be forced to curtail or suspend our operations.

We may be unable to raise additional capital, which would harm our ability to develop our technology and products.

We have incurred significant operating losses and negative operating cash flows since inception and have not achieved profitability. We expect capital outlays and operating expenditures to increase over the next several years as we expand our infrastructure and research and ZFP Therapeutic product development activities. While we believe our financial resources will be adequate to sustain our current operations at least through 2016, we may need to seek additional sources of capital through equity or debt financing. In addition, as we focus our efforts on proprietary human therapeutics, we will need to seek FDA approval of potential products, a process that could cost in excess of hundreds of millions of dollars per product. Furthermore, we may experience difficulties in accessing the capital market due to external factors beyond our control such as volatility in the equity markets for emerging biotechnology companies and general economic and market conditions both in the United States and abroad. We cannot be certain that we will be able to obtain financing on terms acceptable to us, or at all. Our failure to obtain adequate and timely funding will materially adversely affect our business and our ability to develop our technology and ZFP Therapeutic products. Furthermore, any sales of additional equity securities may result in dilutions to our stockholders and any debt financing may include business and financial covenants that restricts our operations.

We are at the development phase of operations and may not succeed or become profitable.

We began operations in 1995 and are in the early phases of ZFP Therapeutic product development, and we have incurred significant losses since inception. To date, our revenues have been generated from collaboration agreements, other collaborations in non-therapeutic applications of our technology, federal government research grants and grants awarded by research foundations. Our focus on higher-value therapeutic product development and related

collaboration requires us to incur substantial expenses associated with product development. In addition, the preclinical or clinical failure of any single product may have a significant effect on the actual or perceived value of our stock. Our business is subject to all of the risks inherent in the development of a new technology, which includes the need to:

- attract and retain qualified scientific and technical staff and management, particularly scientific staff with expertise to develop our early-stage technology into therapeutic products;
- obtain sufficient capital to support the expense of developing our technology platform and developing, testing and commercializing products;
- develop a market for our products; and
- successfully transition from a company with a research focus to a company capable of supporting commercial activities.

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## Risks Relating to our Relationships with Collaborators and Strategic Partners

If conflicts arise between us and our collaborators or strategic partners, these parties may act in their self-interest, which may limit our ability to implement our strategies.

If conflicts arise between our corporate or academic collaborators or strategic partners and us, the other party may act in its self-interest, which may limit our ability to implement our strategies. Some of our academic collaborators and strategic partners are conducting multiple product development efforts within each area that is the subject of the collaboration with us. Our collaborators or strategic partners, however, may develop, either alone or with others, products in related fields that are competitive with the products or potential products that are the subject of these collaborations. Competing products, either developed by the collaborators or strategic partners or to which the collaborators or strategic partners have rights, may result in the withdrawal of partner support for our product candidates.

Some of our collaborators or strategic partners could also become our competitors in the future. Our collaborators or strategic partners could develop competing products, preclude us from entering into collaborations with their competitors, fail to obtain timely regulatory approvals, terminate their agreements with us prematurely, or fail to devote sufficient resources to the development and commercialization of products. Any of these developments could harm our product development efforts.

Our collaborators and strategic partners may control aspects of our clinical trials, which could result in delays and other obstacles in the commercialization of our proposed products.

For some programs, we depend on third party collaborators and strategic partners to design and conduct our clinical trials. As a result, we may not be able to conduct these programs in the manner or on the time schedule we currently contemplate, which may negatively impact our business operations. In addition, if any of these collaborators or strategic partners withdraws support for our programs or proposed products or otherwise impair their development; our business could be negatively affected.

In January 2012, we entered into a collaborative agreement with Shire, pursuant to which we are engaging in a joint program with Shire to research, develop and commercialize human therapeutics and diagnostics for hemophilia, Huntington's disease and other monogenic diseases based on our ZFP technology. In September 2015, we amended the Shire agreement to restructure the collaboration, under which Shire will retain the rights to develop a ZFP Therapeutic for Huntington's disease and another target yet to be named, while returning the rights to us for the development, clinical testing and commercialization of ZFP Therapeutics for hemophilia A and B. Under the amended agreement, we will provide certain target feasibility activities and upon Shire's request, certain research activities under a research plan, agreed upon by both companies. Shire is responsible for clinical development and commercialization of products generated from the research program from and after the acceptance of an IND or CTA for the product.

In addition, in January 2014 we entered into a collaborative agreement with Biogen for the clinical development and commercialization of therapeutics based on our ZFP technology for hemoglobinopathies, including beta-thalassemia and SCD. Under the agreement, we are responsible for all discovery, research and development activities through the first human clinical trial for the first ZFP Therapeutic developed for the treatment of beta-thalassemia. In the SCD program, both parties are responsible for research and development activities through the submission of an IND.

Under the agreement with Biogen, they have control and broad discretion over all or certain aspects of the clinical development and commercialization of any product developed under the agreement, and we will have little, if any, influence on how these programs will be conducted. Our lack of control over the clinical development in our agreement with Biogen could cause delays or other difficulties in the development and commercialization of our



product candidates, which may prevent us from completing the intended IND filings in a timely fashion and receiving any milestone, royalty payments and other benefits under the agreement. In addition, under their respective agreement(s), Biogen and Shire have certain rights to terminate the agreements by providing us with advance notices, therefore, the actual milestone payments that we may receive under these agreements may be lower than the full amounts stated above.

Our collaborators or strategic partners may decide to adopt alternative technologies or may be unable to develop commercially viable products with our technology, which would negatively impact our revenues and our strategy to develop these products.

Our collaborators or strategic partners may adopt alternative technologies, which could decrease the marketability of ZFP technology. Additionally, because many of our collaborators or strategic partners are likely to be working on more than one development project, they could choose to shift their resources to projects other than those they are working on with us. If they do so, this would delay our ability to test our technology and would delay or terminate the development of potential products based on our ZFP technology. Further, our collaborators and strategic partners may elect not to develop products arising out of our collaborative and strategic partnering arrangements or to devote sufficient resources to the development, manufacturing, marketing or sale of these products. In September 2015, we amended our agreement with Shire pursuant to which Shire will no longer continue the clinical development of our ZFP Therapeutics for hemophilia A and B. As a result, we intend to develop these programs either ourselves or seek the support of other partners or collaborators. We may not have sufficient resources and expertise to develop these programs by

ourselves, and we may not be able to identify a suitable partner or negotiate a favorable collaboration agreement to allow us to continue the development of these programs. If any of these events occur, we may not be able to develop our technologies or commercialize our products.

If we do not successfully commercialize ZFP-based research reagents, ZFP-modified cell lines for commercial protein production, or ZFP-engineered transgenic animals under our license agreement with Sigma-Aldrich Corporation or ZFP-based agricultural products with Dow AgroSciences, or if Sigma-Aldrich Corporation or Dow AgroSciences terminates our agreements, our ability to generate revenue under these license agreements may be limited.

In July 2007, we entered into a license agreement with Sigma to collaborate in the application and development of ZFP-based products for use in the laboratory research reagents markets. The agreement provides Sigma with access to our ZFP technology and the exclusive right to use our ZFP technology to develop and commercialize products for use as research reagents and to offer services in related research fields. Under the agreement, Sigma has exclusive rights to develop and distribute ZFP-modified cell lines for commercial production of protein pharmaceuticals and, certain ZFP-engineered transgenic animals for commercial applications. In addition, under our license agreement with DAS relating to plant agriculture, DAS has the exclusive right to develop agricultural products using our ZFP technology in plant cells, plants or plant cell cultures. Both Sigma and DAS have the right to sublicense our technology in their respective areas. In addition to upfront payments, we may also receive additional license fees, shared sublicensing revenues, royalty payments and milestone payments depending on the success of the development and commercialization of the licensed products and services covered under both agreements. The commercial milestones and royalties are typically based upon net sales of licensed products.

We cannot be certain that we or our collaboration partners will succeed in the development of commercially viable products in these fields of use, and there is no guarantee that we or our collaboration partners will achieve the milestones set forth in the respective license agreements. To the extent we or our collaboration partners do not succeed in developing and commercializing products or if we or our collaboration partners fail to achieve such milestones, our revenues and benefits under the license agreements will be limited. In addition, the respective license agreements may be terminated by Sigma and DAS at any time by providing us with a 90-day notice. In the event Sigma or DAS decides to terminate the license agreements, our ability to generate revenue under such license agreements will cease.

Our collaborations with outside scientists may be subject to change, which could limit our access to their expertise.

We work with scientific advisors and collaborators at academic research institutions. These scientists are not our employees and may have other commitments that would limit their availability to us. Although our scientific advisors generally agree not to do competing work, if a conflict of interest between their work for us and their work for another entity arises, we may lose their services. Although our scientific advisors and academic collaborators sign agreements not to disclose our confidential information, it is possible that some of our valuable proprietary knowledge may become publicly known through them, which may cause competitive harm to our business.

#### Risks Relating to our Intellectual Property and Business Operation

Because it is difficult and costly to protect our proprietary rights, and third parties have filed patent applications that are similar to ours, we cannot ensure the proprietary protection of our technologies and products.

Our commercial success will depend in part on obtaining patent protection of our technology and successfully defending any of our patents that may be challenged. The patent positions of pharmaceutical and biotechnology companies can be highly uncertain and can involve complex legal and factual questions. No consistent policy regarding the breadth of claims allowed in biotechnology patents has emerged to date. Accordingly, we cannot predict the breadth of claims allowed in patents we own or license.

We are a party to various license agreements that give us rights under specified patents and patent applications. Our current licenses, as our future licenses frequently will, contain performance obligations. If we fail to meet those obligations, the licenses could be terminated. If we are unable to continue to license these technologies on commercially reasonable terms, or at all, we may be forced to delay or terminate our product development and research activities.

With respect to our present and any future sublicenses, since our rights derive from those granted to our sublicensor, we are subject to the risk that our sublicensor may fail to perform its obligations under the master license or fail to inform us of useful improvements in, or additions to, the underlying intellectual property owned by the original licensor.

We are unable to exercise the same degree of control over intellectual property that we license from third parties as we exercise over our internally developed intellectual property. We do not control the prosecution of certain of the patent applications that we license from third parties; therefore, the patent applications may not be prosecuted as we desire or in a timely manner.

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

- we or our licensors were the first to make the inventions covered by each of our pending patent applications;
- we or our licensors were the first to file patent applications for these inventions;
- the patents of others will not have an adverse effect on our ability to do business;
- others will not independently develop similar or alternative technologies or reverse engineer any of our products, processes or technologies;
- any of our pending patent applications will result in issued patents;
- any patents issued or licensed to us or our collaborators or strategic partners will provide a basis for commercially viable products or will provide us with any competitive advantages;
- any patents issued or licensed to us will not be challenged and invalidated by third parties; or
- we will develop additional products, processes or technologies that are patentable.

Others have filed and in the future are likely to file patent applications that are similar to ours. We are aware that there are academic groups and other companies that are attempting to develop technology that is based on the use of zinc finger, TALE, CRISPR/Cas9 and other DNA-binding proteins, and that these groups and companies have filed patent applications. Several patents have been issued, although we have no current plans to use the associated inventions. If these or other patents issue, it is possible that the holder of any patent or patents granted on these applications may bring an infringement action against our collaborators, strategic partners, or us claiming damages and seeking to enjoin commercial activities relating to the affected products and processes. The costs of litigating the claim could be substantial. Moreover, we cannot predict whether we, our collaborators, or strategic partners would prevail in any actions. In addition, if the relevant patent claims were upheld as valid and enforceable and our products or processes were found to infringe the patent or patents, we could be prevented from making, using, or selling the relevant product or process unless we could obtain a license or were able to design around the patent claims. We can give no assurance that such a license would be available on commercially reasonable terms, or at all, or that we would be able to successfully design around the relevant patent claims. There may be significant litigation in the genomics industry regarding patent and other intellectual property rights, which could subject us to litigation. If we become involved in litigation, it could consume a substantial portion of our managerial and financial resources.

We rely on trade secrets to protect technology where we believe patent protection is not appropriate or obtainable. Trade secrets, however, are difficult to protect. While we require employees, academic collaborators and consultants to enter into confidentiality agreements, we may not be able to adequately protect our trade secrets or other proprietary information or enforce these confidentiality agreements.

Our collaborators, strategic partners, and scientific advisors have rights to publish data and information in which we may have rights. If we cannot maintain the confidentiality of our technology and other confidential information in connection with our collaborations and strategic partnerships, then we may not be able to receive patent protection or protect our proprietary information.

If we use biological and hazardous materials in a manner that causes injury or violates laws, we may be liable for damages.

Our research and development activities involve the controlled use of potentially harmful biological materials as well as hazardous materials, chemicals, and various radioactive compounds typically employed in molecular and cellular biology. We routinely use cells in culture and gene delivery vectors, and we employ small amounts of radioisotopes in trace experiments. Although we maintain up-to-date licensing and training programs, we cannot completely eliminate the risk of accidental contamination or injury from the use, storage, handling, or disposal of these materials. In the event of contamination or injury, we could be held liable for damages that result, and any liability could exceed our resources. We currently carry insurance covering certain claims arising from our use of these materials. However, if we are unable to maintain our insurance coverage at a reasonable cost and with adequate coverage, our insurance may not cover any liability that may arise. We are subject to federal, state, and local laws and regulations governing the use, storage, handling, and disposal of these materials and specified waste products. To date, we have not experienced

significant costs in complying with regulations regarding the use of these materials.

Failure to attract, retain, and motivate skilled personnel and cultivate key academic collaborations will delay our product development programs and our research and development efforts.

Our success depends on our continued ability to attract, retain, and motivate highly qualified management and scientific personnel and our ability to develop and maintain important relationships with leading research and academic institutions and scientists. Competition for skilled and qualified personnel and academic and other research collaborations is intense. We have experienced a rate of employee turnover that we believe is typical of emerging biotechnology companies. If we lose the services of

personnel with the necessary skills, including the members of our senior management team, it could significantly impede the achievement of our research and development objectives. If we fail to negotiate additional acceptable collaborations with academic and other research institutions and scientists, or if our existing collaborations are unsuccessful, our ZFP Therapeutic development programs may be delayed or may not succeed.

#### Risks Relating to our Common Stock and Corporate Organization

Our stock price has been volatile and may continue to be volatile, which could result in substantial losses for investors.

During the three months ended September 30, 2015, the closing price of our common stock, as reported by the NASDAQ Global Select Market, ranged from a low of \$5.64 to high of \$10.86. During the fiscal year ended December 31, 2014, our common stock price fluctuated, ranging from a low of \$9.85 to a high of \$23.86. Volatility in our common stock could cause stockholders to incur substantial losses. An active public market for our common stock may not be sustained, and the market price of our common stock may continue to be highly volatile. The market price of our common stock has fluctuated significantly in response to various factors, some of which are beyond our control, including but not limited to the following:

- announcements by us or collaborators providing updates on the progress or development status of ZFP Therapeutics;
  - data from clinical trials;
  - initiation or termination of clinical trials;
  - changes in market valuations of similar companies;
    - overall market and economic conditions, including the equity markets for emerging biotechnology companies;
  - deviations in our results of operations from the guidance given by us;
  - announcements by us or our competitors of new or enhanced products, technologies or services or significant contracts, acquisitions, strategic relationships, joint ventures or capital commitments;
  - announcement of changes in business and operations by our collaborators and partners;
  - regulatory developments;
  - additions or departures of key personnel;
  - future sales of our common stock or other securities by us, management or directors, liquidation of institutional funds that comprised large holdings of our stock;
  - decreases in our cash balances; and
  - changes, by one or more of Sangamo's security analysts, in recommendations, ratings or coverage of our stock.
- Our stock price is also influenced by public perception of gene therapy and government regulation of potential products.

Reports of serious adverse events in a retroviral gene transfer trial for infants with X-linked severe combined immunodeficiency (X-linked SCID) in France and subsequent FDA actions putting related trials on hold in the United States had a significant negative impact on the public perception and stock price of certain companies involved in gene therapy. Stock prices of these companies declined whether or not the specific company was involved with retroviral gene transfer for the treatment of infants with X-linked SCID, or whether the specific company's clinical trials were placed on hold in connection with these events. Other potential adverse events in the field of gene therapy may occur in the future that could result in greater governmental regulation of our potential products and potential regulatory delays relating to the testing or approval of our potential products. These external events may have a negative impact on public perception of our business, which could cause our stock price to decline.

Anti-takeover provisions in our certificate of incorporation and Delaware law could make an acquisition of the Company more difficult and could prevent attempts by our stockholders to remove or replace current management.

Anti-takeover provisions of Delaware law and in our certificate of incorporation and our bylaws may discourage, delay or prevent a change in control of our company, even if a change in control would be beneficial to our stockholders. In addition, these provisions may frustrate or prevent any attempts by our stockholders to replace or remove our current management by making it more difficult for stockholders to replace members of our board of directors. In particular, under our certificate of incorporation our board of directors may issue up to 5,000,000 shares of preferred stock with rights and privileges that might be senior to our common stock, without the consent of the holders of the common stock. Moreover, without any further vote or action on the part of the stockholders, the board of directors would have the authority to determine the price, rights, preferences, privileges, and restrictions of the preferred stock. This preferred stock, if it is ever issued, may have preference over, and harm the rights of, the holders of common stock.

Although the issuance of this preferred stock would provide us with flexibility in connection with possible acquisitions and other corporate purposes, this issuance may make it more difficult for a third party to acquire a majority of our outstanding voting stock.

Similarly, our authorized but unissued common stock is available for future issuance without stockholder approval.

In addition, our bylaws:

- state that stockholders may not act by written consent but only at a stockholders' meeting;
- establish advance notice requirements for nominations for election to the board of directors or proposing matters that can be acted upon at stockholders' meetings; and
- prohibit stockholders from calling a special meeting of stockholders.

We are also subject to Section 203 of the Delaware General Corporation Law, which provides, subject to certain exceptions, that if a person acquires 15% of our voting stock, the person is an "interested stockholder" and may not engage in "business combinations" with us for a period of three years from the time the person acquired 15% or more of our voting stock. The application of Section 203 may, in some circumstances, deter or prevent a change in control of our company even when such change may be beneficial to our stockholders.



ITEM 6. EXHIBITS

(a) Exhibits:

- 10.1† Amended and Restated Collaboration and License Agreement, dated September 1, 2015, between the Company and Shire
- 31.1 Rule 13a — 14(a) Certification by President and Chief Executive Officer
- 31.2 Rule 13a — 14(a) Certification by Principal Financial and Accounting Officer
- 32.1 Certification Pursuant to 18 U.S.C. Section 1350
- 101.INS XBRL Instance Document
- 101.SCH XBRL Taxonomy Extension Schema Document
- 101.CAL XBRL Taxonomy Extension Calculation Linkbase Document
- 101.DEF XBRL Taxonomy Extension Definition Linkbase Document
- 101.LAB XBRL Taxonomy Extension Label Linkbase Document
- 101.PRE XBRL Taxonomy Extension Presentation Linkbase Document

†Confidential treatment has been requested for certain information contained in this document. Such information has been omitted and filed separately with the Securities and Exchange Commission.

SIGNATURES

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

Dated: October 30, 2015

SANGAMO BIOSCIENCES, INC.

/s/ H. WARD WOLFF

H. Ward Wolff

Executive Vice President and Chief Financial Officer  
(Principal Financial and Accounting Officer)