

SANGAMO BIOSCIENCES INC

Form 10-Q

November 04, 2013

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**UNITED STATES**

**SECURITIES AND EXCHANGE COMMISSION**

**Washington, D.C. 20549**

**FORM 10-Q**

**(Mark One)**

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

**For the quarterly period ended September 30, 2013**

**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**  
**(State or other jurisdiction of**  
**incorporation or organization)**  
**501 Canal Blvd**  
**Richmond, California 94804**  
**(Address of principal executive offices)**  
**(510) 970-6000**  
**(Registrant's telephone number, including area code)**

**68-0359556**  
**(IRS Employer**  
**Identification No.)**

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 31, 2013, 61,797,202 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 view 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.

**Table of Contents****PART I. FINANCIAL INFORMATION****ITEM 1. FINANCIAL STATEMENTS****SANGAMO BIOSCIENCES, INC.****CONDENSED CONSOLIDATED BALANCE SHEETS****(In thousands, except share and per share amounts)**

|                                                                                                                                                                                 | <b>September 30,<br/>2013<br/>(unaudited)</b> | <b>December 31,<br/>2012</b> |
|---------------------------------------------------------------------------------------------------------------------------------------------------------------------------------|-----------------------------------------------|------------------------------|
| <b>Assets</b>                                                                                                                                                                   |                                               |                              |
| Current assets:                                                                                                                                                                 |                                               |                              |
| Cash and cash equivalents                                                                                                                                                       | \$ 80,309                                     | \$ 21,679                    |
| Marketable securities                                                                                                                                                           | 39,913                                        | 41,868                       |
| Interest receivable                                                                                                                                                             | 208                                           | 190                          |
| Accounts receivable                                                                                                                                                             | 4,880                                         | 4,129                        |
| Other current assets                                                                                                                                                            | 149                                           | 203                          |
| Prepaid expenses                                                                                                                                                                | 684                                           | 296                          |
| Total current assets                                                                                                                                                            | 126,143                                       | 68,365                       |
| Marketable securities, non-current                                                                                                                                              | 12,690                                        | 12,584                       |
| Property and equipment, net                                                                                                                                                     | 1,494                                         | 1,543                        |
| Other assets                                                                                                                                                                    | 39                                            | 41                           |
| Total assets                                                                                                                                                                    | \$ 140,366                                    | \$ 82,533                    |
| <b>Liabilities and stockholders' equity</b>                                                                                                                                     |                                               |                              |
| Current liabilities:                                                                                                                                                            |                                               |                              |
| Accounts payable and accrued liabilities                                                                                                                                        | \$ 2,665                                      | \$ 4,013                     |
| Accrued compensation and employee benefits                                                                                                                                      | 2,039                                         | 2,473                        |
| Deferred revenues                                                                                                                                                               | 2,300                                         | 2,304                        |
| Total current liabilities                                                                                                                                                       | 7,004                                         | 8,790                        |
| Deferred revenues, non-current                                                                                                                                                  | 7,221                                         | 8,847                        |
| Total liabilities                                                                                                                                                               | \$ 14,225                                     | 17,637                       |
| Commitments and contingencies                                                                                                                                                   |                                               |                              |
| Stockholders' equity:                                                                                                                                                           |                                               |                              |
| Common stock, \$0.01 par value; 80,000,000 shares authorized, 61,420,655 and 53,058,525 shares issued and outstanding at September 30, 2013 and December 31, 2012, respectively | \$ 614                                        | 531                          |
| Additional paid-in capital                                                                                                                                                      | 419,490                                       | 339,848                      |

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|                                            |            |           |
|--------------------------------------------|------------|-----------|
| Accumulated deficit                        | (293,989)  | (275,509) |
| Accumulated other comprehensive income     | 26         | 26        |
| Total stockholders' equity                 | 126,141    | 64,896    |
| Total liabilities and stockholders' equity | \$ 140,366 | \$ 82,533 |

*See accompanying notes.*

Table of Contents**SANGAMO BIOSCIENCES, INC.****CONDENSED CONSOLIDATED STATEMENTS OF OPERATIONS****(In thousands, except per share amounts)****(Unaudited)**

|                                                               | <b>Three months<br/>ended<br/>September 30,</b> |             | <b>Nine months ended<br/>September 30,</b> |             |
|---------------------------------------------------------------|-------------------------------------------------|-------------|--------------------------------------------|-------------|
|                                                               | <b>2013</b>                                     | <b>2012</b> | <b>2013</b>                                | <b>2012</b> |
| <b>Revenues:</b>                                              |                                                 |             |                                            |             |
| Collaboration agreements                                      | \$ 4,825                                        | \$ 4,190    | \$ 15,065                                  | \$ 9,665    |
| Research grants                                               | 882                                             | 717         | 2,199                                      | 3,058       |
| Total revenues                                                | 5,707                                           | 4,907       | 17,264                                     | 12,723      |
| <b>Operating expenses:</b>                                    |                                                 |             |                                            |             |
| Research and development                                      | 8,703                                           | 7,570       | 26,201                                     | 22,427      |
| General and administrative                                    | 3,163                                           | 3,139       | 9,595                                      | 9,125       |
| Total operating expenses                                      | 11,866                                          | 10,709      | 35,796                                     | 31,552      |
| Loss from operations                                          | (6,159)                                         | (5,802)     | (18,532)                                   | (18,829)    |
| Interest and other income, net                                | 14                                              | 12          | 52                                         | 43          |
| Net loss                                                      | \$ (6,145)                                      | \$ (5,790)  | \$ (18,480)                                | \$ (18,786) |
| Basic and diluted net loss per share                          | \$ (0.11)                                       | \$ (0.11)   | \$ (0.34)                                  | \$ (0.36)   |
| Shares used in computing basic and diluted net loss per share | 54,786                                          | 52,768      | 54,013                                     | 52,664      |

*See accompanying notes.*

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**SANGAMO BIOSCIENCES, INC.**

**CONSOLIDATED STATEMENTS OF COMPREHENSIVE LOSS**

**(In thousands)**

**(Unaudited)**

|                                                             | <b>Three months<br/>ended<br/>September 30,</b> |             | <b>Nine months ended<br/>September 30,</b> |             |
|-------------------------------------------------------------|-------------------------------------------------|-------------|--------------------------------------------|-------------|
|                                                             | <b>2013</b>                                     | <b>2012</b> | <b>2013</b>                                | <b>2012</b> |
| Net loss                                                    | \$ (6,145)                                      | \$ (5,790)  | \$ (18,480)                                | \$ (18,786) |
| Changes in unrealized loss on available-for-sale securities | 27                                              | 22          |                                            | 3           |
| Comprehensive loss                                          | \$ (6,118)                                      | \$ (5,768)  | \$ (18,480)                                | \$ (18,783) |

*See accompanying notes.*



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|                                                                             | <b>Nine months ended<br/>September 30,</b> |             |
|-----------------------------------------------------------------------------|--------------------------------------------|-------------|
|                                                                             | <b>2013</b>                                | <b>2012</b> |
| <b>Operating Activities:</b>                                                |                                            |             |
| Net loss                                                                    | \$ (18,480)                                | \$ (18,786) |
| Adjustments to reconcile net loss to net cash used in operating activities: |                                            |             |
| Depreciation and amortization                                               | 445                                        | 500         |
| Amortization of premium / discount on marketable securities                 | 625                                        | 692         |
| Stock-based compensation                                                    | 4,098                                      | 4,011       |
| Changes in operating assets and liabilities:                                |                                            |             |
| Interest receivable                                                         | (18)                                       | 20          |
| Accounts receivable                                                         | (696)                                      | (2,585)     |
| Prepaid expenses and other assets                                           | (387)                                      | (364)       |
| Accounts payable and accrued liabilities                                    | (1,348)                                    | (3,770)     |
| Accrued compensation and employee benefits                                  | (434)                                      | 159         |
| Deferred revenues                                                           | (1,630)                                    | 11,818      |
| Net cash used in operating activities                                       | (17,825)                                   | (8,305)     |
| <b>Investing Activities:</b>                                                |                                            |             |
| Purchases of investments                                                    | (37,161)                                   | (70,570)    |
| Maturities of investments                                                   | 38,384                                     | 75,155      |
| Purchases of property and equipment                                         | (395)                                      | (363)       |
| Net cash provided by investing activities                                   | 828                                        | 4,222       |
| <b>Financing Activities:</b>                                                |                                            |             |
| Proceeds from public offering, net of issuance costs                        | 69,492                                     |             |
| Proceeds from issuance of common stock                                      | 6,135                                      | 1,041       |
| Net cash provided by financing activities                                   | 75,627                                     | 1,041       |
| Net increase / (decrease) in cash and cash equivalents                      | 58,630                                     | (3,042)     |
| Cash and cash equivalents, beginning of period                              | 21,679                                     | 16,766      |
| Cash and cash equivalents, end of period                                    | \$ 80,309                                  | \$ 13,724   |

*See accompanying notes.*



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**SANGAMO BIOSCIENCES, INC.**

**NOTES TO CONDENSED CONSOLIDATED FINANCIAL STATEMENTS**

**September 30, 2013**

**(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, 2013 are not necessarily indicative of the results that may be expected for the year ending December 31, 2013. The condensed consolidated balance sheet data at December 31, 2012 were derived from the audited consolidated financial statements included in Sangamo's Form 10-K for the year ended December 31, 2012, as filed with the SEC. These financial statements should be read in conjunction with the financial statements and footnotes thereto for the year ended December 31, 2012, included in Sangamo's Form 10-K, as filed with the SEC.

***Use of Estimates and Classifications***

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, 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 agreement 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 agreement entered into with Shire AG (Shire) in January 2012 was 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 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.

## ***Recent Accounting Pronouncement***

In February 2013, the FASB issued ASU No. 2013-02, Comprehensive Income (Topic 220): Reporting of Amounts Reclassified Out of Accumulated Other Comprehensive Income (ASU 2013-02). This newly issued accounting standard requires an entity to provide information about the amounts reclassified out of accumulated other comprehensive income by component. This ASU is effective for reporting periods beginning after December 15, 2012. We adopted this standard in the first quarter of 2013 and the adoption of this standard did not have an impact on our financial position or results of operations.

## **NOTE 2 INVESTMENTS AND FAIR VALUE MEASUREMENT**

### ***Investments***

Sangamo 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 based on quoted market prices, with the unrealized holding gains and losses included in accumulated other comprehensive income. Marketable securities which 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.



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The table below summarizes the Company's available-for-sale securities (in thousands):

|                                                          | <b>Amortized<br/>Cost</b> | <b>Gross<br/>Unrealized<br/>Gains</b> | <b>Gross<br/>Unrealized<br/>(Losses)</b> | <b>Estimated<br/>Fair Value</b> |
|----------------------------------------------------------|---------------------------|---------------------------------------|------------------------------------------|---------------------------------|
| <b>September 30, 2013</b>                                |                           |                                       |                                          |                                 |
| Cash equivalents:                                        |                           |                                       |                                          |                                 |
| Money market funds                                       | \$ 9,758                  | \$                                    | \$                                       | \$ 9,758                        |
| Total                                                    | 9,758                     |                                       |                                          | 9,758                           |
| Available-for-sale securities:                           |                           |                                       |                                          |                                 |
| U.S. government sponsored entity debt securities         | 52,577                    | 26                                    |                                          | 52,603                          |
| Total                                                    | 52,577                    | 26                                    |                                          | 52,603                          |
| Total cash equivalents and available-for-sale securities | \$ 62,335                 | \$ 26                                 | \$                                       | \$ 62,361                       |
| <b>December 31, 2012</b>                                 |                           |                                       |                                          |                                 |
| Cash equivalents:                                        |                           |                                       |                                          |                                 |
| U.S. government sponsored entity debt securities         | \$ 2,997                  | \$                                    | \$                                       | \$ 2,997                        |
| Money market funds                                       | 15,839                    |                                       |                                          | 15,839                          |
| Total                                                    | 18,836                    |                                       |                                          | 18,836                          |
| Available-for-sale securities:                           |                           |                                       |                                          |                                 |
| U.S. government sponsored entity debt securities         | 54,426                    | 26                                    |                                          | 54,452                          |
| Total                                                    | 54,426                    | 26                                    |                                          | 54,452                          |
| Total cash equivalents and available-for-sale securities | \$ 73,262                 | \$ 26                                 | \$                                       | \$ 73,288                       |

As of September 30, 2013, none of the available-for-sale securities held by the Company had material unrealized losses and there were no realized losses for the three and nine months ended September 30, 2013. The Company had no other-than-temporary impairments of available-for-sale securities for the three and nine months ended September 30, 2013 or the twelve months ended December 31, 2012.

***Fair Value Measurement***

The Company measures certain financial assets at fair value on a recurring basis, including cash equivalents and available-for sale-securities. The fair value of these assets 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;

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).

The Company generally classifies its available-for-sale debt instruments 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, and matrix pricing as well as model processes. These models are proprietary to the pricing providers or brokers. These valuation models 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.



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The fair value measurements of our cash equivalents and available-for-sale marketable securities are identified at the following levels within the fair value hierarchy (in thousands):

| <b>September 30, 2013</b>                                       |                  |                 |                  |                |
|-----------------------------------------------------------------|------------------|-----------------|------------------|----------------|
| <b>Fair Value Measurements</b>                                  |                  |                 |                  |                |
|                                                                 | <b>Total</b>     | <b>Level 1</b>  | <b>Level 2</b>   | <b>Level 3</b> |
| <b>Assets:</b>                                                  |                  |                 |                  |                |
| <b>Cash equivalents:</b>                                        |                  |                 |                  |                |
| Money market funds                                              | \$ 9,758         | \$ 9,758        | \$               | \$             |
| <b>Total</b>                                                    | <b>9,758</b>     | <b>9,758</b>    |                  |                |
| <b>Available-for-sale securities:</b>                           |                  |                 |                  |                |
| U.S. government sponsored entity debt securities                | 52,603           |                 | 52,603           |                |
| <b>Total</b>                                                    | <b>52,603</b>    |                 | <b>52,603</b>    |                |
| <b>Total cash equivalents and available-for-sale securities</b> | <b>\$ 62,361</b> | <b>\$ 9,758</b> | <b>\$ 52,603</b> | <b>\$</b>      |

| <b>December 31, 2012</b>                                        |                  |                  |                  |                |
|-----------------------------------------------------------------|------------------|------------------|------------------|----------------|
| <b>Fair Value Measurements</b>                                  |                  |                  |                  |                |
|                                                                 | <b>Total</b>     | <b>Level 1</b>   | <b>Level 2</b>   | <b>Level 3</b> |
| <b>Assets:</b>                                                  |                  |                  |                  |                |
| <b>Cash equivalents:</b>                                        |                  |                  |                  |                |
| U.S. government sponsored entity debt securities                | \$ 2,997         | \$               | \$ 2,997         | \$             |
| Money market funds                                              | 15,839           | 15,839           |                  |                |
| <b>Total</b>                                                    | <b>18,836</b>    | <b>15,839</b>    | <b>2,997</b>     |                |
| <b>Available-for-sale securities:</b>                           |                  |                  |                  |                |
| U.S. government sponsored entity debt securities                | 54,452           |                  | 54,452           |                |
| <b>Total</b>                                                    | <b>54,452</b>    |                  | <b>54,452</b>    |                |
| <b>Total cash equivalents and available-for-sale securities</b> | <b>\$ 73,288</b> | <b>\$ 15,839</b> | <b>\$ 57,449</b> | <b>\$</b>      |

**NOTE 3 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 options, which are anti-dilutive. The total stock options excluded from consideration in the calculation of diluted net loss per share for the nine months ended September 30, 2013 and 2012 were 7,853,936 and 8,075,898, respectively.

## **NOTE 4 MAJOR CUSTOMERS, PARTNERSHIPS AND STRATEGIC ALLIANCES**

### **Collaboration Agreements**

#### ***Collaboration and License Agreement with Shire AG in Human Therapeutics and Diagnostics***

In January 2012, the Company entered into a collaboration and license agreement (the Agreement) with Shire AG (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 zinc finger DNA-binding proteins (ZFP) technology. Under the Agreement, the Company and Shire may 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. In June 2012, Shire selected a fifth gene target for the development of a ZFP therapeutic for Huntington's disease, an inherited neurodegenerative disease for which there are currently no therapies available to slow the disease progression. Shire has the right, subject to certain limitations, to designate two additional gene targets. Pursuant to the 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. The initial research term of the Agreement is six years and is subject to extensions upon mutual agreement and under other specified circumstances.

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Under the terms of the Agreement, the Company is responsible for all research activities through the submission of an Investigative New Drug Application (IND) or European Clinical Trial Application (CTA), while 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. Shire reimburses Sangamo for its internal and external research program-related costs.

The Company received an upfront license fee of \$13.0 million. The Company will also be eligible to receive up to \$213.5 million of contingent payments for each gene target if specified research, regulatory, clinical development, commercialization and sales milestone events occur, including payments for each gene target through the acceptance of an IND or CTA submission totaling \$8.5 million. The Company will also be eligible to receive royalty payments that are a tiered double-digit percentage of net sales of licensed product sold by Shire or its sublicensees developed under the collaboration, if any. To date, no products have been approved and therefore no royalty fees have been earned under the Agreement with Shire.

All contingent payments under the Agreement, when earned, will be non-refundable and non-creditable. The Company has evaluated the contingent payments under the Agreement with Shire 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, revenue for the achievement of milestones that are determined to be substantive will be recognized in its entirety in the period when the milestone is 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 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 Shire apart from the research services to be performed pursuant to the Agreement. As a result, the Company will recognize revenue from the upfront payment on a straight-line basis over a six-year initial research term during which the Company will perform research services. As of September 30, 2013, the Company has deferred revenue of \$9.5 million related to this Agreement.

Revenues recognized under the agreement with Shire for the three and nine months ended September 30, 2013 and September 30, 2012, were as follows (in thousands):

|                                         | <b>Three months ended</b> |             | <b>Nine months ended</b> |             |
|-----------------------------------------|---------------------------|-------------|--------------------------|-------------|
|                                         | <b>September 30,</b>      |             | <b>September 30,</b>     |             |
|                                         | <b>2013</b>               | <b>2012</b> | <b>2013</b>              | <b>2012</b> |
| Revenue related to Shire Collaboration: |                           |             |                          |             |
| Amortization of upfront fee             | \$ 542                    | \$ 542      | \$ 1,625                 | \$ 1,444    |
| Research services                       | 3,968                     | 2,675       | 11,340                   | 4,747       |
| Total                                   | \$ 4,510                  | \$ 3,217    | \$ 12,965                | \$ 6,191    |

Related costs and expenses incurred under the Shire agreement were \$3.5 million and \$2.2 million during the three months ended September 30, 2013 and September 30, 2012, respectively and \$10.3 million and \$3.8 million during the nine months ended September 30, 2013 and September 30, 2012, respectively.

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

In July 2007, Sangamo entered into a license agreement (the Agreement) with Sigma-Aldrich Corporation (Sigma). Under the Agreement, Sangamo agreed to provide Sigma with access to our 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), a wholly-owned subsidiary of Dow Chemical Corporation. Under the 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 the ZFP manufacturing technology to Sigma.

In October 2009, Sangamo expanded its Agreement with Sigma. In addition to the original terms of the 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 Agreement, Sigma made upfront cash payment of \$20.0 million consisting of a \$4.9 million purchase of 636,133 shares of Sangamo common stock, valued at \$4.9 million, 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

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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 and sublicensing revenue and thereafter a reduced royalty rate of 10.5% 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. In April 2013, the Company recognized \$1.3 million in sublicense revenues under the agreement with Sigma.

Revenues recognized under the agreement with Sigma for the three and nine months ended September 30, 2013 and September 30, 2012, were as follows (in thousands):

|                                         | Three months ended<br>September 30,<br>20132012 |        | Nine months ended<br>September 30,<br>20132012 |          |
|-----------------------------------------|-------------------------------------------------|--------|------------------------------------------------|----------|
| Revenue related to Sigma Collaboration: |                                                 |        |                                                |          |
| Royalty revenues                        | \$ 201                                          | \$ 428 | \$ 711                                         | \$ 1,035 |
| License fee and milestone revenues      | 101                                             |        | 1,351                                          | 1,000    |
| Total                                   | \$ 302                                          | \$ 428 | \$ 2,062                                       | \$ 2,035 |

Related costs and expenses incurred under the Sigma agreement were \$0.1 million during both the three months ended September 30, 2013 and 2012. Related costs and expenses incurred under the Sigma agreement were \$0.1 million and \$0.3 million during the nine months ended September 30, 2013 and 2012, respectively.

***Agreement with Dow AgroSciences in Plant Agriculture***

In October 2005, Sangamo entered into an exclusive commercial license agreement (the Agreement) with DAS. Under the Agreement, Sangamo provides DAS with access to its 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 Agreement with DAS 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 Agreement with DAS to extend the period of reagent manufacturing services and research services through December 31, 2012.

The Agreement also provides for minimum sublicense 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 performance obligations with respect to the sublicensing activities to be conducted by DAS. DAS has the right to terminate the Agreement at any time; accordingly, the Company's actual sublicense fees over the term of the Agreement could be lower than \$25.3 million. In addition, each party may

terminate the Agreement upon an uncured material breach by the other party. In the event of any termination of the 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.

Revenues under the agreement were \$0.4 million during the three months ended September 30, 2012, and \$1.3 million during the nine months ended September 30, 2012. Related costs and expenses incurred under the agreement were \$0.1 million during the three months ended September 30, 2012, and \$0.4 million during the nine months ended September 30, 2012. There were no such revenues or related expenses during the three and nine months ended September 30, 2013.

### **Funding from Research Foundations**

#### ***California Institute for Regenerative Medicine***

In October 2009, the California Institute for Regenerative Medicine (CIRM), a State of California entity, granted a \$14.5 million Disease Team Research Award to develop an AIDS-related lymphoma therapy based on the application of ZFN gene editing technology in stem cells. The four year grant supports an innovative research project conducted by a multidisciplinary team of investigators, including investigators from the University of Southern California, City of Hope National Medical Center and Sangamo BioSciences. Sangamo expects to receive funding up to \$5.2 million from the total amount awarded based on expenses incurred for research and development efforts by Sangamo as prescribed in the agreement, and subject to its terms and conditions. The award is intended to substantially fund Sangamo's research and development efforts related to the agreement. The State of California has the

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right to receive, subject to the terms and conditions of the agreement between Sangamo and CIRM, payments from Sangamo resulting from sales of a commercial product resulting from research and development efforts supported by the grant, not to exceed two times the amount Sangamo receives in funding under the agreement with CIRM.

Revenues attributable to research and development performed under the CIRM grant agreement were \$0.5 million and \$0.3 million during the three months ended September 30, 2013 and 2012, respectively and \$ 1.2 million and \$0.9 million during the nine months ended September 30, 2013 and 2012, respectively. Related costs and expenses incurred under the CIRM agreement were \$0.5 million and \$0.3 million during the three months ended September 30, 2013 and 2012, respectively, and \$1.4 million and \$0.9 million during the nine months ended September 30, 2013 and 2012, respectively.

### ***CHDI Foundation, Inc.***

In April 2011, Sangamo entered into an agreement with the CHDI Foundation, Inc. (CHDI) to develop a novel therapeutic for Huntington's disease based on Sangamo's proprietary ZFP technology. Under the agreement with CHDI, and subject to its terms and conditions, CHDI paid the Company \$1.3 million, the total funds due under the agreement, over a period of one year which is intended to substantially fund the Company's research efforts related to the agreement. During 2012, the agreement was amended to extend the project through August 2012 and to increase total potential funding from \$1.3 million to \$2.1 million, plus reimbursement for certain direct expenses related to the project. The research grant from CHDI was completed in August 2012.

Revenues attributable to research and development performed under the CHDI collaboration agreement were \$0.3 million and \$1.1 million during the three and nine months ended September 30, 2012, respectively. Related costs and expenses incurred under the CHDI agreement were \$0.3 million and \$1.1 million during the three and nine months ended September 30, 2012, respectively. There were no revenues or related expenses during the three and nine months ended September 30, 2013.

### ***The Juvenile Diabetes Research Foundation International***

In October 2006, Sangamo entered into an agreement with the Juvenile Diabetes Research Foundation International (JDRF) to provide financial support for one of Sangamo's Phase 2 human clinical studies of the Company's product candidate SB-509, a ZFP Therapeutic that was in development for the treatment of diabetic neuropathy. In January 2010, JDRF and Sangamo amended the agreement and, subject to its terms and conditions, JDRF agreed to provide additional funding of up to \$3.0 million for a Phase 2b trial in diabetic neuropathy.

In October 2011, the Company announced of the termination of its SB-509 program. In March 2012, the Company received a final payment of \$0.8 million for work performed under the JDRF agreements. The Company does not expect to receive additional funding under these agreements.

Revenues attributable to research and development activities performed under the JDRF agreements were \$0 for the three months ended September 30, 2012 and \$0.8 million during the nine months ended September 30, 2012. There were no such revenues during the three and nine months ended September 30, 2013.

## **NOTE 5 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. Realization of deferred tax assets is dependent upon future earnings, if any, the timing and amount of which are uncertain based on the Company's history of losses. Accordingly, the 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.



**Table of Contents****NOTE 6 STOCK-BASED COMPENSATION**

The following table shows total stock-based compensation expense included in the condensed consolidated statement of operations for the three month and nine months ended September 30, 2013 and 2012 (in thousands):

|                                        | <b>Three months ended<br/>September 30,</b> |             | <b>Nine months ended<br/>September 30,</b> |             |
|----------------------------------------|---------------------------------------------|-------------|--------------------------------------------|-------------|
|                                        | <b>2013</b>                                 | <b>2012</b> | <b>2013</b>                                | <b>2012</b> |
| Costs and expenses:                    |                                             |             |                                            |             |
| Research and development               | \$ 732                                      | \$ 774      | \$ 2,140                                   | \$ 2,195    |
| General and administrative             | 659                                         | 630         | 1,958                                      | 1,816       |
| Total stock-based compensation expense | \$ 1,391                                    | \$ 1,404    | \$ 4,098                                   | \$ 4,011    |

**NOTE 7 STOCKHOLDERS EQUITY**

On September 23, 2013, Sangamo completed an underwritten public offering of its common stock, in which the Company sold an aggregate of 7,015,000 shares of its common stock, including 915,000 issued to the Underwriters pursuant to a 30-day overallotment option, at a public offering price of \$10.58 per share. The net proceeds to Sangamo from the sale of shares in this offering, after deducting underwriting discounts and commissions and other estimated offering expenses, were approximately \$69.5 million.

**NOTE 8 SUBSEQUENT EVENTS****Acquisition of Ceregene**

On August 23, 2013, Sangamo and its wholly-owned subsidiary CG Acquisition Sub, Inc., a Delaware corporation ( Merger Sub ), entered into an Agreement and Plan of Merger (the Merger Agreement ) with Ceregene, Inc., ( Ceregene ) and a stockholders representative. Pursuant to the Merger Agreement, the Company acquired all outstanding shares of Ceregene, a privately held biotechnology company focused on the development of adeno-associated virus ( AAV ) gene therapies. The acquired assets include all of Ceregene s therapeutic programs, including CERE-110, an AAV vector delivery system for the treatment of Alzheimer s disease that is currently in a Phase 2 clinical trial, certain intellectual property rights relating to the manufacturing of AAV, and certain toxicology and safety data from Ceregene s human clinical trials (the Acquisition). The Acquisition was closed on October 1, 2013 (the Closing Date).

On the Closing Date, Merger Sub merged with and into Ceregene, with Ceregene continuing as the surviving company and a wholly-owned subsidiaries of the Company. On the Closing Date, each share of Ceregene s issued and outstanding capital stock held by its stockholders converted to the right to receive a portion of the merger consideration for the Acquisition, which consists initially of (i) 100,000 shares of Sangamo common stock with a market value of approximately \$1.2 million on the Closing Date, and (ii) amount of cash and cash equivalent of Ceregene on the Closing Date less certain liabilities and expenses. In addition to such initial merger consideration, during the term of the Merger Agreement, the Company is required to make contingent earn-out payments (the Earn-Out Payments ) to the stockholders of Ceregene as follows:

If the Company grants a third-party license to develop and commercialize Ceregene's CERE-110 for the treatment of Alzheimer's disease or CERE-120 for the treatment of Parkinson's diseases or Huntington's disease (the Earn-Out Products), the Company is required to pay a double digit percentage of any upfront and milestone payments the Company receives for such license, subject to certain reductions based on expenses incurred by the Company in the development of the Earn-Out Products; and

If the Company commercializes any Earn-Out Product itself, the Company is required to pay, for each Earn-Out Product, royalty-like earnout payments as a percentage of net sales that range in the low double digits depending upon the amount of net sales, subject to certain reductions by the Company.

Also on the Closing Date, the Company, Ceregene and certain of its stockholders entered into an indemnity escrow agreement, pursuant to which a portion of the purchase price was deposited in an escrow account for the benefit of the Company to satisfy indemnity obligations of the stockholders under the Merger Agreement. Due to the relatively short time from the Closing Date of the Acquisition to the completion of the accompanying unaudited interim consolidated financial statements, the accounting for the initial purchase price allocation as well as certain required supplemental disclosures with respect to the Acquisition have not been completed.

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### **ITEM 2. MANAGEMENT'S DISCUSSION AND ANALYSIS OF FINANCIAL CONDITION AND RESULTS OF 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, 2012 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 ZFP technology, through early stage clinical testing, strategically partner with biopharmaceutical companies at points of value inflection and have the partner 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 zinc finger DNA-binding proteins (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 ongoing clinical trials, the most advanced of which are a Phase 2 study (SB-728-902 Cohort 5) and a Phase 1/2 study (SB-728-1102) in HIV-infected subjects. We expect to present data from these programs at appropriate scientific and medical meetings in 2013.

In January 2012, we established a collaborative partnership with Shire AG (Shire) to research, develop and commercialize some of our preclinical ZFP Therapeutic development programs, including programs in hemophilia, Huntington's disease and other monogenic diseases. We also have several proprietary preclinical programs in monogenic diseases, including hemoglobinopathies such as sickle cell disease and  $\beta$ -thalassemia and several lysosomal storage disorders. In addition, we have research stage programs in other monogenic diseases, including certain immunodeficiencies.

We believe the potential commercial applications of ZFPs are broad-based and we have also licensed our ZFP platform in fields outside human therapeutics as follows to facilitate the sale or license of ZFNs and ZFP TFs:

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 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

On October 1, 2013, we acquired Ceregene, Inc., ( Ceregene ) a privately held biotechnology company focused on the development of adeno-associated virus ( AAV ) gene therapies. The acquired assets include all of Ceregene s therapeutic programs, including CERE-110, an AAV vector delivery system for the treatment of Alzheimer s disease that is currently in a Phase 2 clinical trial, certain intellectual property rights relating to the manufacturing of AAV, and certain toxicology and safety data from Ceregene s human clinical trials.

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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, 2013, we incurred a consolidated net loss of \$6.1 million, or \$0.11 per share, compared to a net loss of \$5.8 million, or \$0.11 per share, for the same period in 2012. As of September 30, 2013, we had cash, cash equivalents, marketable securities and interest receivable totaling \$133.1 million compared to \$76.3 million as of December 31, 2012. As of September 30, 2013, we had an accumulated deficit of \$294.0 million.

Our revenues have consisted primarily of revenues from our corporate partners for ZFNs and ZFP TFs, contractual payments from strategic partners for research programs and research milestones, and 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 initial 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. We are conducting a Phase 2 and two Phase 1/2 clinical trials to evaluate a ZFP Therapeutic for the treatment of HIV/AIDS. Development of novel therapeutic products is costly and is subject to a lengthy and uncertain regulatory process by 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 the perception of the public.

***Critical Accounting Estimates***

The accompanying discussion and analysis of our financial condition and results of operations are based upon our 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, 2012, as filed with the SEC.

**Results of Operations*****Three months and nine months ended September 30, 2013 and 2012******Revenues***

|           | Three months ended<br>September 30,<br>(in thousands, except percentage values) |      |        |   | Nine months ended September 30,<br>(in thousands, except percentage values) |      |        |   |
|-----------|---------------------------------------------------------------------------------|------|--------|---|-----------------------------------------------------------------------------|------|--------|---|
|           | 2013                                                                            | 2012 | Change | % | 2013                                                                        | 2012 | Change | % |
| Revenues: |                                                                                 |      |        |   |                                                                             |      |        |   |

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|                          |          |          |        |     |           |           |          |       |
|--------------------------|----------|----------|--------|-----|-----------|-----------|----------|-------|
| Collaboration agreements | \$ 4,825 | \$ 4,190 | \$ 635 | 15% | \$ 15,065 | \$ 9,665  | \$ 5,400 | 56%   |
| Research grants          | 882      | 717      | 165    | 23% | 2,199     | 3,058     | (859)    | (28%) |
| Total revenues           | \$ 5,707 | \$ 4,907 | \$ 800 | 16% | \$ 17,264 | \$ 12,723 | \$ 4,541 | 36%   |

Total revenues consist of revenues from collaboration agreements, strategic partnerships and research grants. We anticipate revenues over the next several years will primarily be derived from our collaboration agreements with Shire, Sigma-Aldrich Corporation (Sigma) and Dow AgroSciences LLC (DAS), a wholly-owned subsidiary of Dow Chemical Corporation. In May 2013, the California Institute for Regenerative Medicine (CIRM) granted us a \$6.4 million Strategic Partnership Award to develop a potentially curative ZFP Therapeutic for beta-thalassemia based on the application of our ZFN gene-editing technology in hematopoietic stem cells (HSCs). The four year grant provides matching funds to support a potential Investigational New Drug (IND) application and a Phase 1 clinical trial in transfusion-dependent beta-thalassemia patients.

Revenues from our corporate collaboration and strategic partnering agreements were \$4.8 million for the three months ended September 30, 2013, compared to \$4.2 million in the corresponding period in 2012. The \$0.6 million increase in collaboration agreement revenues was primarily due to an increase of \$1.3 million in research service revenues related to our collaboration and license agreement with Shire. The revenues from Shire include amortization of an upfront payment of \$13.0 million and revenues from research services. These increases were partially offset by a \$0.5 million decrease in revenues related to research services under the agreement with DAS and \$0.2 million decrease in revenues related to other collaboration agreements. Research grant revenues were approximately \$0.9 million for the three months ended September 30, 2013, compared to \$0.7 million in the corresponding period in 2012.

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Revenues from our corporate collaboration and strategic partnering agreements were \$15.1 million for the nine months ended September 30, 2013, compared to \$9.7 million in the corresponding period in 2012. The increase of \$5.4 million in collaboration agreement revenues was primarily attributable to an increase in revenues of \$6.8 million related to the agreement with Shire, partially offset by \$1.3 million in lower revenues related to our agreement with DAS and \$0.1 million decrease in revenues related to other collaboration agreements. Research grant revenues were \$2.2 million for the nine months ended September 30, 2013, compared to \$3.1 million in the corresponding period in 2012. The decrease of \$0.9 million in research grant revenues was primarily due to the completion of our research grants with Juvenile Diabetes Research Foundation International and the CHDI Foundation in 2012.

***Operating Expenses***

|                            | Three months ended September 30,<br>(in thousands, except percentage values) |           |          |     | Nine months ended September 30,<br>(in thousands, except percentage values) |           |          |     |
|----------------------------|------------------------------------------------------------------------------|-----------|----------|-----|-----------------------------------------------------------------------------|-----------|----------|-----|
|                            | 2013                                                                         | 2012      | Change   | %   | 2013                                                                        | 2012      | Change   | %   |
| Operating expenses:        |                                                                              |           |          |     |                                                                             |           |          |     |
| Research and development   | \$ 8,703                                                                     | \$ 7,570  | \$ 1,133 | 15% | \$ 26,201                                                                   | \$ 22,427 | \$ 3,774 | 17% |
| General and administrative | 3,163                                                                        | 3,139     | 24       | 1%  | 9,595                                                                       | 9,125     | 470      | 5%  |
| Total expenses             | \$ 11,866                                                                    | \$ 10,709 | \$ 1,157 | 11% | \$ 35,796                                                                   | \$ 31,552 | \$ 4,244 | 13% |

**Research and development**

Research and development expenses consist primarily of salaries and personnel related expenses, including stock-based compensation, laboratory supplies, pre-clinical and clinical studies, manufacturing expenses, allocated facilities expenses, subcontracted research expenses and expenses for trademark registration and technology licenses. 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 Shire will increase our research and development expenses during the term of the agreement. Pursuant to the terms of the agreement with Shire, future expenses related to research activities related to the collaboration will be reimbursed by Shire, including employee and external research costs related to the programs. The reimbursement funds received from Shire will be recognized as revenue as the costs are incurred and collection is assured.

Research and development expenses were \$8.7 million for the three months ended September 30, 2013, compared to \$7.6 million in the corresponding period in 2012. The increase of \$1.1 million in research and development expenses was primarily due to an increase of \$1.0 million in external expenses, lab supplies and other costs related to our hemophilia, hemoglobinopathies and Huntington's disease programs and \$0.2 million in salaries and benefits, partially offset by a decrease of \$0.1 million in clinical trial expenses.

Research and development expenses were \$26.2 million for the nine months ended September 30, 2013, compared to \$22.4 million in the corresponding period in 2012. The increase of \$3.8 million in research and development expenses was primarily due to an increase of \$4.2 million in external expenses, lab supplies and other costs related to our hemophilia, hemoglobinopathies and Huntington's disease programs and \$0.6 million in salaries and benefits, partially offset by a decrease of \$1.0 million in clinical trial and manufacturing expenses, primarily attributable to our SB-728-T HIV/AIDS programs.

## **General and administrative**

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

General and administrative expenses were \$3.2 million for the three month period ended September 30, 2013 and \$3.1 million for the corresponding period in 2012. The increase was primarily related to higher corporate legal fees of \$0.1 million.

General and administrative expenses were \$9.6 million for the nine month period ended September 30, 2013 and \$9.1 million for the corresponding period in 2012. The increase was primarily related to higher patent related legal fees of \$0.3 million, higher professional fees of \$0.1 million and higher employee related costs of \$0.2 million, partially offset by lower allocated expenses of \$0.1 million.



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### **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, 2013, we had cash, cash equivalents, marketable securities and interest receivable totaling \$133.1 million compared to \$76.3 million as of December 31, 2012 with the increase primarily attributable to the completion of an underwritten public offering of the Company's common stock in September 2013, in which 7,015,000 shares of Sangamo common stock were sold at a public offering price of \$10.58 per share. The net proceeds to the Company from the sale of shares in this offering, after deducting underwriting discounts and commissions and other estimated offering expenses, were approximately \$69.5 million.

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 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 our agreement with Shire, we received an upfront license fee of \$13.0 million. We are also eligible to receive milestone payments, however, our eligibility is based on our achievement of specified research, regulatory, clinical development, commercialization and sales milestones and depends upon ours and Shire's ability to continue to progress our programs under collaboration. We will also be eligible to receive royalty payments that are a tiered double-digit percentage of net sales of products developed under the collaboration, if any.

Under the terms of our agreement to acquire Ceregene, we issued 100,000 shares of our common stock, with a market value of approximately \$1.2 million on the closing date of the acquisition, to the stockholders of Ceregene, and we also agreed to make certain contingent earn-out payments to the stockholders of Ceregene based upon revenues generated from license or sales transaction of certain existing products of Ceregene. We do not expect the acquisition, including the operation of the ongoing Phase 2 clinical trial of Ceregene product candidates, to have any significant impact on our operating expenses or cash balance in 2013.

#### *Cash Flow*

Net cash used in operating activities for the nine months ended September 30, 2013 and 2012 was \$17.8 million and \$8.3 million, respectively. Net cash used in operating activities for the nine months ended September 30, 2013 primarily reflected the net loss for the period, the decrease in deferred revenues related to our collaboration agreement with Shire and decrease in account payable and accrued liability, partially offset by stock-based compensation and other non-cash expenses included in net loss. Net cash used in operating activities for the nine months ended September 30, 2012 primarily reflects the net loss for the period, an increase in deferred revenues due to the \$13.0 million upfront received from Shire under the collaboration and license agreement, a decrease in accounts payable and accrued liabilities and an decrease in accounts receivable, partially offset by stock-based compensation.

Net cash provided by investing activities for the nine months ended September 30, 2013 and 2012 was \$0.1 million and \$4.4 million, respectively. Cash flows from investing activities for both periods primarily related to purchases and maturities of investments.

Net cash provided by financing activities for the nine months ended September 30, 2013 and 2012 was \$75.6 million and \$1.0 million, respectively. The increase for the nine month period ended September 30, 2013 was primarily attributable to \$69.5 million in net proceeds from the public offering of the Company's common stock completed in September 2013, as well as proceeds from the issuance of common stock upon exercise of stock options. Net cash provided by financing activities for the nine month period ended September 30, 2012 was primarily attributable to proceeds from the issuance of common stock upon 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 2014. 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.

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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 collaboration with Shire;
- 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 of our product candidates;
- 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, 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, 2013 have not changed materially from those discussed in Item 7A of our Form 10-K for the year ended December 31, 2012 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.

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.

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**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. You should also consider the risk factors set forth in our Annual Report on Form 10-K for the year ended December 31, 2012 ( 2012 Annual Report ) 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 2012 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 2012 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.*

In December 2008, in collaboration with scientists at the University of Pennsylvania, we filed an Investigational New Drug (IND) application for a Phase 1 trial of our CCR5 ZFN-based therapeutic, SB-728-T, for treatment of HIV/AIDS. In September 2009, we announced the FDA's review and acceptance of our IND application to initiate an open-label, repeat-dosing Phase 1 clinical trial of SB-728-T (SB-728-902). Preliminary data from these studies demonstrated that, to date, treatment of aviremic HIV-infected subjects with SB-728-T has been well-tolerated. We also have an on-going Phase 2 (SB-728-902, Cohort 5) and two Phase 1/2 trials (SB-728-1101 and 1002) for this indication. In addition, we have previously completed enrollment and the treatment phase of a Phase 1 and several Phase 2 clinical trials of our ZFP Therapeutic, SB-509, for diabetic neuropathy and ALS and 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. Since we have increased our focus on therapeutic research and development, investors increasingly assess the value of our technology 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 our 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 Phase 1 clinical program to develop SB-728-T for the treatment of HIV/AIDS. 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 May 2013, we presented preliminary data from these two ongoing clinical trials. Two of four evaluable subjects in Cohort 5 showed a decrease of greater than one log in their viral load during a sixteen week treatment interruption (TI) with one subject achieving a transiently undetectable viral load during the TI period. In subjects in which viral load decreased, a measureable anti-HIV immune response was also observed. 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. In October 2013, we presented new data from our ongoing Phase 2 clinical trial (SB-728-902 Cohort 5) for treatment of HIV/AIDS, demonstrating functional control of the virus at or below the limit of detection in HIV-infected subjects treated with SB-728-T, as well as additional data demonstrating depletion of the HIV viral reservoir in SB-728-T treated subjects in cohorts 1-3 of the SB-728-902 study. We expect to present a full data set from these trials in the second half of 2013. 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.

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In addition, the initial results from the Phase 1 clinical trial of our ZFP Therapeutic product, SB-509, became available in the first half of 2006 and the complete data set was presented in June 2008. The primary end point of the trial was clinical and laboratory safety; however, we collected some preliminary efficacy data that showed trends of clinical improvement in some subjects. Notwithstanding this preliminary efficacy data, the top-line data from our Phase 2b clinical study for SB-509-901 did not meet the key primary or secondary endpoints for the study and as a result we discontinued development of our SB-509 program in October 2011.

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 during the next several years, 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.

***We have limited experience in conducting clinical trials.***

Our ZFP Therapeutics may fail to show the desired safety and efficacy in initial clinical trials. We have an ongoing Phase 2 trial and two Phase 1/2 studies of a ZFP Therapeutic for HIV/AIDS. However, the FDA will require additional clinical testing which involves significantly greater resources, commitments and expertise and so 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 clinical trials and may not possess the necessary resources and expertise to complete such trials, and there is no guarantee that we will be able to enter into collaborative relationships with third parties that can provide us with the funding and expertise for such trials. In our collaborative agreement to develop ZFP Therapeutics with Shire AG (Shire), we are responsible for all activities through submission of IND Applications and European Clinical Trial Applications (CTA) and Shire is responsible for clinical development and commercialization of products arising from the alliance.



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***While we have stated that we intend to file IND applications for several ZFP Therapeutic programs over the next three years, 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 seven IND applications by the end of 2015. The preparation and submission of IND applications requires us to conduct rigorous and time-consuming pre-clinical 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 pre-clinical tests may produce negative or inconclusive results, which may lead us to decide, or regulators may require us, to conduct additional pre-clinical testing. If we cannot obtain positive results in pre-clinical testing, we may decide to abandon the projects altogether. 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 pre-clinical 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 affect 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.

***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 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

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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, are unable or unwilling to advance our programs, or if they do not diligently pursue product approval, this may slow our progress and defer our revenues. Our partners may sublicense or abandon development programs or we may have disagreements with our partners, which would cause associated product development to slow or cease. 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 would not only 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 and 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. 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 gene modification 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 gene modification. 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 benefit of this technology 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 modification 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 gene 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.

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***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 which 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 and 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 which 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 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 programs, 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.

***We may not be able to fully realize the expected benefits from the acquisition of Ceregene, Inc., and the operation of the new business of Ceregene, Inc. may subject us to additional risks.***

On October 1, we closed the acquisition of Ceregene Inc. ( Ceregene ), including all of its therapeutic programs and related intellectual property and other assets. Although we expect to realize strategic, operational and financial benefits as a result of the acquisition, we cannot be certain whether, and to what extent, such benefits will be achieved in the future. In particular, the success of the acquisition will depend on our ability to efficiently and successfully integrate Ceregene's business, including the prosecution of its CERE-110 Phase 2 clinical trial, and to apply Ceregene's technology for a delivery vector based on adeno-associated virus (AAV) to advance our ZFP Therapeutics. There is no guarantee that any existing and future clinical trials of Ceregene's product candidates, including CERE-110, will produce positive results, and failure to do so may adversely affect our ability to validate the AAV delivery

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technology and apply such technology to our ZFP products as well as negatively impact our stock price. In April 2013, Ceregene reported that its top line data for the CERE-120 Phase 2b clinical trial for Parkinson's disease did not demonstrate statistically significant efficacy in the primary endpoint. In addition, even if we obtain positive data from such clinical trials, there is no guarantee that the AAV delivery technology can be applied to our ZFP Therapeutics safely and effectively.

The acquisition of Ceregene also subjects us to additional operational and financial risks, including the following:

additional costs that we may need to incur in order to conduct and complete Ceregene's therapeutic programs, including the CERE-110 Phase 2 clinical trial, and to comply with new regulatory requirements;

difficulties acquiring and developing the necessary expertise to continue the development of AAV technologies and other acquired assets of Ceregene;

difficulties in coordinating research and development activities;

uncertainties in the business relationships with our collaborators and suppliers due to the acquisition;

difficulties in integrating Ceregene's accounting systems and procedures, including internal control over financial reporting as required by Sarbanes-Oxley Act; and

lack of previous experiences in conducting Phase 2 trials of a gene therapy based on AAV vector delivery system.

In addition, the market price of our common stock may decline as a result of the merger if the integration of Ceregene is unsuccessful, takes longer than expected or fails to achieve the expected benefits to the extent anticipated by financial analysts or investors, or the effect of the acquisition on our financial results is otherwise not consistent with the expectations of financial analysts or investors.

## **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;

TALE proteins; 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.



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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 Dow AgroSciences (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.

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, 2012, 2011 and 2010 were \$22.3 million, \$35.8 million and \$24.9 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

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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 strategic partnering agreements, other collaborations in non-therapeutic applications of our technology, federal government research grants and grants awarded by research foundations. As of September 30, 2013, we had an accumulated deficit of \$294.0 million. From 2005 to date, we have generated an aggregate of approximately \$226.7 million in net 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 expand and extend our research and development activities into human therapeutic product development. 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 2014, 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. 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 strategic partners, other collaborations in non-therapeutic applications of our technology, and federal government and research foundation grants. Our focus on higher-value therapeutic product development and related strategic partnerships 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.

**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.

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***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 research and collaborative agreement with Shire AG (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. Under this agreement, we are responsible for all research activities through the submission of an IND or European Clinical Trial Application (CTA), while 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. Under the agreement, we may be eligible to receive milestone payments upon the achievement of specified clinical development, commercialization and post-commercialization milestones. The total amount of potential milestone payments for each gene target, assuming the achievements of all specified milestones in the agreement, is \$213.5 million. We may receive royalty payments based on specified percentages of net sales of products, if any. Once an IND or CTA is submitted, Shire will have control and broad discretion over all aspects of the clinical development and commercialization of any product developed under the program, and we will have little, if any, influence on how such programs will be conducted. Our lack of control over the clinical development of gene targets in our agreement with Shire could cause delays or other difficulties in the development and commercialization of our product candidates, which may prevent us from receiving any milestone, royalty payments and other benefits under the agreement.

***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. 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 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. This relationship was expanded in October 2009 when we amended our license agreement with Sigma to provide Sigma with the 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 June 2008, following a research period, Dow AgroSciences (DAS) exercised its commercial license option under a license agreement with us relating to plant agriculture. This agreement provides DAS with the exclusive right to develop agricultural products using our ZFP technology in plant cells, plants, or plant cell cultures. Both companies also 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.

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***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 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.



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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 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, 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, 2013, the closing price our common stock price, as reported by the NASDAQ Global Select Market, ranged from a low of \$7.92 to high of \$11.28. During the fiscal year ended December 31, 2012, our common stock price fluctuated, ranging from a low of \$2.95 to a high of \$6.49. 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 or changes in recommendation, estimate or coverage by securities analysts;

announcements by us or our competitors of new or enhanced products, technologies or services or significant contracts, mergers or acquisitions, strategic relationships, joint ventures or capital commitments;

regulatory developments;

additions or departures of key personnel;

future sales of our common stock or other securities by us, management, employees or directors, liquidation of institutional funds that comprised large holdings of our stock; and

changes in our cash balances.

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***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.

***If securities and/or industry analysts fail to continue publishing research about our business, if they change their recommendations adversely or if our results of operations do not meet their expectations, our stock price and trading volume could decline.***

The trading market for our common stock will be influenced by the research and reports that industry or securities analysts publish about us or our business. If one or more of these analysts cease coverage of our company or fail to publish reports on us regularly, we could lose visibility in the financial markets, which in turn could cause our stock price or trading volume to decline. In addition, it is likely that in some future period our operating results will be below the expectations of securities analysts or investors. If one or more of the analysts who cover us downgrade our stock, or if our results of operations do not meet their expectations, our stock price could 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.

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**ITEM 6. EXHIBITS**

(a) Exhibits:

|         |                                                                                                                                                                                                                                                                   |
|---------|-------------------------------------------------------------------------------------------------------------------------------------------------------------------------------------------------------------------------------------------------------------------|
| 2.1     | Agreement and Plan of Merger, dated August 26, 2013, by and among the Company, CG Acquisition Sub, Inc., and Ceregene, Inc. and the Stockholders Representative (incorporated by reference to Exhibit 2.1 to Current Report on Form 8-K filed on October 7, 2013) |
| 10.1    | Second Amendment to Triple Net Laboratory Lease, between the Company and Point Richmond R&D Associates II, LLC, dated March 15, 2007                                                                                                                              |
| 10.2    | Third Amendment to Triple Net Laboratory Lease, between the Company and Point Richmond R&D Associates II, LLC, dated August 1, 2013                                                                                                                               |
| 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.*

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**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: November 4, 2013

SANGAMO BIOSCIENCES, INC.

/s/ H. WARD WOLFF

**H. Ward Wolff**

**Executive Vice President and Chief Financial  
Officer**

**(Principal Financial and Accounting Officer)**