SANGAMO THERAPEUTICS, INC
Form 10-Q
November 08, 2018
Ion

UNITED STATES

SECURITIES AND EXCHANGE COMMISSION

Washington, D.C. 20549

FORM 10-Q

(Mark One)

QUARTERLY REPORT PURSUANT TO SECTION 13 OR 15(d) OF THE SECURITIES EXCHANGE ACT OF 1934
For the quarterly period ended September 30, 2018

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 THERAPEUTICS, INC.

(exact name of registrant as specified in its charter)

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

501 Canal Blvd

Richmond, California 94804

(Address of principal executive offices)

(510) 970-6000

(Registrant's telephone number, including area code)

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

Indicate by check mark whether the registrant has submitted electronically every Interactive Data File required to be submitted 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 such files). Yes No

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

Large accelerated filer	Accelerated filer
Non-accelerated filer	Smaller reporting company
Emerging growth company	

If an emerging growth company, indicate by check mark if the registrant has elected not to use the extended transition period for complying with any new or revised financial accounting standards provided pursuant to Section 13(a) of the Exchange Act.

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

As of November 1, 2018, 102,092,863 shares of the issuer's common stock, par value $0.01 per share, were outstanding.

INDEX

SANGAMO THERAPEUTICS, INC.

PART I. FINANCIAL INFORMATION

PART II. OTHER INFORMATION

Unless otherwise indicated or the context suggests otherwise, references in this Quarterly Report on Form 10-Q, or Quarterly Report, to "Sangamo," the "Company," "we," "us," and "our" refer to Sangamo Therapeutics, Inc. and our subsidiari including, TX Cell SA.

ZFP Therapeutic®, Engineering Genetic Cures®, and Pioneering Genetic Cures® are registered trademarks of Sangamo Therapeutics, Inc. Any third-party trade names, trademarks and service marks appearing in this Quarterly Report are the property of their respective holders.

Convenience translations between Euros (€) and U.S. dollars provided herein are based on the noon buying rate in New York City for cable transfers in foreign currencies as certified for customs purposes by the Federal Reserve Bank of New York on October 29, 2018, or €1.00 = $1.1622. We do not represent that Euros were, could have been, or could be, converted into U.S. dollars at such rate or at any other rate.

2

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;
- the acquisition of TxCell S.A., including the anticipated benefits thereof;
- partnering, other acquisition and other strategic transactions;
- revenues from existing and new collaborations;
- our research and development and other expenses;
- sufficiency of our cash resources;
- our operational and legal risks; and
- our plans, objectives, expectations and intentions and any other statements that are not historical facts.

In some cases, you can identify forward-looking statements by terms such as: “anticipates,” “believes,” “continues,” “could,” “estimates,” “expects,” “intends,” “may,” “plans,” “seeks,” “should” and “will.” These statements reflect our current views wit to future events and are based on assumptions and subject to risks and uncertainties. Given these risks and uncertainties, you should not place undue reliance on these forward-looking statements. We discuss many of these risks in greater detail under the headings “Risk Factors” and “Management’s Discussion and Analysis of Financial Conditions and Results of Operations” in this Quarterly Report. Except as required by law, we undertake no obligation to update any forward-looking statements to reflect events or circumstances arising after the date of such statements. Readers are cautioned not to place undue reliance on the forward-looking statements, which speak only as of the date of this Quarterly Report.

PART I. FINANCIAL INFORMATION

ITEM 1. FINANCIAL STATEMENTS
SANGAMO THERAPEUTICS, INC.

CONDENSED CONSOLIDATED BALANCE SHEETS

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

	September 30, 2018	December 31, 2017
ASSETS		
Current assets:		
Cash and cash equivalents	$39,298	$49,826
Marketable securities	419,272	193,482
Interest receivable	683	240
Accounts receivable	5,567	3,343
Prepaids and other current assets	3,382	1,506
Total current assets	468,202	248,397
Marketable securities, non-current	—	1,012
Property and equipment, net	50,497	31,066
Goodwill	1,585	1,585
Other non-current assets	6,379	1,181
Non-current restricted cash	79,941	3,500
Total assets	$606,604	$286,741
LIABILITIES AND STOCKHOLDERS' EQUITY		
Current liabilities:		
Accounts payable and accrued liabilities	$16,424	$11,035
Accrued compensation and employee benefits	6,605	5,479
Deferred revenues	51,094	28,345
Total current liabilities	74,123	44,859
Deferred revenues, non-current	123,917	29,244
Build-to-suit lease obligation	26,928	24,738
Non-current liabilities	1,730	—
Total liabilities	226,698	98,841
Commitments and contingencies		
Stockholders' equity:		
Common stock, $0.01 par value; 160,000,000 shares authorized, 101,839,668 and 85,598,534 shares issued and outstanding at September 30, 2018 and December 31, 2017, respectively	1,018	856
Additional paid-in capital	923,164	682,809

Accumulated deficit	(544,032)	(495,479)
Accumulated other comprehensive loss	(244)	(286)
Total stockholders' equity	379,906	187,900
Total liabilities and stockholders' equity	$606,604	$286,741

See accompanying notes.

SANGAMO THERAPEUTICS, INC.

CONDENSED CONSOLIDATED STATEMENTS OF OPERATIONS

(Unaudited; in thousands, except per share amounts)

	Three Months Ended September 30,		Nine Months Ended September 30,	
	2018	2017	2018	2017
Revenues:				
Collaboration agreements	$23,538	$11,759	$57,378	$23,042
Research grants	24	53	237	448
Total revenues	23,562	11,812	57,615	23,490
Operating expenses:				
Research and development	28,810	18,425	81,612	46,351
General and administrative	10,993	6,422	32,381	19,734
Total operating expenses	39,803	24,847	113,993	66,085
Loss from operations	(16,241)	(13,035)	(56,378)	(42,595)
Interest and other income, net	3,398	681	6,708	1,118
Net loss	$(12,843)	$(12,354)	$(49,670)	$(41,477)
Basic and diluted net loss per share	$(0.13)	$(0.15)	$(0.52)	$(0.55)
Shares used in computing basic and diluted net loss per share	101,725	83,750	95,165	75,814

See accompanying notes.

SANGAMO THERAPEUTICS, INC.

CONDENSED CONSOLIDATED STATEMENTS OF COMPREHENSIVE LOSS

(Unaudited; in thousands)

	Three Months Ended September 30,		Nine Months Ended September 30,	
	2018	2017	2018	2017
Net loss	$(12,843)	$(12,354)	$(49,670)	$(41,477)
Change in unrealized gain (loss) on available-for-sale securities	(88)	12	43	(131)
Comprehensive loss	$(12,931)	$(12,342)	$(49,627)	$(41,608)

See accompanying notes.

SANGAMO THERAPEUTICS, INC.

CONDENSED CONSOLIDATED STATEMENTS OF CASH FLOWS

(Unaudited; in thousands)

	Nine Months Ended September 30,	
	2018	2017
Operating Activities:		
Net loss	$(49,670)	$(41,477)
Adjustments to reconcile net loss to net cash used in operating activities:		
Depreciation and amortization	1,668	1,030
Amortization of (discount) premium on marketable securities	(4,043)	(310)
Stock-based compensation	10,374	6,969
Taxes paid related to net share settlement of equity awards	—	(71)
Other	715	—
Net changes in operating assets and liabilities:		
Interest receivable	(443)	(259)
Accounts receivable	(2,225)	1,539
Prepaid expenses and other assets	(1,851)	(743)
Accounts payable and accrued liabilities	789	3,676
Accrued compensation and employee benefits	1,127	1,589
Non-current liabilities	1,730	—
Deferred revenues	118,540	56,078
Net cash provided by operating activities	76,711	28,021
Investing Activities:		
Purchases of marketable securities	(451,239)	(229,595)
Maturities of marketable securities	230,547	127,093
Purchases of property and equipment	(15,028)	(2,873)
Other investment	(5,221)	—
Net cash used in investing activities	(240,941)	(105,375)
Financing Activities:		
Proceeds from public offering of common stock, net of issuance costs	215,757	81,573
Taxes paid related to net share settlement of equity awards	(83)	—
Proceeds from issuance of common stock	14,469	3,614
Net cash provided by financing activities	230,143	85,187
Net increase in cash, cash equivalents, and restricted cash	65,913	7,833
Cash, cash equivalents, and restricted cash, beginning of period	53,326	22,061
Cash, cash equivalents, and restricted cash, end of period	$119,239	$29,894
Supplemental disclosure of noncash investing activities:		
Property and equipment included in accrued liabilities	$5,813	$50

See accompanying notes.

7

SANGAMO THERAPEUTICS, INC.

NOTES TO CONDENSED CONSOLIDATED FINANCIAL STATEMENTS

September 30, 2018

(Unaudited)

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

Overview

Sangamo Therapeutics, Inc. was incorporated in the state of Delaware in 1995 and changed its name from Sangamo Biosciences, Inc. in January 2017 ("Sangamo" or the "Company"). Sangamo is focused on the research, development and commercialization of novel genomic therapies for unmet medical needs. Sangamo's genome editing and gene regulation technology platform is enabled by the engineering of a class of transcription factors known as zinc finger DNA-binding proteins ("ZFPs").

Sangamo is currently working on a number of long-term development projects that involve experimental technology. The projects may require several years and substantial expenditures to complete and ultimately may be unsuccessful. The Company plans to finance operations with available cash resources, collaborations and strategic partnerships, research grants and from the issuance of equity or debt securities. Sangamo believes that its available cash, cash equivalents, marketable securities and interest receivable as of September 30, 2018, along with expected revenues from collaborations, strategic partnerships and research grants, will be adequate to fund its operations at least through the next twelve months. Sangamo will need to raise substantial additional capital to fund the development, manufacturing and potential commercialization of its product candidates. Additional capital may not be available on terms acceptable to the Company, if at all. If adequate funds are not available, or if the terms of potential funding sources are unfavorable, the Company's business and ability to develop its technology and product candidates could be harmed. Furthermore, any sales of additional equity securities may result in dilution to the Company's stockholders, and any debt financing may include covenants that restrict the Company's business.

Basis of Presentation

The accompanying unaudited condensed consolidated financial statements 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, 2018 are not necessarily indicative of the results that may be expected for the year ending December 31, 2018. The condensed consolidated balance sheet data at December 31, 2017 were derived from the audited consolidated financial statements included in Sangamo's Annual Report on Form 10-K for the year ended December 31, 2017, (the "2017 Annual Report"), as filed with the SEC. The accompanying condensed consolidated financial statements and related financial information should be read in conjunction with the audited financial statements and footnotes thereto for the year ended December 31, 2017, included in the 2017 Annual Report.

Use of Estimates

The preparation of financial statements in conformity with generally accepted accounting principles requires management to make estimates and assumptions that affect the amounts reported in the financial statements and the accompanying notes. On an ongoing basis, management evaluates its estimates, including critical accounting policies or estimates related to revenue recognition, clinical trial accruals, 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.

Cash and Cash Equivalents

Sangamo considers all highly-liquid investments purchased with original maturities of three months or less at the purchase date to be cash equivalents. Cash and cash equivalents consist of cash and deposits in money market investment accounts.

Marketable Securities

Sangamo classifies its marketable securities as available-for-sale which are recorded at estimated fair value based on quoted market prices or observable market inputs of almost identical assets. Unrealized holding gains and losses are included in accumulated other comprehensive income.

The Company’s investments are subject to a periodic impairment review. 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. Realized gains and losses on available-for-sale securities are included in other income, which is determined using the specific identification method.

Fair Value Measurements

The carrying amounts for financial instruments consisting of cash and cash equivalents, accounts receivable, accounts payable and accrued liabilities approximate fair value due to their short maturities. Marketable securities and liabilities are stated at their estimated fair values. The counterparties to the agreements relating to the Company's investment securities consist of the US Treasury, governmental agencies and various major corporations and financial institutions with investment-grade high credit ratings.

Revenue Recognition

Effective January 1, 2018, the Company adopted the provisions of Accounting Standards Codification ("ASC"), Topic 606, Revenue from Contracts with Customers ("Topic 606") resulting in a change to its accounting policy for revenue recognition. Topic 606 establishes a unified model to determine how revenue is recognized.

The Company's contract revenues consist of strategic partnering collaboration agreements and research activity grants and licensing. Research and licensing agreements typically include upfront signing or license fees, cost reimbursements, research services, minimum sublicense fees, milestone payments and royalties on future licensee's product sales. The Company has both fixed and variable consideration. Non-refundable upfront fees and funding of research and development activities are considered fixed, while milestone payments are identified as variable consideration. 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. Revenues under grant agreements are recognized when the related qualified research expenses are incurred. Deferred revenue represents the portion of research or license payments received but not earned.

In determining the appropriate amount of revenue to be recognized as it fulfills its obligations under its agreements, the Company performs the following steps: (i) identification of the promised goods or services in the contract; (ii) determination of whether the promised goods or services are performance obligations including whether they are distinct in the context of the contract; (iii) measurement of the transaction price, including the constraint on variable consideration; (iv) allocation of the transaction price to the performance obligations based on estimated selling prices; and (v) recognition of revenue when (or as) the Company satisfies each performance obligation.

A performance obligation is a promise in a contract to transfer a distinct good or service to the customer and is the unit of account in Topic 606. The Company's performance obligations include license rights, development services, and services associated with regulatory submission and approval processes. Significant management judgment is required to determine the level of effort required under an arrangement and the period over which the Company expects to complete its performance obligations under the arrangement. If the Company cannot reasonably estimate when its performance obligations either are completed or become inconsequential, then revenue recognition is deferred until the Company can reasonably make such estimates. The Company includes the unconstrained amount of estimated variable consideration in the transaction price. The amount included in the transaction price is constrained to the amount for which it is probable that a significant reversal of cumulative revenue recognized will not occur. At the end of each subsequent reporting period, the Company re-evaluates the estimated variable consideration included in the transaction price and any related constraint, and if necessary, adjusts its estimate of the overall transaction price. Revenue is then recognized over the remaining estimated period of performance using the cumulative catch-up method. The estimated period of performance and project costs are reviewed quarterly and adjusted, as needed, to

reflect the Company's current assumptions regarding the timing of its deliverables.

As part of the accounting for these arrangements, the Company must develop assumptions that require judgment to determine the stand-alone selling price of each performance obligation identified in the contract. The Company uses key assumptions to determine the stand-alone selling price, which may include forecasted revenues, development timelines, reimbursement rates for personnel costs, discount rates and probabilities of technical and regulatory success.

During the nine months ended September 30, 2018, revenues related to the hemophilia A collaboration agreement with Pfizer Inc. ("Pfizer") and Kite Pharma, Inc. ("Kite"), a wholly-owned subsidiary of Gilead Sciences, Inc., represented 46% and 29%, respectively, of the Company's total revenue. During the nine months ended September 30, 2017, revenues related to the Company's hemophilia A collaboration agreement with Pfizer and the hemoglobinopathies agreement with Bioverativ, a Sanofi company ("Bioverativ") represented 44% and 39%, respectively, of total revenue. Receivables from collaborations are typically unsecured and are concentrated in the biopharmaceutical industry. Accordingly, the Company may be exposed to credit risk generally associated with biopharmaceutical companies or specific to its collaboration agreements. To date, the Company has not experienced any losses related to these receivables.

Funds received from third parties under contract or grant arrangements are recorded as revenue if the Company is deemed to be the principal participant in the arrangements because the activities under the contracts or grants are part of the Company's

development programs. Contract funds received are not refundable and are recognized when the related qualified research and development costs are incurred and there is reasonable assurance that the funds will be received. Funds received in advance are recorded as deferred revenue.

Recent Accounting Pronouncements

Recently Adopted

In May 2014, the Financial Accounting Standards Board ("FASB") issued Accounting Standards Updated ("ASU") 2014-09, Revenue from Contracts with Customers ("Topic 606"). This standard outlines a single comprehensive model for entities to use in accounting for revenue arising from contracts with customers and supersedes most current revenue recognition guidance, including industry-specific guidance. The main principle of Topic 606 is to recognize revenues when promised goods or services are transferred to customers in an amount that reflects the consideration that is expected to be received for those goods or services. Topic 606 provides companies with two implementation methods: (i) apply the standard retrospectively to each prior reporting period presented (full retrospective application); or (ii) apply the standard retrospectively with the cumulative effect of initially applying the standard as an adjustment to the opening balance of retained earnings of the annual reporting period that includes the date of initial application (modified retrospective application). The Company implemented this standard under the modified retrospective method. This guidance is effective for annual reporting periods beginning after December 15, 2017, including interim periods within that reporting period. Under Topic 606, an entity recognizes revenue when its customer obtains control of promised goods or services, in an amount that reflects the consideration which the entity expects to receive in exchange for those goods or services. To determine revenue recognition for arrangements that an entity determines are within the scope of Topic 606, the entity performs the following five steps: (i) identify the contract(s) with a customer; (ii) identify the performance obligations in the contract; (iii) determine the transaction price; (iv) allocate the transaction price to the performance obligations in the contract; and (v) recognize revenue when (or as) the entity satisfies a performance obligation. Topic 606 also impacts certain other areas, such as the accounting for costs to obtain or fulfill a contract. The Company adopted Topic 606 effective January 1, 2018, using the modified retrospective method with a cumulative effect adjustment of $1.1 million reflected as a decrease to the opening balance of accumulated deficit and a decrease to deferred revenues, respectively.

Refer below for a summary of the amount by which each financial statement line item was affected by the impact of the cumulative adjustment and as compared with the guidance that was in effect prior to the adoption:

	Impact of Topic 606 Adoption on Condensed Consolidated Balance Sheet as of January 1, 2018		
(in thousands)	As reported under Topic 606	Adjustments	Balances without adoption of Topic 606
Deferred revenue, current portion	$29,626	$ 1,281	$28,345
Deferred revenue, noncurrent portion	$26,846	$ (2,398)	$29,244
Accumulated deficit	$(494,362)	$ 1,117	$(495,479)

(in thousands)	Impact of Topic 606 Adoption on Condensed Consolidated Balance Sheet as of September 30, 2018		
	As reported under Topic 606	Adjustments	Balances without adoption of Topic 606
Deferred revenue, current portion	$51,094	$ 12,741	$63,835
Deferred revenue, noncurrent portion	$123,917	$ (3,130)	$120,787
Accumulated deficit	$(544,032)	$ (9,611)	$(553,643)

(in thousands)	Impact of Topic 606 Adoption on Condensed Consolidated Statement of Operations and Comprehensive Loss for the Three Months Ended September 30, 2018			Impact of Topic 606 Adoption on Condensed Consolidated Statement of Operations and Comprehensive Loss for the Nine Months Ended September 30, 2018		
	As reported under Topic 606	Adjustments	Balances without adoption of Topic 606	As reported under Topic 606	Adjustments	Balances without adoption of Topic 606
Collaboration revenue	$23,538	$ (4,461)	$19,077	$57,378	$ (8,495)	$48,883
Net loss	$(12,843)	$ (4,461)	$(17,304)	$(49,670)	$ (8,495)	$(58,165)
Net loss per share - basic and diluted:	$(0.13)	$ (0.04)	$(0.17)	$(0.52)	$ (0.09)	$(0.61)

	Impact of Topic 606 Adoption on Condensed Consolidated Statement of Cash Flows for the Nine Months Ended September 30, 2018		
(in thousands)	As reported under Topic 606	Adjustments	Balances without adoption of Topic 606
Net loss	$(49,670)	$ (8,495)	$(58,165)
Changes in deferred revenue	$118,540	$ 8,495	$127,035

In November 2016, the FASB issued ASU 2016-18, Statement of Cash Flows ("Topic 230"). The Company adopted Topic 230 in the beginning of fiscal 2018, which requires the statement of cash flows to explain the change during the period relating to total cash, cash equivalents, and restricted cash. The Company adopted this standard using the retrospective transition method by restating its condensed consolidated statements of cash flows to include restricted cash of $3.5 million as of January 1, 2018 and $79.9 million in the ending cash, cash equivalents, and restricted cash balances for the nine months ended September 30, 2018. The restricted cash balance as of September 30, 2018 includes the letter of credit for $3.5 million established as a deposit for the Brisbane build-to-suit lease and $76.4 million to acquire the equity of TxCell S.A., a French société anonyme ("TxCell"). Net cash flows for the nine months ended September 30, 2017, changed as a result of including restricted cash with cash and cash equivalents when reconciling the beginning-of-period and end-of-period amounts presented on the statements of cash flows. Restricted cash was included in other current and other non-current assets on the Company's condensed consolidated balance sheets.

The following table provides a reconciliation of cash, cash equivalents, and restricted cash within the statement of financial position that sum to the total of the same amounts in the statement of cash flows:

	As of September 30,	
(in thousands)	2018	2017
Cash and cash equivalents	$39,298	$29,894
Restricted cash included in other non-current assets	79,941	—
Total cash, cash equivalents, and restricted cash shown in the statements of cash flows	$119,239	$29,894

In August 2018, the SEC adopted amendments to certain disclosure requirements in Securities Act Release No. 33-10532, Disclosure Update and Simplification. These amendments eliminate, modify, or integrate into other SEC requirements certain disclosure rules. Among the amendments is the requirement to present an analysis of changes in stockholders' equity in the interim financial statements included in quarterly reports on Form 10-Q. The analysis, which can be presented as a footnote or separate statement, is required for the current and comparative quarter and year-to-date interim periods. The amendments are effective for all filings made on or after November 5, 2018. In light of the anticipated timing of effectiveness of the amendments and expected proximity of effectiveness to the filing date for most filers' quarterly reports, the SEC's Division of Corporate Finance issued a Compliance and Disclosure Interpretation related to Exchange Act Forms, or CDI – Question 105.09, that provides transition guidance related to this disclosure requirement. CDI – Question 105.09 states that the SEC would not object if the filer's first presentation

of the changes in shareholders' equity is included in its Form 10-Q for the quarter that begins after the effective date of the amendments. As such, the Company adopted these SEC amendments on November 5, 2018 and will present the analysis of changes in stockholders' equity in its interim financial statements in its March 31, 2019 Form 10-Q. The Company does not anticipate that the adoption of these SEC amendments will have a material effect on the Company's financial position, results of operations, cash flows or shareholders' equity.

Not yet adopted

In February 2016 the FASB issued ASU 2016-02, Leases ("ASU 2016-02"). ASU 2016-02 amends a number of aspects of lease accounting, including requiring lessees to recognize almost all leases with a term greater than one year as a right-of-use asset and corresponding liability, measured at the present value of the lease payments. The guidance will become effective for the Company beginning in the first quarter of 2019 with early adoption permitted and will be adopted using a modified retrospective approach. The Company expects to adopt the new standard on January 1, 2019 and use the effective date as the date of initial application. Consequently, financial information will not be updated and the disclosures required under the new standard will not be provided for dates and periods before January 1, 2019.

The Company expects that this standard will have a material effect on the financial statements. While the Company continues to assess the various impacts of adoption, the most significant effects will primarily relate to (1) the recognition of a right-of-use assets and lease liabilities on the balance sheet for the Company's existing operating leases; (2) the derecognition of existing assets and liabilities for sale-leaseback transactions arising from build-to-suit lease arrangements for which construction is complete and we are leasing the constructed asset that currently do not qualify for sale accounting; (3) the derecognition of existing assets and liabilities for certain assets under construction in build-to-suit lease arrangements that the Company will lease when construction is complete; and (4) providing significant new disclosures about leasing activities.

NOTE 2—FAIR VALUE MEASUREMENT

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

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

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

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

	September 30, 2018 Fair Value Measurements			
	Total	Level 1	Level 2	Level 3
Assets:				
Cash equivalents:				
Money market funds	$18	$ 18	$—	$ —
Total	18	18	—	—
Marketable securities:				
Commercial paper securities	317,119	—	317,119	—
Corporate debt securities	83,579	—	83,579	—
U.S. government-sponsored entity debt securities	18,574	—	18,574	—
Total	419,272	—	419,272	—
Total cash equivalents and marketable securities	$419,290	$ 18	$419,272	—

	December 31, 2017 Fair Value Measurements			
	Total	Level 1	Level 2	Level 3
Assets:				
Cash equivalents:				
Money market funds	$24,290	$24,290	$—	$ —
Commercial paper securities	4,595	—	4,595	—
Total	28,885	24,290	4,595	—
Marketable securities:				
Commercial paper securities	110,247	—	110,247	—
Corporate debt securities	75,755	—	75,755	—
U.S. government-sponsored entity debt securities	8,492	—	8,492	—
Total	194,494	—	194,494	—

Total cash equivalents and marketable securities	$223,379	$24,290	$199,089	—

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

NOTE 3—MARKETABLE SECURITIES

The Company classifies its marketable securities as available-for-sale and records its investments at estimated fair value based on quoted market prices or observable market inputs of substantially identical assets. Unrealized holding gains and losses are included

in accumulated other comprehensive income (loss). Investments that have maturities beyond one year as of the end of the reporting period are classified as non-current.

The Company's investments are subject to a periodic impairment review. 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. Realized gains and losses on available-for-sale securities are included in other income, which is determined using the specific identification method

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

	Amortized Cost	Gross Unrealized Gains	Gross Unrealized (Losses)	Estimated Fair Value
September 30, 2018				
Cash equivalents:				
Money market funds	$ 18	$ —	$ —	$ 18
Total	18	—	—	18
Available-for-sale securities:				
Commercial paper securities	317,203	11	(95)	317,119
Corporate debt securities	83,684	—	(105)	83,579
U.S. government-sponsored entity debt securities	18,598	—	(24)	18,574
Total	419,485	11	(224)	419,272
Total cash equivalents and available-for-sale securities	$ 419,503	$ 11	$ (224)	$ 419,290
December 31, 2017				
Cash equivalents:				
Money market funds	$ 24,290	$ —	$ —	$ 24,290
Commercial paper securities	4,595	—	—	4,595
Total	28,885	—	—	28,885
Available-for-sale securities:				
Commercial paper securities	110,365	—	(118)	110,247
Corporate debt securities	75,886	—	(131)	75,755
U.S. government-sponsored entity debt securities	8,498	—	(6)	8,492
Total	194,749	—	(255)	194,494
Total cash equivalents and available-for-sale securities	$ 223,634	—	$ (255)	$ 223,379

The Company had no material realized losses or other-than-temporary impairments of its investments for the nine months ended September 30, 2018 and 2017. As of September 30, 2018, all of the Company's investments had maturity dates within one year. The Company has the intent and ability to hold its investments for a period of time sufficient to allow for any anticipated recovery in market value.

NOTE 4—BASIC AND DILUTED NET LOSS PER SHARE

Basic net loss per share has been computed by dividing 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.

The total number of shares subject to stock options and restricted stock units outstanding, which are all anti-dilutive, were excluded from consideration in the calculation of diluted net loss per share. Stock options and restricted stock units outstanding as of September 30, 2018 and 2017 were 8,770,775 and 9,581,024, respectively.

NOTE 5—MAJOR CUSTOMERS, PARTNERSHIPS AND STRATEGIC ALLIANCES

Collaboration Agreements

Kite Pharma, Inc.

In February 2018, the Company entered into a collaboration and license agreement with Kite, for the research, development and commercialization of potential engineered cell therapies for cancer. Kite will be responsible for all clinical development and commercialization of any resulting products. The Kite agreement became effective on April 5, 2018 when the waiting periods under the Hart-Scott-Rodino Antitrust Improvements Act of 1976, as amended, and other customary closing conditions were completed.

Subject to the terms of this agreement, the Company granted Kite an exclusive, royalty-bearing, worldwide, sublicensable license, under the Company's relevant patents and know-how, to develop, manufacture and commercialize, for the purpose of treating cancer, specific cell therapy products that may result from the research program and that are engineered ex vivo using selected zinc finger nucleases ("ZFNs") and adeno-associated viral vectors ("AAVs") developed under the research program, to express chimeric antigen receptors ("CARs"), T-cell receptors ("TCRs") or NK-cell receptors ("NKRs") directed to candidate targets.

During the research program term and subject to certain exceptions, except pursuant to this agreement, the Company is prohibited from researching, developing, manufacturing and commercializing, for the purpose of treating cancer, any cell therapy product that, as a result of ex vivo genome editing, expresses a CAR, TCR or NKR that is directed to a target expressed on or in a human cancer cell. After the research program term concludes and subject to certain exceptions, except pursuant to this agreement, the Company will be prohibited from developing, manufacturing and commercializing, for the purpose of treating cancer, any cell therapy product that, as a result of ex vivo genome editing, expresses a CAR, TCR or NKR that is directed to a candidate target.

Following the effective date, in April 2018, the Company received a $150.0 million upfront payment from Kite. In addition, Kite will reimburse the Company's direct costs to conduct the joint research program, and Kite will be responsible for all subsequent development, manufacturing and commercialization of any licensed products. Sangamo is also eligible to receive contingent development- and sales-based milestone payments that could total up to $3.01 billion if all of the specified milestones set forth in this agreement are achieved. Of this amount, approximately $1.26 billion relates to the achievement of specified research, clinical development, regulatory and first commercial sale milestones, and approximately $1.75 billion relates to the achievement of specified sales-based milestones if annual worldwide net sales of licensed products reach specified levels. Each development- and sales-based milestone payment is payable (i) only once for each licensed product, regardless of the number of times that the associated milestone event is achieved by such licensed product, and (ii) only for the first ten times that the associated milestone event is achieved, regardless of the number of licensed products that may achieve such milestone event. In addition, the Company will be entitled to receive escalating, tiered royalty payments with a percentage in the single digits based on potential future annual worldwide net sales of licensed products. These royalty payments will be subject to reduction due to patent expiration, entry of biosimilar products to the market and payments made under certain licenses for third-party intellectual property.

The initial research term in the agreement is six years. Kite has an option to extend the research term of the agreement for up to two additional one-year periods for a separate upfront fee of $10.0 million per year. All contingent payments under the agreement, when earned, will be non-refundable and non-creditable. The transaction price of $185.9 million includes the upfront license fee of $150.0 million and $35.9 million estimated reimbursable service costs for identified research projects over the estimated performance period. Estimated fees for the presumed exercise of the research term extension options and all milestone amounts are fully constrained. As part of its evaluation of the constraint, the Company considered numerous factors, including the fact that achievement of the milestones at this time is uncertain and contingent upon future periods when the uncertainty related to the variable consideration is resolved. The Company will re-evaluate the transaction price, including the estimated variable consideration included in the transaction price and all constrained amounts, in each reporting period and as uncertain events are resolved or other changes in circumstances occur. None of the development and sales-based milestone payments have been included in transaction price.

Kite has the right to terminate this agreement, in its entirety or on a per licensed product or per candidate target basis, for any reason after a specified notice period. Each party has the right to terminate this agreement on account of the other party's bankruptcy or material, uncured breach.

The Company has identified the primary performance obligations within the Kite agreement as a license to the technology and on-going services. The Company concluded that the license is not discrete as it does not have stand-alone value to Kite apart from the services to be performed by the Company pursuant to the agreement. As a result, the Company recognizes revenue from the upfront payment on a straight-line basis through June 2024, the estimated period the Company will perform research services. The estimated period of performance and project cost is reviewed quarterly and adjusted, as needed, to reflect the Company's current assumptions regarding the timing of its deliverables. As of September 30, 2018, the Company had deferred revenue of $137.8 million related to this agreement.

Revenues recognized under the agreement for the three and nine months ended September 30, 2018 were as follows (in thousands):

	September 30, 2018	
	Three Months Ended	Nine Months Ended
Revenue related to Kite Collaboration:		
Recognition of upfront fee	$6,296	$12,249
Research services	2,732	4,295
Total	$9,028	$16,544

Pfizer Inc.

SB-525 Global Collaboration and License Agreement

In May 2017, the Company entered into an exclusive, global collaboration and license agreement with Pfizer, pursuant to which it established a collaboration for the research, development and commercialization of SB-525, its gene therapy product candidate for hemophilia A, and closely related products.

Under this agreement, the Company is responsible for conducting the Phase 1/2 clinical trial and certain manufacturing activities for SB-525, while Pfizer is responsible for subsequent worldwide development, manufacturing, marketing and commercialization of SB-525. Sangamo may also collaborate in the research and development of additional AAV-based gene therapy products for hemophilia A.

The Company received an upfront fee of $70.0 million and is eligible to receive development milestone payments contingent on the achievement of specified clinical development, intellectual property, regulatory and first commercial sale milestones for SB-525 and potentially other products. In addition, Sangamo is eligible to receive up to $208.5 million in payments upon the achievement of specified clinical development, intellectual property and regulatory milestones and up to $266.5 million in payments upon first commercial sale milestones for SB-525 and potentially other products. The total amount of potential clinical development, intellectual property, regulatory, and first commercial sale milestone payments, assuming the achievement of all specified milestones in the hemophilia A Pfizer agreement, is up to $475.0 million, which includes up to $300.0 million for SB-525 and up to $175.0 million for other products that may be developed under the agreement, subject to reduction on account of payments made under certain licenses for third party intellectual property. In addition, Pfizer agreed to pay the Company royalties for each potential licensed product developed under the agreement that are an escalating tiered, double-digit percentage of the annual net sales of such product and are subject to reduction due to patent expiration, entry of biosimilar products to the market and payment made under certain licenses for third party intellectual property. To date, no milestone payments have been received and no products have been approved and therefore no royalty fees have been earned under the hemophilia A Pfizer agreement. Sangamo is responsible for internal and external research costs as part of the upfront fee and has the ability to request additional reimbursement from Pfizer if certain conditions are met.

None of the clinical or regulatory milestones have been included in the $70.0 million transaction price, as all milestone amounts are fully constrained. As part of its evaluation of the constraint, the Company considered numerous factors, including the fact that achievement of the milestones at this time is uncertain and contingent upon future periods when the uncertainty related to the variable consideration is resolved. The Company will re-evaluate the transaction price, including its estimated variable consideration included in the transaction price and all constrained amounts, in each reporting period and as uncertain events are resolved or other changes in circumstances occur.

Subject to the terms of the agreement, the Company granted Pfizer an exclusive, worldwide, royalty-bearing license, with the right to grant sublicenses, to use certain technology controlled by the Company for the purpose of developing, manufacturing and commercializing SB-525 and related products. Pfizer granted the Company a non-exclusive, worldwide, royalty free, fully paid license, with the right to grant sublicenses, to use certain manufacturing technology developed under the agreement and controlled by Pfizer to manufacture the Company's products that utilize the AAV delivery system. During a specified period, neither the Company nor Pfizer will be permitted to clinically develop or commercialize, outside of the collaboration, certain AAV-based gene therapy products for hemophilia A.

Unless earlier terminated, the agreement has a term that continues, on a per product and per country basis, until the later of (i) the expiration of patent claims that cover the product in a country, (ii) the expiration of regulatory exclusivity for a product in a country, and (iii) fifteen years after the first commercial sale of a product in a country. Pfizer has the right to terminate the agreement without cause in its entirety or on a per product or per country basis. The agreement may also be terminated by either party based on an uncured material breach by the other party or the bankruptcy of the other party. Upon termination for any reason, the license granted by the Company to Pfizer to develop, manufacture and commercialize SB-525 and related products will automatically terminate. Upon termination by the Company for cause or by Pfizer in any country or countries, Pfizer will automatically grant the

Company an exclusive, royalty-bearing license under certain technology controlled by Pfizer to develop, manufacture and commercialize SB-525 in the terminated country or countries.

The Company has identified the performance obligations within the hemophilia A Pfizer agreement as a license to the technology and on-going services. The Company concluded that the license is not discrete as it does not have stand-alone value to Pfizer apart from the services to be performed by the Company pursuant to the agreement. As a result, the Company recognizes revenue from the upfront payment based on proportional performance through 2020, the estimated period the Company will perform research services. The estimated period of performance and project cost is reviewed quarterly and adjusted, as needed, to reflect the Company's current assumptions regarding the timing of its deliverables. As of September 30, 2018, the Company had deferred revenue of $21.5 million related to this agreement.

Revenues recognized under the agreement for the three and nine months ended September 30, 2018 and 2017 were as follows (in thousands):

	Three Months Ended September 30,		Nine Months Ended September 30,	
	2018	2017	2018	2017
Revenue related to Pfizer Collaboration for SB-525:				
Recognition of upfront fee	$10,421	$6,624	$26,262	$10,384

C9ORF72 Research Collaboration and License Agreement

In December 2017, the Company entered into a separate exclusive, global collaboration and license agreement with Pfizer for the development and commercialization of potential gene therapy products that use ZFP transcription factors ("TFs") to treat amyotrophic lateral sclerosis ("ALS") and frontotemporal lobar degeneration ("FTLD") linked to mutations of the C9ORF72 gene. Pursuant to this agreement, the Company agreed to work with Pfizer on a research program to identify, characterize and preclinically develop ZFP-TFs that bind to and specifically reduce expression of the mutant form of the C9ORF72 gene.

The Company received a $12.0 million upfront payment from Pfizer and is eligible to receive up to $60.0 million in development milestone payments from Pfizer contingent on the achievement of specified preclinical development, clinical development and first commercial sale milestones, and up to $90.0 million commercial milestone payments if annual worldwide net sales of the licensed products reach specified levels. In addition, Pfizer will pay the Company royalties based on an escalating tiered, mid- to high-single digit percentage of the annual worldwide net sales of the licensed products. These royalty payments are subject to reduction due to patent expiration, entry of biosimilar products to the market and payments made under certain licenses for third party intellectual property. Each party will be responsible for the cost of its performance of the research program. Pfizer will be operationally and financially responsible for subsequent development, manufacturing and commercialization of the licensed products.

None of the clinical or regulatory milestones have been included in the $12.0 million transaction price, as all milestone amounts are fully constrained. As part of its evaluation of the constraint, the Company considered numerous factors, including the fact that achievement of the milestones at this time is uncertain and contingent upon future periods when the uncertainty related to the variable consideration is resolved. The Company will re-evaluate the transaction price, including is estimated variable consideration included in the transaction price and all constrained amounts, in each reporting period and as uncertain events are resolved or other changes in circumstances occur.

Subject to the terms of this agreement, the Company granted Pfizer an exclusive, royalty-bearing, worldwide, license under the Company's relevant patents and know-how to develop, manufacture and commercialize gene therapy products that use resulting ZFP-TFs that satisfy pre-agreed criteria. During a specified period, neither the Company nor Pfizer will be permitted to research, develop, manufacture or commercialize outside of the collaboration any ZFPs that specifically bind to the C9ORF72 gene.

Unless earlier terminated, the agreement has a term that continues, on a per licensed product and per country basis, until the later of (i) the expiration of patent claims that cover the licensed product in a country, (ii) the expiration of regulatory exclusivity for a licensed product in a country, and (iii) fifteen years after the first commercial sale of a licensed product in a major market country. Pfizer also has the right to terminate the agreement without cause in its entirety or on a per product or per country basis. The agreement may also be terminated by either party based on an uncured material breach by the other party or the bankruptcy of the other party. The agreement will also terminate if the Company is unable to identify any lead candidates for development within a specified period of time or if Pfizer elects not to advance a lead candidate beyond a certain development milestone within a specified period of time. Upon termination for any reason, the license granted by the Company to Pfizer to develop, manufacture and commercialize licensed products under the agreement will automatically terminate. Upon termination by the Company for cause or by Pfizer without cause for any licensed product or licensed products in any country or countries, the Company will have the right to negotiate with Pfizer to obtain a non-exclusive, royalty-bearing license under certain technology controlled by Pfizer to develop, manufacture and commercialize the licensed product or licensed products in the terminated country or countries.

Following termination by the Company for Pfizer's material breach, Pfizer will not be permitted to research, develop, manufacture or commercialize ZFPs that specifically bind to the C9ORF72 gene for a period of time. Following termination by Pfizer for the Company's material breach, the Company will not be permitted to research, develop, manufacture or commercialize ZFPs that specifically bind to the C9ORF72 gene for a period of time.

The Company has identified the performance obligations within this agreement as a license to the technology and on-going services. The Company concluded that the license is not discrete as it does not have stand-alone value to Pfizer apart from the services to be performed by the Company pursuant to the agreement. As a result, the Company recognizes revenue from the upfront payment based on proportional performance through March 31, 2019 the estimated period the Company will perform research services. The estimated period of performance and project cost is reviewed quarterly and adjusted, as needed, to reflect the Company's current assumptions regarding the timing of its deliverables. As of September 30, 2018, the Company had deferred revenue of $10.5 million related to this agreement. During the three and nine months ended September 30, 2018 the Company recognized revenue of $0.4 million and $1.5 million, respectively, related to the upfront fee that was received upon entering into the agreement.

Bioverativ, a Sanofi company.

In January 2014, the Company entered into an exclusive worldwide collaboration and license agreement with Bioverativ to develop therapeutics for hemoglobinopathies, focused on beta-thalassemia and sickle cell disease ("SCD"). Under the agreement, the Company is jointly conducting two research programs: the beta-thalassemia program and the SCD program. In the beta-thalassemia program, the Company is responsible for all discovery, research and development activities through the first human clinical trial. In the SCD program, both parties are responsible for research and development activities through the submission of an investigational new drug ("IND") application for ZFP therapeutics intended to treat SCD.

Under both programs, Bioverativ is responsible for subsequent worldwide clinical development, manufacturing and commercialization of licensed products developed under the agreement. At the end of the specified research terms for each program or under certain specified circumstances, Bioverativ has the right to step in and take over any of our remaining activities. Furthermore, the Company has an option to co-promote in the United States any licensed products to treat beta-thalassemia and SCD developed under the agreement, and Bioverativ will compensate the Company for such co-promotion activities. Subject to the terms of the agreement, the Company has granted Bioverativ an exclusive, royalty-bearing license, with the right to grant sublicenses, to use certain ZFP and other technology controlled by the Company for the purpose of researching, developing, manufacturing and commercializing licensed products developed under the agreement. The Company also granted Bioverativ a

non-exclusive, worldwide, royalty-free, fully paid license, with the right to grant sublicenses, under the Company's interest in certain other intellectual property developed pursuant to the agreement. During the term of the agreement, the Company is not permitted to research, develop, manufacture or commercialize, outside of the agreement, certain gene therapy products that target genes relevant to the licensed products.

Under the agreement, the Company received an upfront license fee of $20.0 million and is eligible to receive development and sales milestone payments upon the achievement of specified regulatory, clinical development and sales milestones. In addition, the Company will also be eligible to receive up to $115.8 million in payments upon the achievement of specified clinical development and regulatory milestones, as well as up to $160.5 million in payments upon the achievement of specified sales milestones. The total amount of potential regulatory, clinical development, and sales milestone payments, assuming the achievement of all specified milestones in the agreement, is up to $276.3 million. In addition, the Company will receive royalty payments for each licensed product that are a tiered double-digit percentage of annual net sales of each product. Bioverativ reimburses Sangamo for agreed upon costs incurred in connection with research and development activities conducted by Sangamo. To date, no milestone payments have been received and no products have been approved and therefore no royalty fees have been earned under the Bioverativ agreement.

The agreement may be terminated by (i) the Company or Bioverativ for the uncured material breach of the other party, (ii) the Company or Bioverativ for the bankruptcy or other insolvency proceeding of the other party; (iii) Bioverativ, upon 180 days' advance

written notice to the Company and (iv) Bioverativ, for certain safety reasons upon written notice to, and after consultation with, the Company. As a result, actual future milestone payments could be lower than the amounts stated above.

All contingent payments under the agreement, when earned, will be non-refundable and non-creditable. The transaction price of $75.7 million includes the upfront license fee of $20.0 million and $55.7 million estimated reimbursable service costs for identified research projects over the estimated performance period, as all milestone amounts are fully constrained. As part of its evaluation of the constraint, the Company considered numerous factors, including the fact that achievement of the milestones at this time is uncertain and contingent upon future periods when the uncertainty related to the variable consideration is resolved. The Company will re-evaluate the transaction price, including the estimated variable consideration included in the transaction price and all constrained amounts, in each reporting period and as uncertain events are resolved or other changes in circumstances occur. None of the clinical or regulatory milestones have been included in transaction price.

The Company has identified the performance obligations within this arrangement as a license to the technology and on-going research services activities. The Company concluded that the license is not discrete as it does not have stand-alone value to Bioverativ apart from the research services to be performed pursuant to the agreement. As a result, the Company recognizes revenue from the upfront payment based on proportional performance through 2022, the estimated period the Company will perform research services. The estimated period of performance and project cost is reviewed quarterly and adjusted, as needed, to reflect the Company's current assumptions regarding the timing of its deliverables. As of September 30, 2018, the Company had deferred revenue of $5.1 million related to this agreement.

Revenues recognized under the agreement for the three and nine months ended September 30, 2018 and 2017 were as follows (in thousands):

	Three Months Ended September 30,		Nine Months Ended September 30,	
	2018	2017	2018	2017
Revenue related to Bioverativ agreement:				
Recognition of upfront fee	$1,094	$442	$3,432	$1,326
Research services	1,146	2,959	7,707	7,736
Total	$2,240	$3,401	$11,139	$9,062

California Institute for Regenerative Medicine

In May 2018, the California Institute for Regenerative Medicine ("CIRM") granted a Strategic Partnership Award for $8.0 million to fund the clinical studies of a potentially curative ZFP Therapeutic for the treatment of beta-thalassemia based on the application of Sangamo's ZFN genome editing technology. The grant exists through December 31, 2022 and provides matching funds to support the evaluate ST-400, a gene-edited cell therapy candidate for people with transfusion-dependent beta-thalassemia. As of September 30, 2018, the Company had received $1.7 million under the award.

Under the terms of the CIRM grants, the Company is obligated to pay royalties and licensing fees based on a low single digit royalty percentage on net sales of CIRM-funded product candidates or CIRM-funded technology. The Company has the option to decline any and all amounts awarded by CIRM and as an alternative to revenue sharing, the Company has the option to convert the award to a loan. No such election has been made as of the date of the

issuance of these financial statements. In the event that the Company terminates a CIRM-funded clinical trial, it will be obligated to repay the remaining CIRM funds on hand, therefore as of September 30, 2018, the $1.7 million related to this award is recorded as a loan in other long-term liabilities on the accompanying consolidated balance sheet.

Shire International GmbH

In January 2012, the Company entered into a collaboration and license agreement with Shire to research, develop and commercialize a ZFP therapeutic for treating Huntington's disease. The Company received an upfront license fee of $13.0 million. In 2014, Sangamo recognized a $1.0 million milestone payment related to the hemophilia program. Shire does not have any milestone payment obligations, but is required to pay single digit percentage royalties to the Company, up to a specified maximum cap, on the commercial sales of therapeutic products for Huntington's disease. The Company is required to pay single digit percentage royalties to Shire, up to a specified maximum cap, on commercial sales of therapeutic products from programs returned under the original agreement (which include blood clotting Factors VIII and IX) that use two zinc fingers.

Pursuant to the agreement, the Company granted Shire an exclusive, world-wide, royalty-bearing license, with the right to grant sublicenses, to use the Company's ZFP technology for the purpose of developing and commercializing human therapeutic and diagnostic products for the HTT gene. During the term of the agreement, the Company is not permitted to research, develop or commercialize, outside of the agreement, certain products that target the HTT gene. The Company satisfied the deliverables and research services responsibilities within the amended arrangement which were completed in 2017. The agreement may be terminated by (i) the Company or Shire, in whole or in part, for the uncured material breach of the other party, (ii) the Company or Shire for the

bankruptcy or other insolvency proceeding of the other party and (iii) Shire, in its entirety, effective upon at least 90 days' advance written notice.

The Company has concluded that the license is not a separate unit of accounting as it does not have stand-alone value to Shire apart from the research services to be performed pursuant to the Shire agreement. The Company satisfied the deliverables and research services responsibilities within the amended arrangement which were completed in 2017. As a result, the Company recognized the remaining $2.3 million of deferred revenue from the upfront payment during the year ended December 31, 2017.

Revenues recognized under the agreement for the three and nine months ended September 30, 2018 and 2017 were as follows (in thousands):

	Three Months Ended September 30,		Nine Months Ended September 30,	
	2018	2017	2018	2017
Revenue related to Shire agreement:				
Recognition of upfront fee	$—	$1,166	$—	$2,333
Research services	—	6	—	116
Total	$—	$1,172	$—	$2,449

NOTE 6—INCOME TAXES

On December 22, 2017, the U.S. government enacted comprehensive tax legislation commonly referred to as the Tax Cuts and Jobs Act (the "Tax Act"). The Tax Act makes broad and complex changes to the U.S. tax code that affected 2017, the current year and onwards, including, but not limited to, a reduction of the U.S. federal corporate tax rate from as high as 35% to 21%, a general elimination of U.S. federal income taxes on dividends from foreign subsidiaries, net operating loss deduction limitations, and 100% disallowance of entertainment expense.

In addition, on December 22, 2017, the SEC staff issued Staff Accounting Bulletin No. 118 ("SAB 118") which provides guidance on accounting for the tax effects of the Tax Act. SAB 118 provides a measurement period that should not extend beyond one year from the Tax Act enactment date for companies to complete the accounting under Accounting Standards Codification 740, Income taxes for the year ended December 31, 2017. In accordance with SAB 118, a company must reflect the income tax effects of those aspects of the Tax Act for which the accounting under ASC 740 is complete. The Company is still within the measurement period as of September 30, 2018 and no further conclusions have been made, as the Company reviews the law change and the impact to the Company.

Due to the Company's valuation allowance against its deferred tax assets, it does not expect that the provisions of the Tax Act will have a material impact on the Company's financial position, results of operations, or income tax expense or benefit.

NOTE 7—STOCK-BASED COMPENSATION

The following table shows total stock-based compensation expense included in the condensed consolidated statements of operations for the three and nine months ended September 30, 2018 and 2017 (in thousands):

	Three Months Ended September 30,		Nine Months Ended September 30,	
	2018	2017	2018	2017
Research and development	$2,093	$1,331	$5,972	$3,766
General and administrative	1,717	888	4,402	3,203
Total stock-based compensation expense	$3,810	$2,219	$10,374	$6,969

NOTE 8—COMMITMENTS AND CONTINGENCIES

Brisbane Build-to-Suit Lease

In November 2017, the Company entered into a long-term property lease which includes construction by the lessor of a building with approximately 87,700 square feet of space, in Brisbane, California. Substantial completion of the building is estimated to occur in the last quarter of 2018. The lease agreement expires in May 2029, approximately ten years after substantial completion of the building. A letter of credit for $3.5 million was established as the deposit and is classified as restricted cash within restricted cash and

other noncurrent assets in the accompanying financial statements. The Company has two options to extend the lease term for up to a combined additional ten years.

The Company is deemed, for accounting purposes only, to be the owner of the entire project including the building shell, even though it is not the legal owner as a result of the cold shell condition of the building and involvement in the construction process. In connection with the Company's accounting for this transaction, the Company capitalized the costs of construction as a build-to-suit property within property and equipment, net, and recognize a corresponding build-to-suit lease obligation, including interest. Fair value of the building was estimated at $20.9 million using comparable market prices per square foot for similar space for public real estate transactions in the surrounding area and is considered a Level 2 fair value measurement. As of September 30, 2018, $39.5 million was capitalized with a corresponding build-to-suit lease obligation recognized related to this lease for the building and construction costs.

Contingencies

Sangamo is not party to any material pending legal proceedings or contingencies. From time to time, the Company may be involved in legal proceedings arising in the ordinary course of business.

NOTE 9— STOCKHOLDERS' EQUITY

In April 2018, Sangamo completed an underwritten public offering of its common stock, in which the Company sold an aggregate of 14.2 million shares of its common stock at a public offering price of $16.25 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 $215.8 million.

In May 2017, the Company entered into an amended and restated sales agreement with Cowen and Company, LLC ("Cowen") (the "ATM Facility") pursuant to which the Company may offer and sell, in its sole discretion, shares of common stock having an aggregate offering price of up to $75.0 million through Cowen acting as the Company's sales agent. Sales of the Company's common stock, if any, will be made at market prices by any method that is deemed to be an "at the market offering" as defined in Rule 415 under the Securities Act of 1933, as amended. The Company has not sold any common stock under the ATM Facility. As of September 30, 2018, the full $75.0 million provided for under the ATM Facility remained available for sale, subject to certain conditions as specified in the agreement.

NOTE 10— SUBSEQUENT EVENTS

On July 20, 2018, the Company entered into a Share Purchase Agreement (the "SPA") with certain shareholders (the "Sellers") of TxCell, and the Company and TxCell entered into a Tender Offer Agreement (the "TOA"). Pursuant to the SPA and the TOA, the Company expects to acquire 100% of the equity interests of TxCell for approximately €72 million, on a debt-free and cash-free basis.

On October 1, 2018, pursuant to the SPA, the Company purchased all of the ordinary shares of TxCell the "Ordinary Shares") held by the Sellers for €2.58 per share in cash (such per share price being the "Offer Price" and such purchase

being the "Block Transaction"). The Sellers owned 13,519,036 Ordinary Shares, which represented approximately 53% of the share capital and voting rights of TxCell. Subsequent to the completion of the Block Transaction, as of November 7, 2018, we owned approximately 80% of the share capital and voting rights of TxCell.

Promptly following the completion of the Block Transaction, the Company designated a number of directors on the board of directors of TxCell representing a majority of the TxCell board.

Pursuant to the TOA, on November 1, 2018, the Company, commenced a cash tender offer (the "Offer") to acquire all of the Ordinary Shares of TxCell not held by the Company for the Offer Price. In addition, the Company has agreed to: (a) grant to certain employees (including certain members of management) of TxCell stock options to purchase approximately 150,000 shares of Company common stock, which will be granted under the Company's existing 2018 Equity Incentive Plan, with standard vesting conditions; and (b) enter into arrangements with holders of 495,396 "free shares" of TxCell, pursuant to which the Company would purchase such shares from the holders thereof from time to time through mid-2021. The purchase price for each such free share will be based on the performance of the Company's stock price from the announcement of the transactions contemplated by the SPA and TOA (at which time each free share was valued at €2.58 per share through the time of purchase (such that, for example, if the Company's stock price doubles during that time period, the value of each free share would similarly double).

The Sellers and TxCell have made limited representations and warranties in the TOA as are customary for such an agreement governed under French law. The TOA also contains customary termination rights.

If, following completion of the Offer, as it may be extended, the Company owns at least 95% of the share capital and voting rights of TxCell, it plans to acquire the remaining Ordinary Shares for the Offer Price through a compulsory squeeze-out procedure under French law. At this time, the Company is assessing the accounting impact of the agreement.

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," "strategy," "will," "intend" 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 our actual results, performance or achievements, or industry results, to be materially different from any future results, performance or achievements expressed or implied by such forward-looking statements, including but not limited to those described under the caption "Risk Factors" in Part II, Item 1A of this Quarterly Report on Form 10-Q. 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, 2017, or the 2017 Annual Report, as filed with the Securities and Exchange Commission, or SEC, on March 1, 2018.

Overview

We are a clinical stage biotechnology company focused on translating ground-breaking science into genomic therapies that transform patients' lives using our platform technologies in genome editing, gene therapy, gene regulation and cell therapy.

We are a leader in the research and development of zinc finger proteins, or ZFPs, a naturally occurring class of proteins found in humans. We have used our knowledge and expertise to develop a proprietary technology platform in both genome editing and gene regulation. ZFPs can be engineered to make zinc finger nucleases, or ZFNs, proteins that can be used to specifically modify DNA sequences by adding or knocking out specific genes, or genome editing, and ZFP transcription factors, or ZFP TFs, proteins that can be used to increase or decrease gene expression, or gene regulation. In the process of developing this platform, we have accrued significant scientific, manufacturing and regulatory capabilities and know-how that are generally applicable in the broader field of gene therapy and have capitalized this knowledge into a conventional gene therapy platform.

With the acquisition of Tx Cell S.A., or TxCell, we are now also focused on the development of platforms for innovative, personalized T-cell immunotherapies for the treatment of severe inflammatory and autoimmune diseases with high unmet medical need. Through our subsidiary, TxCell, we believe we will accelerate our entry in to the clinic with a CAR-Treg (which is a regulatory T cell, or Treg, genetically modified with a chimeric antigen receptor, or CAR) therapy. We are targeting solid organ transplantation as well as a range of autoimmune diseases, such as multiple sclerosis, rheumatoid arthritis, inflammatory bowel diseases or inflammatory skin diseases. In addition, we intend to use our ZFN gene editing technology to potentially develop next-generation autologous and allogeneic CAR-Treg cell therapies for use in treating autoimmune diseases.

Our strategy is to maximize the value and therapeutic use of our technology platforms. In certain therapeutic areas we intend to capture the value of our proprietary genome editing and gene therapy products by forward integrating into manufacturing, development and commercial operations. In other therapeutic areas we intend to partner with biopharmaceutical companies to develop products.

We have an ongoing Phase 1/2 clinical trial evaluating SB-525, a gene therapy for the treatment of hemophilia A, a bleeding disorder. We also have ongoing Phase 1/2 clinical trials evaluating three product candidates using

our proprietary in vivo genome editing approach: SB-FIX for the treatment of hemophilia B, a bleeding disorder; SB-318, for the treatment of Mucopolysaccharidosis Type I, or MPS I; and SB-913 for the treatment of Mucopolysaccharidosis Type II, or MPS II. MPS I and MPS II are rare lysosomal storage disorders, or LSDs. We also have an ongoing Phase1/2 clinical trial evaluating ST-400, developed using our proprietary ZFN-mediated ex vivo cell therapy platform, for the treatment of beta-thalassemia, a blood disorder. We also plan to initiate a Phase 1/2 clinical trial of for TxCell's first CAR-Treg investigational product candidate for solid organ transplant, or TX 200, in 2019.

In August 2018, we announced positive preliminary data from the Phase 1/2 clinical trial evaluating SB-525, a cDNA gene therapy candidate for hemophilia A, or the Alta study. SB-525 is being developed as part of a global collaboration between us and Pfizer Inc. for the development and commercialization of potential gene therapy programs for hemophilia A. In October 2018, the independent safety monitoring committee, or SMC, of the Phase 1/2 Alta Study evaluating SB-525 for hemophilia A reviewed accumulated safety and efficacy data from the six patients enrolled in three dose cohorts. As of that review, SB-525 exhibited dose dependent efficacy on serum factor levels and was generally well-tolerated with no treatment-related serious adverse events and no use of tapering courses of oral steroids. The SMC recommended that the study continue with escalation to an additional dose. We plan to present safety and efficacy data from the Alta Study after dose escalation is complete and the clinical trial has progressed to the cohort expansion phase.

In October 2018, the SMC reviewed accumulated safety and efficacy data from both the EMPOWERS Study and the CHAMPIONS Study. In accordance with the recommendation of the SMC, the second patient enrolled in the EMPOWERS Study received the 5e13 vg/kg dose, or the highest dose.

We also announced in September 2018 preliminary safety and efficacy data from the Phase 1/2 clinical trial evaluating SB-913 for the treatment of MPS II, or the CHAMPIONS Study. In cohort 2 of the CHAMPIONS study, at 16 weeks post-dosing, mean reductions were observed in total urinary glycosaminoglycans, or GAGs (which is a key biomarker of MPS II disease pathophysiology), dermatan sulfate, and heparan sulfate of 51%, 32%, and 61%, respectively. Due to the sensitivity of the current assay we are utilizing to measure plasma iduronate-2-sulfatase, or IDS, enzyme levels, we were unable to detect IDS in any of the patients over the 16 weeks following treatment with SB-913. In October, the independent SMC of the CHAMPIONS Study reviewed accumulated safety and efficacy data from all three cohorts and made the following three recommendations: 1) proceed to the cohort expansion phase of the clinical trial with the dose used at the third dose cohort (5e13 vg/kg); 2) initiate screening and enrollment of adolescent subjects (12 to 17 years of age); and 3) initiate the withdrawal of enzyme replacement therapy, or ERT, when appropriate.

In addition, we have proprietary preclinical and discovery stage programs in other LSDs, hematological disorders and monogenic diseases, including certain central nervous system, or CNS, disorders, cancer immunotherapy, immunology and infectious disease.

In October 2018, we completed the acquisition of approximately 53% of the outstanding share capital and voting rights of TxCell for approximately €34.9 million pursuant to a July 2018 Share Purchase Agreement with TxCell and certain of its shareholders, or the SPA. We refer to this purchase as the Block Transaction. Following our acquisition of control of TxCell, TxCell operates as our subsidiary. Subsequent to the completion of the Block Transaction, as of November 7, 2018, we owned approximately 80% of the share capital and voting rights of TxCell. We recently initiated a simplified cash tender offer to acquire the remaining ordinary shares of TxCell at a price of €2.58 per share, the same price per share paid in the Block Transaction as contemplated by the July 2018 Tender Offer Agreement between Sangamo and TxCell, or the TOA. We expect to close the tender offer late in fourth quarter of 2018. If we successfully acquire at least 95% of the outstanding ordinary shares of TxCell, we will launch a squeeze out procedure to acquire any remaining TxCell ordinary shares. Following the expected squeeze out procedure, we plan to delist TxCell from Euronext Paris. For more information relating to the acquisition of TxCell, or the TxCell Acquisition, see Note 10 to our consolidated financial statements appearing elsewhere in this Quarterly Report on Form 10-Q.

In February 2018, we entered into a global collaboration and license agreement with Kite Pharma, Inc., or Kite, a wholly owned subsidiary of Gilead Sciences, Inc., for the research, development and commercialization of potential engineered cell therapies for cancer. The Kite agreement became effective in April 2018 when the waiting periods under the Hart-Scott-Rodino Antitrust Improvements Act of 1976, as amended and other customary closing conditions were completed. In this collaboration, we are working together with Kite on a research program under which we are designing ZFNs and AAVs to disrupt and insert certain genes in T cells and natural killer, or NK, cells, including the insertion of genes that encode chimeric antigen receptors, T-cell receptors, and NK-cell receptors directed to mutually agreed targets. Kite is responsible for all clinical development and commercialization of any resulting products.

In December 2017, we entered into a research collaboration and license agreement with Pfizer Inc., or Pfizer, for the development and commercialization of potential gene therapy products that use ZFP TFs to treat amyotrophic lateral sclerosis, or ALS, and frontotemporal lobar degeneration, or FTLD, linked to mutations of the C9ORF72 gene. Under this agreement, we are working with Pfizer on a research program to identify, characterize and preclinically develop

ZFP TFs that satisfy pre-agreed criteria. Pfizer is responsible for subsequent development, manufacturing and commercialization of licensed products.

In May 2017, we entered into a global collaboration and license agreement with Pfizer for the research, development and commercialization of SB-525, our gene therapy product candidate for hemophilia A, and closely related products. Under this agreement, we are responsible for conducting the Phase 1/2 clinical trial and certain manufacturing activities for SB-525, while Pfizer is responsible for subsequent worldwide development, manufacturing, marketing and commercialization of SB-525. We and Pfizer may also collaborate in the research and development of additional AAV-based gene therapy products for hemophilia A.

We have also established a collaborative partnership with Bioverativ, a Sanofi company, or Bioverativ, to research, develop and commercialize therapeutic gene-edited cell therapy products in hemoglobinopathies, including beta-thalassemia and sickle cell disease, or SCD. Bioverativ is responsible for subsequent development, manufacturing and commercialization of licensed products.

We have a substantial intellectual property position in the genome editing field including the design, selection, composition and use of engineered ZFPs to support our research and development activities. With the TxCell Acquisition, we also have gained an intellectual property position in the Treg and CAR-Treg fields. As of September 30, 2018, we either owned outright or have exclusively licensed the commercial rights to over 904 patents issued in the United States and foreign jurisdictions, and over 702 patent applications pending worldwide. We continue to license and file new patent applications that strengthen our core and accessory

patent portfolio. We believe that our intellectual property position is a critical element in our ability to research, develop and commercialize products and services based on genome editing, gene therapy, gene regulation and cell therapy.

Comparability

We adopted Accounting Standards Codification Topic 606—Revenue from Contracts with Customers, or Topic 606, on January 1, 2018, resulting in a change to our accounting policy for revenue recognition. We used the modified retrospective method and recognized the cumulative effect of initially applying Topic 606 as an adjustment to the opening balances of deferred revenues and accumulated deficit at January 1, 2018. Accordingly, comparative information has not been adjusted and continues to be reported under previous accounting standards. Refer to Note 1 in Part I, Item 1 of this Quarterly Report on Form 10-Q for additional information.

Critical Accounting Estimates

The accompanying discussion and analysis of our financial condition and results of operations are based upon our condensed consolidated financial statements and the related disclosures, which have been prepared in accordance with accounting principles generally accepted 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 condensed 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. Except for the change to our accounting policy for revenue recognition as a result of adopting Topic 606, there have been no significant changes in our critical accounting policies and estimates disclosed in our 2017 Annual Report.

Results of Operations

Three and nine months ended September 30, 2018 and 2017

Revenues

	Three Months Ended September 30, (in thousands, except percentage values)				Nine Months Ended September 30, (in thousands, except percentage values)			
	2018	2017	Change	%	2018	2017	Change	%
Revenues:								
Collaboration agreements	$23,538	$11,759	$11,779	100%	$57,378	$23,042	$34,336	149%
Research grants	24	53	(29)	(55%)	237	448	(211)	(47%)
Total revenues	$23,562	$11,812	$11,750	99%	$57,615	$23,490	$34,125	145%

Total revenues consisted of revenues from collaboration agreements and research grants. We anticipate revenues over the next several years will be derived primarily from our collaboration agreements with Kite, Pfizer and Bioverativ as we continue to recognize in revenues upfront payments received under such agreements over time.

The increase in revenues from our collaboration agreements for the three months ended September 30, 2018 were primarily due to $9.0 million in revenues related to the Kite agreement, which took effect in April 2018 and $3.8 million in revenues related to the hemophilia A Pfizer agreement, partially offset by a decrease of $1.2 million in revenue related to our agreement with Shire International GmbH, formerly Shire AG, or Shire, as we recognized the remaining deferred revenue under such agreement in December 2017, and $1.2 million in revenues related to our agreement with Bioverativ. Revenues from Kite included $6.3 million related to partial recognition of an upfront license fee of $150.0 million and $2.7 million from research services. The revenues from Pfizer reflect the partial recognition of an upfront fee of $70.0 million under the hemophilia A Pfizer agreement and upfront fee of $12.0 million under the C9ORF72 Pfizer agreement. Revenues from Bioverativ included $1.1 million related to partial recognition of an upfront license fee of $20.0 million and $1.1 million from research services. Research grant revenues were approximately $0.0 million and $0.1 million for the three months ended September 30, 2018 and 2017, respectively.

The increase in revenues from our collaboration agreements for the nine months ended September 30, 2018 was primarily attributable to $16.5 million in revenues related to our agreement with Kite, $15.9 million in revenues related to the hemophilia A Pfizer Agreement, $2.0 million in revenues related to our agreement with Bioverativ, and $1.5 million in revenues related to the C9ORF72 Pfizer agreement, partially offset by a decrease of $2.4 million in revenue from our agreement with Shire. Research grant revenues were approximately $0.2 million and $0.4 million for the nine months ended September 30, 2018 and 2017, respectively.

Operating Expenses

	Three Months Ended September 30, (in thousands, except percentage values)				Nine Months Ended September 30, (in thousands, except percentage values)			
	2018	2017	Change	%	2018	2017	Change	%
Operating expenses:								
Research and development	$28,810	$18,425	$10,385	56%	$81,612	$46,351	$35,261	76%
General and administrative	10,993	6,422	4,571	71%	32,381	19,734	12,647	64%
Total expenses	$39,803	$24,847	$14,956	60%	$113,993	$66,085	$47,908	72%

Research and Development Expenses

Research and development expenses consist primarily of salaries and personnel-related expenses, including stock-based compensation, laboratory supplies, preclinical and clinical studies, manufacturing expenses, allocated facilities expenses, contracted research expenses and expenses for technology licenses. We expect to continue to devote substantial resources to research and development in the future, including in connection with the TxCell Acquisition, and expect research and development expenses to increase in the next several years if we are successful in advancing our clinical programs and if we are able to progress our earlier stage product candidates into clinical trials.

The increase of $10.4 million in research and development expenses for the three months ended September 30, 2018 was primarily due to increases of $4.6 million in manufacturing and clinical trial expenses as our programs move further into the clinic. A portion of the increase was also due to $3.0 million in salaries and benefits expense, $1.0 million in lab supply expense, $0.8 million in research and pre-clinical expense, and $0.8 million in stock-based compensation expense. The increases were primarily due to the growth of our business to support the continued advancement of our product candidates into clinical trials.

The increase of $35.3 million in research and development expenses for the nine months ended September 30, 2018 was primarily due to increases of $16.9 million in manufacturing and research expenses as our programs move further into the clinic, $7.8 million in salaries and benefits expense, $3.4 million in research and pre-clinical expense, $2.8 million in lab supply expense, $2.2 million in stock-based compensation expense, $1.3 million in facility expense, and $0.5 million in consultant expense. The increases were primarily due to the growth of our business to support the continued advancement of our product candidates into clinical trials.

General and Administrative Expenses

The increase of $4.6 million in general and administrative expenses for the three months ended September 30, 2018 was primarily due to increases of $1.3 million in legal expense, $1.1 million in salaries and benefits expense, $0.8 million in stock-based compensation expense, $0.7 million in consultant expense, and $0.6 million in facility expense. The increases were primarily due to the growth of our business to support the continued advancement of our product candidates into clinical trials.

The increase of $12.6 million in general and administrative expenses for the nine months ended September 30, 2018 was primarily due to increases of $3.4 million in salaries and benefits expense, $3.2 million in consultant expense,

$1.6 million in legal expense, $1.4 million in facility expense, $1.2 million in stock-based compensation expense, $0.5 million in audit expense, and $0.3 million in travel expense.

Interest and other income, net

The increase of $2.7 million and $5.6 million in interest and other income, net, for the three and nine months ended September 30, 2018 and 2017, respectively, were primarily due a transition from government securities to corporate securities.

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, 2018, we had cash, cash equivalents, marketable securities and interest receivable, totaling $459.3 million, excluding restricted cash, compared to $244.6 million as of December 31, 2017, with the increase primarily attributable to $215.8

million net proceeds from our April 2018 issuance of common stock and $150.0 million from our February 2018 collaboration and license agreement with Kite, which became effective in April 2018.

Our most significant use of capital pertains to salaries and benefits for our employees and external research and development expenses, such as manufacturing, clinical trials and preclinical activity related to our therapeutic programs. Our cash and investment balances are held in a variety of interest bearing instruments, including 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.

In October 2018, we closed the Block Transaction of the TxCell Acquisition, acquiring 53% of the ordinary shares of TxCell, and recently launched a cash tender offer as contemplated by the TOA. In the TxCell Acquisition, we expect to acquire 100% of the equity interests of TxCell for approximately €72 million, on a debt-free and cash-free basis. See "—Overview" above for further discussion of the TxCell Acquisition.

In April 2018, we completed an underwritten public offering of our common stock, in which we sold an aggregate of 14.2 million shares of our common stock at a public offering price of $16.25 per share. The net proceeds to us from the sale of shares in this offering, after deducting underwriting discounts and commissions and other estimated offering expenses, were approximately $215.8 million.

In February 2018, we entered into a global collaboration and license agreement with Kite for the research, development and commercialization of potential engineered cell therapies for cancer. In April 2018, the Kite agreement became effective when the waiting periods under the Hart-Scott-Rodino Antitrust Improvements Act of 1976, as amended and other customary closing conditions were satisfied. Following the effective date, we received a $150.0 million upfront payment from Kite.

In May 2017, we entered into an amended and restated sales agreement with Cowen and Company, LLC pursuant to which we may offer and sell, in our sole discretion, shares of common stock having an aggregate offering price of up to $75.0 million through Cowen acting as our sales agent. Sales of our common stock, if any, will be made at market prices by any method that is deemed to be an "at the market offering" as defined in Rule 415 under the Securities Act of 1933, as amended. As of September 30, 2018, the full $75.0 million provided for under the sales agreement remained available for sale, subject to certain conditions as specified in the agreement.

Cash Flows

Operating activities. Net cash provided by operating activities was $76.7 million for the nine months ended September 30, 2018, primarily reflecting the increase in deferred revenue due to the $150.0 million upfront license payment from Kite, and stock-based compensation for the period offset by the net loss and increase in other assets. Net cash provided by operating activities for the nine months ended September 30, 2017 primarily reflected increases in deferred revenue as a result of the hemophilia A Pfizer Agreement, accrued liabilities, and stock-based compensation for the period offset by the net loss and decrease in prepaid assets.

Investing activities. Net cash used in investing activities for the nine months ended September 30, 2018 and 2017, was $240.9 million and $105.4 million, respectively. Cash flows from investing activities for both periods primarily related to purchases and maturities of investments.

Financing activities. Net cash provided by financing activities for the nine months ended September 30, 2018 was $230.1 million primarily related to our April 2018 underwritten public offering of our common stock, which generated net proceeds of approximately $215.8 million, with the remainder primarily related to the $14.5 million from the

issuance of common stock upon exercise of stock options. Net cash provided by financing activities for the nine months ended September 30, 2017 was $85.2 million primarily related to the completion of our June 2017 underwritten public offering of our common stock, which generated net proceeds of approximately $78.1 million.

Operating Capital and Capital Expenditure Requirements

We anticipate continuing to incur operating losses for at least the next several years. We plan to finance operations with available cash resources, collaborations and strategic partnerships, research grants and from the issuance of equity or debt securities. We believe that our available cash, cash equivalents, marketable securities and interest receivable as of September 30, 2018, along with expected revenues from collaborations, strategic partnerships and research grants, will be adequate to fund our operations at least through the next twelve months. However, we will need to raise substantial additional capital to fund the development, manufacturing and potential commercialization of our product candidates. 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 gene therapy products would be harmed. Furthermore, any sales of additional equity securities may result in dilution to our stockholders, and any debt financing may include covenants that restrict our business.

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

- the initiation, progress, timing and completion of clinical trials for our product candidates and potential product candidates;
- the outcome, timing and cost of regulatory approvals;
- the success of our collaboration agreements;
- 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 and timing of establishing sales, marketing, manufacturing and distribution capabilities;
- 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, including the costs associated with such acquisitions and investments, such as the TxCell Acquisition; and
- the possible costs of litigation.

Off-Balance Sheet Arrangements

We do not have any off-balance sheet arrangements as defined in Regulation S-K, Item 303(a)(4)(ii) of Regulation S-K.

Contractual Obligations and Commercial Commitments

Our future minimum contractual commitments were reported in our 2017 Annual Report and there have been no material changes outside the ordinary course of business in the previously disclosed contractual commitments during the nine months ended September 30, 2018. See "—Overview" above and Note 10 to our consolidated financial statements appearing elsewhere in this Quarterly Report on Form 10-Q for a discussion of the TxCell Acquisition, which increased our contractual commitments in July 2018.

ITEM 3. QUANTITATIVE AND QUALITATIVE DISCLOSURES ABOUT MARKET RISK

Our exposure to market risk relates to our cash, cash equivalents and investments. The goals of our investment policy are preservation of capital, fulfillment of liquidity needs and capturing a market rate of return based on our investment policy parameters and market conditions. We select investments that maximize interest income to the extent possible within these guidelines. To achieve our goals, we maintain a portfolio of cash equivalents and investments in securities of high credit quality and with varying maturities to match projected cash needs.

The securities in our investment portfolio are not leveraged, are classified as available-for-sale and are, due to their short-term nature, subject to minimal interest rate risk. Our investments currently consist of U.S. Treasury securities, U.S. government-sponsored enterprise securities and corporate notes. Our investment policy, approved by our Board of Directors, limits the amount we may invest in any one type of investment issuer, thereby reducing credit risk concentrations. All investments have a fixed interest rate and are carried at market value, which approximates cost. We do not use derivative financial instruments in our investment portfolio. We do not believe that a change in interest rates would have a material negative impact on the value of our investment portfolio. Our market risks at September 30, 2018 have not changed materially from those discussed in Item 7A of our 2017 Annual Report.

ITEM 4. CONTROLS AND PROCEDURES

Evaluation of 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 principal executive officer and principal financial officer, as appropriate, to allow timely decisions regarding required disclosure.

Under the supervision of our principal executive officer and principal financial officer, we evaluated the effectiveness of our disclosure controls and procedures (as defined in Rules 13a-15(e) and 15d-15(e) of the Exchange Act) as of September 30, 2018. Based on that evaluation, our principal executive officer and principal financial officer have concluded that our disclosure controls and procedures were effective at the reasonable assurance level.

Inherent Limitations on Controls and Procedures

Our management, including the principal executive officer and principal financial officer, does not expect that our disclosure controls and procedures and our internal control over financial reporting will prevent all error and all fraud. A control system, no matter how well designed and operated, can only provide reasonable assurances that the objectives of the control system are met. The design of a control system reflects resource constraints; the benefits of controls must be considered relative to their costs. Because there are inherent limitations in all control systems, no evaluation of controls can provide absolute assurance that all control issues and instances of fraud, if any, for our company have been or will be detected. As these inherent limitations are known features of the disclosure and financial reporting processes, it is possible to design into the processes safeguards to reduce, though not eliminate, these risks. These inherent limitations include the realities that judgments in decision-making can be faulty and that breakdowns occur because of simple error or mistake. Controls can also be circumvented by the individual acts of some persons, by collusion of two or more people, or by management override of the control. The design of any system of controls is based in part upon certain assumptions about the likelihood of future events. While our disclosure controls and procedures and our internal control over financial reporting are designed to provide reasonable assurance of achieving their objectives, there can be no assurance that any design will succeed in achieving its stated goals under all future conditions. Over time, controls may become inadequate because of changes in conditions or deterioration in the degree of compliance with the policies or procedures. Because of the inherent limitations in a cost-effective control system, misstatements due to error or fraud may occur and not be detected.

Change in Internal Control over Financial Reporting

There has been no change in our internal control over financial reporting that occurred during the three months ended September 30, 2018 that has materially affected, or is reasonably likely to materially affect, our internal control over financial reporting.

PART II. OTHER INFORMATION

ITEM 1. LEGAL PROCEEDINGS

We are not party to any material pending legal proceedings. From time to time, we may be involved in legal proceedings arising in the ordinary course of business.

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 contained herein and in our Annual Report on Form 10-K filed with the Securities and Exchange Commission on March 1, 2018, including our consolidated financial statements and related notes, before making an investment decision regarding our common stock. If any of the events described in the following risk factors occurs, our business, operating results and financial condition could be seriously harmed. Unless otherwise indicated or the context suggests otherwise, references in this Quarterly Report on Form 10-Q to "Sangamo," "we," "us," and "our" refer to Sangamo Therapeutics, Inc. and our consolidated subsidiaries, including TxCell S.A.

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

Our success depends substantially on the results of clinical trials of our lead therapeutic programs, and we may not be able to demonstrate safety and efficacy of our product candidates.

We do not have any products that have gained regulatory approval. Our failure to enroll sufficient patients to conduct the trials, demonstrate safety or obtain positive clinical trial results, or our inability to meet the expected timeline of clinical trials or release of data for these programs, would have a material adverse effect on our business operations and financial conditions, which may cause a significant decline in our stock price.

Our ability to conduct clinical trials successfully and on a timely basis for these programs is subject to a number of additional risks, including but are not limited to the following:

•disagreement with the design or implementation of our clinical trials;

•the ability to identify and recruit sufficient number of acceptable patients to complete enrollment of trials;

•failure to demonstrate that a product candidate is safe and effective for its proposed indication;

•the occurrence of unexpected adverse events or toxicity;

•disagreement with the U.S. Food and Drug Administration, or FDA, or foreign regulatory authorities, on the interpretation of data from preclinical studies or our clinical trial results;

•failure of clinical trials to meet the level of statistical significance required for approval;

•the insufficiency of data collected from clinical trials of our product candidates to support the submission and filing of a biologics license application, or BLA, or other submission or to obtain regulatory approval;

•changes in the approval policies or regulations that render our preclinical and clinical data insufficient for approval;

•failure to obtain approval of our manufacturing processes or facilities of third-party manufacturers with whom we contract for clinical and commercial supplies or our own manufacturing facility;

•defects in the preparation and manufacturing of our product candidates;

•failure by third parties, including vendors, manufacturers and clinical trial organizations, to provide timely and adequate supplies and services;

•development of similar gene therapies by our competitors;

•unexpected costs and expenses and lack of sufficient funding for these programs; and

•loss of licenses to critical intellectual properties.

We have ongoing Phase 1/2 clinical trials evaluating product candidates for the treatment of hemophilia A (SB-525), hemophilia B (SB-FIX), MPS I (SB-318), MPS II (SB-913), and beta-thalassemia (ST-400). We also plan to initiate a Phase 1/2 clinical trial of TxCell's first CAR-Treg (which is a regulatory T cell, or Treg, genetically modified with a chimeric antigen receptor, or CAR) investigational product candidate for solid organ transplant, or TX200, in 2019.

Even if we are able to complete our Phase 1/2 clinical trials for these programs successfully, we will be required to conduct additional clinical trials with larger patient populations, before obtaining the necessary regulatory approval to commercialize any products, which involves significantly greater resources, commitments and expertise. We also have limited experience in conducting later stage clinical trials and may not possess the necessary resources and expertise to complete such trials. Therefore, we may be required to scale up our operations and enter into collaborative relationships with pharmaceutical companies that could assume responsibility for late-stage development and commercialization. In this regard, while we have entered into collaborative agreements to provide funding and assistance in the development of certain product candidates through the clinical trial process, there is no guarantee that we will be able to enter into future collaborative relationships with third parties that can provide us with the funding and expertise for later stage trials. In addition, there is no guarantee that any positive results achieved in our Phase 1/2 clinical trials will be indicative of long-term efficacy and safety in later stage clinical trials. If a larger patient population does not demonstrate an acceptable safety and efficacy profile, or if any positive results in our Phase 1/2 clinical trials are not reproducible, our products may not receive approval from the FDA or foreign regulatory authorities, which could have a material adverse effect on our business that would cause our stock price to decline significantly.

In addition, we have not yet reached agreement with regulatory authorities on the development pathway for our product candidates. As a result, we have not yet determined what endpoints would support approval for certain of our programs. Due to the novelty of certain programs, such as SB-318 and SB-913, the endpoints needed to support regulatory approvals may be different than originally anticipated. For example, in order to support regulatory approval for SB-318 and SB-913, we may be required to detect certain levels of enzymes in patients. In this regard, in September 2018, we announced preliminary safety and efficacy data from the Phase 1/2 clinical trial evaluating SB-913, or the CHAMPIONS study. In cohort 2 of the CHAMPIONS study, at 16 weeks post-dosing, mean reductions were observed in total urinary glycosaminoglycans, or GAGs (which is a key biomarker of MPS II disease pathophysiology), dermatan sulfate, and heparan sulfate of 51%, 32%, and 61%, respectively. Due to the sensitivity of the current assay we are utilizing to measure plasma iduronate-2-sulfatase, or IDS, enzyme levels, we were unable to detect IDS in any of the patients over the 16 weeks following treatment with SB-913. While we are currently developing a more sensitive assay to detect IDS at lower levels, there can be no guarantee that we will be able to develop such an assay nor that we may, in the future, be able to detect IDS in patients or otherwise show direct evidence of efficacy or gene editing, which may delay or preclude any regulatory approvals for SB-913.

Even if a product candidate were to successfully obtain approval from the FDA and comparable foreign regulatory authorities, any approval might contain significant limitations related to use restrictions for specified age groups, warnings, precautions or contraindications, or may be subject to burdensome post-approval study or risk management requirements. If we are unable to obtain regulatory approval for one of our product candidates in one or more jurisdictions, or any approval contains significant limitations, we may not be able to obtain sufficient funding to continue the development of that product or generate revenues attributable to that product candidate. Also, any regulatory approval of our current or future product candidates, once obtained, may be withdrawn.

Success in preclinical studies or early clinical trials may not be indicative of results obtained in later trials. Likewise, preliminary data from clinical trials should be considered carefully and with caution since the final data may be materially different from the preliminary data, particularly as more patient data become available.

Results from preclinical studies or previous clinical trials are not necessarily predictive of future clinical trial results, and interim results of a clinical trial are not necessarily indicative of final results. Our product candidates may fail to show the desired safety and efficacy in clinical development despite demonstrating positive results in preclinical studies or having successfully advanced through initial clinical trials or preliminary stages of clinical trials. In addition, from time to time, we have and may in the future publish or report interim or preliminary data from our clinical trials, such as the preliminary data we recently announced from the CHAMPIONS study and the Phase 1/2

clinical trial evaluating SB-525, or the Alta study. Interim or preliminary data from clinical trials that we may conduct may not be indicative of the final results of the trial and are subject to the risk that one or more of the clinical outcomes may materially change as patient enrollment continues and/or more patient data become available. Interim or preliminary data also remain subject to audit and verification procedures that may result in the final data being materially different from the interim or preliminary data. As a result, interim or preliminary data should be considered carefully and with caution until the final data are available.

We have ongoing Phase 1/2 clinical trials evaluating product candidates for the treatment of hemophilia A (SB-525), hemophilia B (SB-FIX), MPS I (SB-318), MPS II (SB-913), and beta-thalassemia (ST-400), and there is no guarantee that we can achieve positive final safety and efficacy results in our Phase 1/2 clinical trials for these product candidates. Furthermore, these programs (other than TX200 that we acquired through the TxCell Acquisition) are novel in-vivo gene therapy or genome editing therapies that utilize adeno-associated viral, or AAV, vector to deliver therapeutic levels of zinc finger nuclease, or ZFN, into the patient’s blood stream. The AAV delivery system has not been validated in human clinical trials previously, and if such delivery system does not meet the safety criteria or cannot produce the desirable efficacy results we expect, we may be forced to suspend or terminate the affected program.

There is a high failure rate for drugs, biologic products and cell therapies proceeding through clinical trials. Many companies in the pharmaceutical and biotechnology industries have suffered significant setbacks in late-stage clinical trials even after achieving promising results in preclinical testing and earlier-stage clinical trials. Data obtained from preclinical and clinical activities are subject to varying interpretations, which may delay, limit or prevent regulatory approval. In addition, we may experience regulatory delays or rejections as a result of many factors, including due to changes in regulatory policy during the period of our product candidate development. Any such delays could materially and adversely affect our business, financial condition, results of operations and prospects.

Our potential products are subject to a lengthy and uncertain regulatory approval process in each jurisdiction where approval is sought.

A regulatory authority such as the FDA or the European Medicines Agency, or EMA, must approve any human therapeutic product before it can be marketed in such jurisdiction. 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 Investigational New Drug application, or IND, to the FDA. Certain countries outside of the United States have a similar process that requires the submission of a clinical trial application much like the IND prior to the commencement of human clinical trials. In the European Union, for example, a clinical trial authorization application, or CTA, must be submitted for each clinical protocol to each country's national health authority and an independent ethics committee. Only after an IND becomes effective and/or the applicable CTA has been accepted may clinical trials begin. While we have stated our intention to submit additional IND and CTA applications in the future, this is only a statement of intent, and we may not be able to do so because the associated product candidates may not meet the necessary preclinical requirements. In addition, there can be no assurance that, once submitted, an IND or CTA will result in the actual initiation of clinical trials or that we will be able to meet our targeted timeline for the initiation of clinical trials. Clinical trials are subject to oversight by institutional review boards, or IRBs, and the applicable regulatory authority. In addition, our proposed clinical studies in the United States may require review from the Recombinant DNA Advisory Committee, or RAC, which is the advisory board to the NIH focusing on clinical trials involving gene transfer.

Clinical trials:

•must be conducted in conformance with the FDA's good clinical practices, within the guidelines of the International Council for Harmonisation of Technical Requirements for Registration of Pharmaceuticals for Human Use, or ICH, and other applicable regulations;

•must meet requirements for IRB oversight;

•must follow Institutional Biosafety Committee, or IBC, and NIH RAC guidelines where applicable;

•must meet requirements for informed consent;

•are subject to continuing FDA or similar foreign government oversight;

•may require oversight by a Data Monitoring Committee, or DMC;

•may require large numbers of test subjects; and

•may be suspended by a commercial partner, the FDA, applicable foreign regulatory authorities 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 or applicable foreign regulatory authorities find deficiencies in our INDs or their foreign equivalents or the conduct of these trials.

If we are not able to obtain the necessary regulatory approval to commercialize our products or if such approval is delayed or suspended, it would have a material adverse effect on our business operations and trading price of our common stock.

We may encounter difficulties that may delay, suspend or scale back our efforts to advance additional early research programs through preclinical development, IND and foreign equivalent submissions and into clinical development.

We intend to advance early research programs through preclinical development and to submit new INDs, CTAs and equivalent filings in foreign regulatory jurisdictions necessary to commence and conduct human clinical trials evaluating the preclinical candidates in our pipeline. The preparation and submission of INDs and their foreign equivalents requires us to conduct rigorous and time-consuming preclinical testing, studies, and prepare documentation relating to, among other things, the toxicity, safety, manufacturing, chemistry and clinical protocol of our product candidates. 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 products and fail to demonstrate consistency in the formulation of the drug. Our preclinical tests may produce negative or

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

We may not successfully identify, acquire, develop or commercialize new potential product candidates.

Part of our business strategy is to expand our product candidate pipeline by identifying and validating new product candidates, which we may develop ourselves, in-license or otherwise acquire from others. For example, through our acquisition of TxCell, or the TxCell Acquisition, we recently acquired the rights among others to TxCell's first CAR-Treg product candidate, TX200, and its CAR-Treg technology and know-how. In this regard, we intend to use our ZFN gene editing technology to potentially develop next-generation autologous and allogeneic CAR-Treg cell therapies for use in treating autoimmune diseases, and expect that the TxCell Acquisition will accelerate our entry into the clinic with a CAR-Treg therapy. However, we are new to the field of immunology and to the use of CARs with Tregs, and we may not be successful at developing a CAR-Treg therapy that can be used in patients. Moreover, we may not achieve the expected accelerated development timeline. If we are unable to successfully develop and obtain regulatory approval for TX200 or other CAR-Treg therapies and effectively commercialize them, or if we are unable to achieve the expected accelerated development timeline, we may not realize the anticipated benefits from the TxCell Acquisition, resulting in possible impairments or other charges or losses which may materially and adversely affect our results of operations and financial condition.

In addition, in the event that our existing product candidates do not receive regulatory approval or are not successfully commercialized, then the success of our business will depend on our ability to continue to expand our product pipeline through in-licensing or other acquisitions. We may be unable to identify relevant product candidates. If we do identify such product candidates, we may be unable to reach acceptable terms with any third party from which we desire to in-license or acquire them. Even if we are able to successfully identify and acquire such product candidates, we may not be able to successfully manage the risks associated with integrating acquired or in-licensed product candidates or technologies or the risks arising from anticipated and unanticipated problems in connection with an acquisition or in-licensing transaction. Further, while we seek to mitigate risks and liabilities of potential acquisitions and in-licensing transactions through, among other things, due diligence, there may be risks and liabilities that such due diligence efforts fail to discover, that are not disclosed to us, or that we inadequately assess. Any failure in identifying and managing these risks and uncertainties effectively, including in connection with the TxCell Acquisition, would have a material adverse effect on our business. Additionally, we may not realize the anticipated benefits of such transactions for a variety of reasons, including the possibility that acquired product candidates, such as TX200, prove not to be safe or effective in clinical trials, the integration of an acquired product candidate, technology or business gives rise to unforeseen difficulties and expenditures, or that the expected benefits will not otherwise be realized or will not be realized within the expected timeframe.

We may encounter substantial delays in our clinical trials or we may fail to demonstrate safety and efficacy to the satisfaction of applicable regulatory authorities.

Before obtaining marketing approval from regulatory authorities for the sale of our product candidates, we must conduct extensive clinical trials to demonstrate the safety and efficacy of the product candidates. Clinical testing is expensive, time consuming and uncertain as to outcome. We cannot guarantee that any clinical trials will be conducted as planned or completed on schedule, if at all. A failure of one or more clinical trials can occur at any stage of testing. Events that may prevent successful or timely completion of clinical development include:

•delays in reaching a consensus with regulatory authorities on trial design;

•delays in reaching agreement on acceptable terms with prospective CROs and clinical trial sites;

•delays in opening clinical trial sites or obtaining required IRB or independent ethics committee approval at each clinical trial site;

•delays in recruiting suitable subjects to participate in our clinical trials;

•imposition of a clinical hold by regulatory authorities as a result of a serious adverse event or after an inspection of our clinical trial operations or trial sites;

•failure by us, any CROs we engage or any other third parties to adhere to clinical trial requirements;

•failure to perform in accordance with FDA good clinical practices, or GCP, or applicable regulatory guidelines in the European Union and other countries;

•delays in the testing, validation, manufacturing and delivery of our product candidates to the clinical sites, including delays by third parties with whom we have contracted to perform certain of those functions;

•delays in having subjects complete participation in a trial or return for post-treatment follow-up;

•clinical trial sites or subjects dropping out of a trial;

•selection of clinical endpoints that require prolonged periods of clinical observation or analysis of the resulting data;

•occurrence of serious adverse events associated with the product candidate that are viewed to outweigh its potential benefits;

•occurrence of serious adverse events in trials of the same class of agents conducted by other sponsors; or

•changes in regulatory requirements and guidance that require amending or submitting new clinical protocols.

Any inability to successfully complete preclinical and clinical development could result in additional costs to us or impair our ability to generate revenues from product sales, regulatory and commercialization milestones and royalties. In addition, if we make manufacturing or formulation changes to our product candidates, we may need to conduct additional studies to bridge our modified product candidates to earlier versions. Clinical trial delays also could shorten any periods during which we may have the exclusive right to commercialize our product candidates or allow our competitors to bring products to market before we do, which could impair our ability to successfully commercialize our product candidates and may harm our business, financial condition, results of operations and prospects.

Additionally, if the results of our clinical trials are inconclusive or if there are safety concerns or serious adverse events associated with our product candidates, we may:

•be delayed in obtaining marketing approval for our product candidates, if at all;

•obtain approval for indications or patient populations that are not as broad as intended or desired;

•obtain approval with labeling that includes significant use or distribution restrictions or safety warnings;

•be subject to changes in the way the product is administered;

•be required to perform additional clinical trials to support approval or be subject to additional post-marketing testing requirements;

•have regulatory authorities withdraw, or suspend, their approval of the product or impose restrictions on its distribution in the form of a modified risk evaluation and mitigation strategy;

•be subject to the addition of labeling statements, such as warnings or contraindications;

•be sued; or

•experience damage to our reputation.

We may not be able to find acceptable patients or may experience delays in enrolling patients for our clinical trials, which could delay or prevent us from proceeding with clinical trials of our product candidates.

Identifying and qualifying patients to participate in clinical trials of our product candidates is critical to our success. The timing of our clinical trials depends on our ability to recruit patients to participate as well as completion of required follow-up periods. For example, hemophilia trials often take longer to enroll due to the availability of existing treatments. We have been unable to enroll a patient in our hemophilia B clinical trial. In addition, we have only recently begun to enroll patients into the Phase 1/2 clinical trials evaluating SB-318 for the treatment of MPS I and ST-400 for the treatment of beta-thalassemia. If we are not able to enroll the necessary number of patients in a timely manner, we may not be able to complete the clinical trial. We may face similar challenges or delays in our other or potential future clinical trials. If patients are unwilling to participate in our gene therapy studies because of negative publicity from adverse events related to the biotechnology or gene therapy fields, competitive clinical trials for similar patient populations or for other reasons, the timeline for recruiting patients, conducting studies and obtaining regulatory approval of our product candidates may be delayed. These delays could result in increased costs, delays in advancing our product candidates, delays in testing the effectiveness of our product candidates or termination of the clinical trials altogether.

We may not be able to identify, recruit and enroll a sufficient number of patients, or those with required or desired characteristics, to complete our clinical trials in a timely manner. Patient enrollment and trial completion is affected by factors including:

•size of the patient population and process for identifying subjects;

•design of the trial protocol;

•eligibility and exclusion criteria;

•perceived risks and benefits of the product candidate under study;

•perceived risks and benefits of gene therapy-based approaches to treatment of diseases;

•availability of competing therapies and clinical trials;

•severity of the disease under investigation;

•availability of genetic testing for potential patients;

•proximity and availability of clinical trial sites for prospective subjects;

•ability to obtain and maintain subject consent;

•risk that enrolled subjects will drop out before completion of the trial;

•patient referral practices of physicians; and

•ability to monitor subjects adequately during and after treatment.

Our current product candidates are being developed to treat rare conditions. We may not be able to initiate or continue clinical trials if we cannot enroll a sufficient number of eligible patients to participate in the clinical trials required by regulatory authorities. We may need to expand the conduct of our clinical trials to foreign countries so that we may be better able to access and enroll subjects. Our ability to successfully initiate, enroll and complete a clinical trial in any foreign country is subject to numerous risks unique to conducting business in foreign countries, including:

•difficulty in establishing or managing relationships with contract research organizations, or CROs, and physicians;

•different standards for the conduct of clinical trials;

•absence in some countries of established groups with sufficient regulatory expertise for review of gene therapy protocols;

•our inability to locate qualified local consultants, physicians and partners; and

•the potential burden of complying with a variety of foreign laws, medical standards and regulatory requirements, including the regulation of pharmaceutical and biotechnology products and treatment.

If we have difficulty enrolling a sufficient number of patients to conduct our clinical trials as planned, we may need to delay, limit or terminate ongoing or planned clinical trials, any of which would have an adverse effect on our business, financial condition, results of operations and prospects.

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

During the conduct of clinical trials, patients report changes in their health, including illnesses, injuries and discomforts, to their study doctor. Often, it is not possible to determine whether or not the product candidate being studied caused these conditions, particularly as many of the diseases we are studying have complex comorbidities. If clinical experience indicates that our product candidates have side effects or cause serious or life-threatening side effects, the development of the product candidate may fail or be delayed, or, if the product candidate has received regulatory approval, such approval may be revoked, which would severely harm our business, prospects, operating results and financial condition.

There have been several significant adverse side effects in gene therapy treatments in the past, including reported cases of leukemia and death seen in other trials using other genomic therapies. Gene therapy is still a relatively new approach to disease treatment and additional adverse side effects could develop. There also is the potential risk of significantly delayed adverse events following exposure to gene therapy products due to persistent biologic activity of the genetic material or other components of products

used to carry the genetic material. Possible adverse side effects that could occur with treatment with gene therapy products include an immunologic reaction early after administration that, while not necessarily adverse to the patient's health, could substantially limit the effectiveness of the treatment.

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

We may be unable to obtain additional orphan drug designations or orphan drug exclusivity for any product. If our competitors are able to obtain orphan drug exclusivity for products that constitute the same drug and treat the same indications as our product candidates, we may not be able to have competing products approved by the applicable regulatory authority for a significant period of time.

Regulatory authorities in some jurisdictions, including the United States and the European Union, may designate drugs for relatively small patient populations as orphan drugs. Under the Orphan Drug Act of 1983, the FDA may designate a product candidate as an Orphan Drug if it is intended to treat a rare disease or condition, which is generally defined as having a patient population of fewer than 200,000 individuals in the United States, or a patient population greater than 200,000 in the United States where there is no reasonable expectation that the cost of developing the drug will be recovered from sales in the United States. In the European Union, the European Medicines Agency's Committee for Orphan Medicinal Products grants such designation to promote the development of products that are intended for the diagnosis, prevention or treatment of a life-threatening or chronically debilitating condition affecting not more than five in 10,000 persons in the European Union. Additionally, orphan designation is granted for products intended for the diagnosis, prevention or treatment of a life-threatening, seriously debilitating or serious and chronic condition and when, without incentives, it is unlikely that sales of the drug in the European Union would be sufficient to justify the necessary investment in developing the drug or biologic product.

Our four most advanced product candidates, SB-525, SB-FIX, SB-318 and SB-913 have all been granted Orphan Drug Designation by the FDA, and SB-525 and SB-318 and SB-913 have also been designated Orphan Medicinal Products by the European Medicines Agency, or EMA. If we request such designation for our other current or future product candidates, there can be no assurances that the FDA or the EMA will grant any of our product candidates such designation. Additionally, such designation does not guarantee that any regulatory agency will accelerate regulatory review of, or ultimately approve, that product candidate, nor does it limit the ability of any regulatory agency to grant such designation to product candidates of other companies that treat the same indications as our product candidates prior to our product candidates receiving exclusive marketing approval.

Generally, if a product candidate with an orphan drug designation receives the first marketing approval for the indication for which it has such designation, the product is entitled to a period of marketing exclusivity, which precludes the FDA or the EMA from approving another marketing application for a product that constitutes the same drug treating the same indication for that marketing exclusivity period, except in limited circumstances. If another sponsor receives such approval before we do (regardless of our orphan drug designation), we will be precluded from

receiving marketing approval for our product for the applicable exclusivity period. The applicable period is seven years in the United States and 10 years in the European Union. The exclusivity period in the United States can be extended by six months if the BLA sponsor submits pediatric data that fairly respond to a written request from the FDA for such data. The exclusivity period in the European Union can be reduced to six years if a product no longer meets the criteria for orphan drug designation or if the product is sufficiently profitable so that market exclusivity is no longer justified. Orphan drug exclusivity may be revoked if any regulatory agency determines that the request for designation was materially defective or if the manufacturer is unable to assure sufficient quantity of the product to meet the needs of patients with the rare disease or condition.

Even if we obtain orphan drug exclusivity for a product candidate, that exclusivity may not effectively protect the product candidate from competition because different drugs can be approved for the same condition. In the United States, even after an orphan drug is approved, the FDA may subsequently approve another drug for the same condition if the FDA concludes that the latter drug is not the same drug or is clinically superior in that it is shown to be safer, more effective or makes a major contribution to patient care. In the European Union, marketing authorization may be granted to a similar medicinal product for the same orphan indication if:

•the second applicant can establish in its application that its medicinal product, although similar to the orphan medicinal product already authorized, is safer, more effective or otherwise clinically superior;

•the holder of the marketing authorization for the original orphan medicinal product consents to a second orphan medicinal product application; or

•the holder of the marketing authorization for the original orphan medicinal product cannot supply sufficient quantities of orphan medicinal product.

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

We expect to rely, to some extent, on our strategic partners to provide funding in support of our research and to perform independent research and preclinical and clinical testing. Our technology is broad-based, and we do not currently possess the resources necessary to fully develop and commercialize potential products that may result from our technologies or the resources or capabilities to complete the lengthy marketing approval processes that may be required for the products. Therefore, we plan to rely on strategic partnerships to help us develop and commercialize our products. We have entered into collaborative agreements to provide funding and assistance in the development of certain product candidates through the clinical trial process. For example, we have an agreement with Kite for potential engineered cell therapies for cancer, two separate agreements with Pfizer, one for SB-525 for hemophilia A, and another for amyotrophic lateral sclerosis and frontotemporal lobar degeneration linked to mutations of the C9ORF72 gene, and an agreement with Bioverativ for our beta-thalassemia and sickle cell disease product candidates.

If we are unable to find additional partners or if the partners we are unable or unwilling to advance our programs, or if they do not diligently pursue product approval, this may slow our progress and adversely affect our ability to generate revenues. In addition, our partners may sublicense or abandon development programs or we may have disagreements or disputes with our partners, which would cause associated product development to slow or cease. In addition, the business or operations of our partners may change significantly through restructuring, acquisition or other strategic transactions or decisions that may negatively impact their ability to advance our programs.

There can be no assurance that we will be able to establish further strategic collaborations for our products. We may require significant time to secure collaborations or partners because we need to effectively market the benefits of our technology to these future collaborators and partners, which may direct the attention and resources of our research and development personnel and management away from our primary business operations. Further, each collaboration or partnering arrangement will involve the negotiation of terms that may be unique to each collaborator or partner. These business development efforts may not result in a collaboration or partnership.

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

Under typical partnering agreements, we would expect to receive revenue for the research and development of our product candidates 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 Relationships with Collaborators and Strategic Partners.”

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

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

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

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

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

Effective delivery of ZFNs and ZFP TFs into the appropriate target cells and tissues is critical to the success of the therapeutic applications of our ZFP technology. In order to have a meaningful therapeutic effect, product candidates based 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 these product candidates as a nucleic acid that encodes the ZFN or ZFP TF. We use different formulations to deliver the ZFN or ZFP TF depending on the required duration of expression, the targeted tissue and the indication that we intend to treat, including our proprietary AAV delivery system. However, there can be no assurances that we will be able to effectively deliver our ZFNs and ZFP TFs to produce a beneficial therapeutic effect.

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

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

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

Even if we, our collaborators or strategic partners are successful in using our ZFP technology in drug discovery, protein production, therapeutic development or other areas in which we have licensed our technology, such as plant agriculture, they may not be able to commercialize the resulting products or may decide to use other methods

competitive with this technology. To date, no company has received marketing approval or has developed or commercialized any therapeutic or agricultural products based on our ZFP technology. Should our technology fail to provide safe, effective, useful or commercially viable approaches to the discovery and development of these product candidates, 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 products may not gain market acceptance among physicians, patients, healthcare payers and the medical community.

Even if we obtain regulatory approval for any of our product candidates that we may develop or acquire in the future, the applicable product may not gain market acceptance among physicians, healthcare payors, patients or the medical community. Market acceptance of any of our product candidates for which we receive approval depends on a number of factors, including:

•the efficacy and safety of such product candidates as demonstrated in clinical trials;

•the clinical indications and patient populations for which the product candidate is approved;

•acceptance by physicians, major cancer treatment centers and patients of the drug as a safe and effective treatment;

•the adoption of novel gene therapies by physicians, hospitals and third-party payors;

•the potential and perceived advantages of product candidates over alternative treatments;

•the safety of product candidates seen in a broader patient group, including its use outside the approved indications;

•any restrictions on use together with other medications;

•the prevalence and severity of any side effects;

•product labeling or product insert requirements of the FDA or other regulatory authorities;

•the timing of market introduction of our products as well as competitive products;

•the development of manufacturing and distribution processes for our product candidates;

•the cost of treatment in relation to alternative treatments;

•the availability of coverage and adequate reimbursement and the willingness of patients to pay out-of-pocket in the absence of coverage or inadequacy of reimbursement by third-party payors and government authorities;

•relative convenience and ease of administration; and

•the effectiveness of our sales and marketing efforts and those of our collaborators.

If any of our product candidates are approved but fail to achieve market acceptance among physicians, patients, healthcare payors or treatment centers, we will not be able to generate significant revenues, which would compromise our ability to become profitable.

Even if we are able to commercialize our product candidates, the products may not receive coverage and adequate reimbursement from third-party payors in the United States and in other countries in which we seek to commercialize our products, which could harm our business.

Our ability to commercialize any product successfully will depend, in part, on the extent to which coverage and adequate reimbursement for these products and related treatments will be available from government health administration authorities, private health insurers and other organizations.

Government authorities and third-party payors, such as private health insurers and health maintenance organizations, determine which medications they will cover and establish reimbursement levels. A primary trend in the healthcare industry is cost containment. Government authorities and third-party payors have attempted to control costs by limiting coverage and the amount of reimbursement for particular medications. Increasingly, third-party payors are requiring that drug companies provide them with predetermined discounts from list prices and are challenging the prices charged for medical products. Third-party payors may also seek additional clinical evidence, beyond the data required to obtain regulatory approval, demonstrating clinical benefits and value in specific patient populations before covering our products for those patients. We cannot be sure that coverage and adequate reimbursement will be available for any product that we commercialize and, if reimbursement is available, what the level of reimbursement will be. Coverage and reimbursement may impact the demand for, or the price of, any product candidate for which we obtain regulatory approval. If reimbursement is not available or is available only at limited levels, we may not be able to successfully commercialize any product candidate for which we obtain regulatory approval.

There may be significant delays in obtaining coverage and reimbursement for newly approved drugs, and coverage may be more limited than the purposes for which the drug is approved by the FDA or comparable foreign regulatory authorities. Moreover, eligibility for coverage and reimbursement does not imply that any drug will be paid for in all cases or at a rate that covers our costs, including research, development, manufacture, sale and distribution. Interim reimbursement levels for new drugs, if applicable, may also not be sufficient to cover our costs and may only be temporary. Reimbursement rates may vary according to the use of the drug and the clinical setting in which it is used, may be based on reimbursement levels already set for lower cost drugs and may be incorporated into existing payments for other services. Net prices for drugs may be reduced by mandatory discounts or rebates required by government healthcare programs or private payors and by any future relaxation of laws that presently restrict imports of drugs from countries where they may be sold at lower prices than in the United States. Third-party payors in the United States often rely upon Medicare coverage policy and payment limitations in setting their own reimbursement policies, but also have their own methods and approval process apart from Medicare determinations. Our inability to promptly obtain coverage and profitable reimbursement rates from both government-funded and private payors for any approved products that we develop could have a material adverse effect on our operating results, our ability to raise capital needed to commercialize products and our overall financial condition.

Recently enacted and future legislation, including potentially unfavorable pricing regulations or other healthcare reform initiatives, may increase the difficulty and cost for us to obtain regulatory approval of and commercialize our product candidates and affect the prices we may obtain.

The regulations that govern, among other things, regulatory approvals, coverage, pricing and reimbursement for new drug products vary widely from country to country. In the United States and some foreign jurisdictions, there have been a number of legislative and regulatory changes and proposed changes regarding the healthcare system that could prevent or delay regulatory approval of our product candidates, restrict or regulate post-approval activities and affect our ability to successfully sell any product candidates for which we obtain regulatory approval. In particular, in March 2010, the Patient Protection and Affordable Care Act, as amended by the Health Care and Education Reconciliation Act, collectively, the Affordable Care Act, was enacted, which substantially changes the way healthcare is financed by both governmental and private insurers, and significantly impacts the U.S. pharmaceutical industry. The Affordable Care Act and its implementing regulations, among other things, addressed a new methodology by which rebates owed by manufacturers under the Medicaid Drug Rebate Program are calculated for certain drugs and biologics, including products similar to our product candidates, that are inhaled, infused, instilled, implanted or injected, increased the minimum Medicaid rebates owed by manufacturers under the Medicaid Drug Rebate Program, extended the Medicaid Drug Rebate Program to utilization of prescriptions of individuals enrolled in Medicaid managed care organizations, subjected manufacturers to new annual fees and taxes for certain branded prescription drugs, created a new Patient Centered Outcomes Research Institute, which provides incentives to programs that increase the federal government's comparative effectiveness research, established a new Medicare Part D coverage gap discount program, in which manufacturers must agree to offer 50% (and 70% commencing January 1, 2019) point-of-sale discounts off negotiated prices of applicable brand drugs to eligible beneficiaries during their coverage gap period, as a condition for the manufacturer's outpatient drugs to be covered under Medicare Part D, and created a licensure framework for follow-on biologic products.

Some of the provisions of the Affordable Care Act have yet to be fully implemented, and there have been legal and political challenges to certain aspects of the Affordable Care Act. Since January 2017, President Trump has signed two executive orders and other directives designed to delay, circumvent, or loosen certain requirements mandated by the Affordable Care Act. Concurrently, Congress has considered legislation that would repeal or repeal and replace all or part of the Affordable Care Act. While Congress has not passed repeal legislation, two bills affecting the implementation of certain taxes under the Affordable Care Act have been signed into law. The Tax Cuts and Jobs Act of 2017 includes a provision repealing, effective January 1, 2019, the tax-based shared responsibility payment imposed by the Affordable Care Act on certain individuals who fail to maintain qualifying health coverage for all or part of a year that is commonly referred to as the "individual mandate". Additionally, on January 22, 2018, President Trump signed a continuing resolution on appropriations for fiscal year 2018 that delayed the implementation of certain Affordable Care Act-mandated fees. Further, the Bipartisan Budget Act of 2018, among other things, amends the Affordable Care Act, effective January 1, 2019, to close the coverage gap in most Medicare drug plans, commonly referred to as the "donut hole". More recently, in July 2018, the Centers for Medicare & Medicaid Services, or CMS, published a final rule permitting further collections and payments to and from certain Affordable Care Act qualified health plans and health insurance issuers under the Affordable Care Act risk adjustment program in response to the outcome of federal district court litigation regarding the method CMS uses to determine this risk adjustment. Congress may consider other legislation to repeal, or repeal and replace, other elements of the Affordable Care Act.

Other legislative changes have been proposed and adopted in the United States since the Affordable Care Act was enacted. In August 2011, the Budget Control Act of 2011, among other things, created measures for spending reductions by Congress. A Joint Select Committee on Deficit Reduction, tasked with recommending a targeted deficit reduction of at least $1.2 trillion for the years 2013 through 2021, was unable to reach required goals, thereby triggering the legislation's automatic reduction to several government programs. This includes aggregate reductions of Medicare payments to providers of 2% per fiscal year, which went into effect in April 2013, and, due to subsequent

legislative amendments to the statute, including the Bipartisan Budget Act of 2018, will remain in effect through 2027 unless additional Congressional action is taken. In January 2013, President Obama signed into law the American Taxpayer Relief Act of 2012, or the ATRA, which, among other things, further reduced Medicare payments to several providers, including hospitals and cancer treatment centers, and increased the statute of limitations period for the government to recover overpayments to providers from three to five years.

Also, there has been heightened governmental scrutiny recently over pharmaceutical pricing practices in light of the rising cost of prescription drugs and biologics. Such scrutiny has resulted in several recent Congressional inquiries and proposed and enacted federal and state legislation designed to, among other things, bring more transparency to product pricing, review the relationship between pricing and manufacturer patient programs, and reform government program reimbursement methodologies for pharmaceutical products, some of which are included in the Trump administration’s budget proposal for fiscal year 2019. Additionally, the Trump administration released a “Blueprint” to lower drug prices and reduce out of pocket costs of drugs that contains additional proposals to increase manufacturer competition, increase the negotiating power of certain federal healthcare programs, incentivize manufacturers to lower the list price of their products and reduce the out of pocket costs of drug products paid by consumers. The U.S. Department of Health and Human Services has begun the process of soliciting feedback on some of these measures and, at the same, is immediately implementing others under its existing authority. Although a number of these, and other potential, proposed measures

will require authorization through additional legislation to become effective. Congress and the Trump administration have each indicated that it will continue to seek new legislative and/or administrative measures to control drug costs. At the state level, legislatures have increasingly passed legislation and implemented regulations designed to control pharmaceutical and biological product pricing, including price or patient reimbursement constraints, discounts, restrictions on certain product access and marketing cost disclosure and transparency measures, and, in some cases, have been designed to encourage importation from other countries and bulk purchasing.

There have been, and likely will continue to be, legislative and regulatory proposals at the foreign, federal and state levels directed at broadening the availability of healthcare and containing or lowering the cost of healthcare. We cannot predict the initiatives that may be adopted in the future. The continuing efforts of the government, insurance companies, managed care organizations and other payors of healthcare services to contain or reduce costs of healthcare and/or impose price controls may adversely affect:

•the demand for our product candidates, if we obtain regulatory approval;

•our ability to set a price that we believe is fair for our products;

•our ability to generate revenue and achieve or maintain profitability;

•the level of taxes that we are required to pay; and

•the availability of capital.

Any reduction in reimbursement from Medicare or other government programs may result in a similar reduction in payments from private payors, which may adversely affect our future profitability.

Legislative and regulatory proposals have been made to expand post-approval requirements and restrict sales and promotional activities for pharmaceutical products. We cannot be sure whether additional legislative changes will be enacted, or whether the FDA regulations, guidance or interpretations will be changed, or what the impact of such changes on the regulatory approvals of our product candidates, if any, may be.

In the United States, the European Union and other potentially significant markets for our product candidates, government authorities and third-party payors are increasingly attempting to limit or regulate the price of medical products and services, particularly for new and innovative products and therapies, which has resulted in lower average selling prices. Furthermore, the increased emphasis on managed healthcare in the United States and on country and regional pricing and reimbursement controls in the European Union will put additional pressure on product pricing, reimbursement and usage, which may adversely affect our future product sales and results of operations. These pressures can arise from rules and practices of managed care groups, judicial decisions and governmental laws and regulations related to Medicare, Medicaid and healthcare reform, pharmaceutical reimbursement policies and pricing in general.

Price controls may be imposed in foreign markets, which may adversely affect our future profitability.

In some countries, particularly member states of the European Union, the pricing of prescription drugs is subject to governmental control. In these countries, pricing negotiations with governmental authorities can take considerable time after receipt of regulatory approval for a product. In addition, there can be considerable pressure by governments and other stakeholders on prices and reimbursement levels, including as part of cost containment measures. Political, economic and regulatory developments may further complicate pricing negotiations, and pricing negotiations may continue after reimbursement has been obtained. Reference pricing used by various European Union member states

and parallel distribution, or arbitrage between low-priced and high-priced member states, can further reduce prices. In some countries, we, or our collaborators, may be required to conduct a clinical trial or other studies that compare the cost-effectiveness of our product candidates to other available therapies in order to obtain or maintain reimbursement or pricing approval. Publication of discounts by third-party payors or authorities may lead to further pressure on the prices or reimbursement levels within the country of publication and other countries. If reimbursement of our products is unavailable or limited in scope or amount, or if pricing is set at unsatisfactory levels, our business could be adversely affected.

Even if we complete the necessary preclinical and clinical studies, we cannot predict when or if we will obtain regulatory approval to commercialize a product candidate or the approval may be for a more narrow indication than we expect.

We cannot commercialize a product until the appropriate regulatory authorities have reviewed and approved the product candidate. Even if our product candidates demonstrate safety and efficacy in clinical studies, the regulatory agencies may not complete their review processes in a timely manner, or we may not be able to obtain regulatory approval. Additional delays may result if an FDA Advisory Committee or other regulatory advisory group or authority recommends non-approval or restrictions on approval. In addition, we may experience delays or rejections based upon additional government regulation from future legislation or administrative action, or changes in regulatory agency policy during the period of product development, clinical studies and the review process. Regulatory agencies also may approve a treatment candidate for fewer or more limited indications than requested or may

grant approval subject to the performance of post-marketing studies. In addition, regulatory agencies may not approve the labeling claims that are necessary or desirable for the successful commercialization of our treatment candidates. For example, the development of certain product candidates for pediatric use is an important part of our current business strategy, and if we are unable to obtain regulatory approval for the desired age ranges, our business may suffer.

Even if we obtain regulatory approval for a product candidate, our products will remain subject to regulatory scrutiny.

Even if we obtain regulatory approval in a jurisdiction, the regulatory authority may still impose significant restrictions on the indicated uses or marketing of our product candidates, or impose ongoing requirements for potentially costly post-approval studies, post-market surveillance or patient or drug restrictions. For example, the FDA typically advises that patients treated with gene therapy undergo follow-up observations for potential adverse events for a 15-year period. Additionally, the holder of an approved BLA is obligated to monitor and report adverse events and any failure of a product to meet the specifications in the BLA. The holder of an approved BLA must also submit new or supplemental applications and obtain FDA approval for certain changes to the approved product, product labeling or manufacturing process. Advertising and promotional materials must comply with FDA rules and are subject to FDA review, in addition to other potentially applicable federal and state laws.

In addition, products are subject to payment of annual program user fees and continual review and periodic inspections by the FDA and other regulatory authorities for compliance with good manufacturing practices, or GMP, and adherence to commitments made in the BLA. If we or a regulatory agency discovers previously unknown problems with a product such as adverse events of unanticipated severity or frequency, or problems with the facility where the product is manufactured, a regulatory agency may impose restrictions relative to that product or the manufacturing facility, including requiring recall or withdrawal of the product from the market or suspension of manufacturing.

Moreover, if any of our product candidates is approved, our product labeling, advertising and promotion will be subject to regulatory requirements and continuing regulatory review. The FDA and foreign regulatory authorities strictly regulate the promotional claims that may be made about drug and biologic products. In particular, a product may not be promoted for uses that are not approved by the FDA as reflected in the product’s approved labeling.

If we fail to comply with applicable regulatory requirements following approval of any of our product candidates, a regulatory agency may:

•issue a warning letter asserting that we are in violation of the law;

•seek an injunction or impose civil or criminal penalties or monetary fines;

•suspend or withdraw regulatory approval;

•suspend any ongoing clinical studies;

•refuse to approve a pending marketing application, such as a BLA or supplements to a BLA submitted by us;

•seize product; or

•refuse to allow us to enter into supply contracts, including government contracts.

Any government investigation of alleged violations of law could require us to expend significant time and resources in response and could generate negative publicity. The occurrence of any event or penalty described above may inhibit our ability to commercialize our product candidates and generate revenues.

Failure to obtain regulatory approval in international jurisdictions would prevent our product candidates from being marketed abroad.

In addition to regulations in the United States, to market and sell our products in the European Union, many Asian countries and other jurisdictions, we must obtain separate regulatory approvals and comply with numerous and varying regulatory requirements. The approval procedure varies among countries and can involve additional testing. The time required to obtain approval may differ substantially from that required to obtain FDA approval. The regulatory approval process outside the United States generally includes all of the risks associated with obtaining FDA approval. Clinical trials accepted in one country may not be accepted by regulatory authorities in other countries. In addition, many countries outside the United States require that a product be approved for reimbursement before it can be approved for sale in that country. A product candidate that has been approved for sale in a particular country may not receive reimbursement approval in that country. We may not be able to obtain approvals from regulatory authorities outside the United States on a timely basis, if at all. Approval by one regulatory authority outside the United States does not ensure approval by regulatory authorities in other countries or jurisdictions or by the FDA. We may not be able to file for regulatory approvals and may not receive necessary approvals to commercialize our products in any market. If we are unable to obtain approval of any of our product candidates by the FDA or regulatory authorities in the European Union, Asia or elsewhere, the commercial

prospects of that product candidate may be significantly diminished, our business prospects could decline and this could materially adversely affect our business, results of operations and financial condition.

Our current and future relationships with healthcare providers, customers and third-party payors subject us to applicable anti-kickback, fraud and abuse and other healthcare laws and regulations. If we fail to comply with federal, state and foreign laws and regulations, including healthcare, privacy and data security laws and regulations, we could face substantial penalties and our business, results of operations, financial condition and prospects could be adversely affected.

Healthcare providers, physicians and third-party payors will play a primary role in the recommendation and prescription of any product candidates for which we obtain regulatory approval. Our current and future arrangements with healthcare providers, third-party payors and customers may expose us to broadly applicable fraud and abuse and other healthcare laws and regulations that may constrain the business or financial arrangements and relationships through which we would market, sell and distribute our products. As a biotechnology company, even though we will not control referrals of healthcare services or bill directly to Medicare, Medicaid or other third-party payors, federal and state healthcare laws and regulations pertaining to fraud and abuse and patients' rights are and will be applicable to our business. Restrictions under applicable federal and state healthcare laws and regulations that may affect our ability to operate include the following:

•the federal healthcare Anti-Kickback Statute prohibits, among other things, persons from knowingly and willfully soliciting, offering, receiving or providing remuneration, directly or indirectly, overtly or covertly, in cash or in kind, to induce or reward, or in return for, either the referral of an individual for, or the purchase, order or recommendation of, any good or service, for which payment may be made under a federal healthcare program such as Medicare and Medicaid;

•federal civil and criminal false claims laws and civil monetary penalty laws, including the federal False Claims Act, which can be enforced through civil whistleblower or qui tam actions, prohibits, among other things, individuals or entities from knowingly presenting, or causing to be presented, to the federal government, including the Medicare and Medicaid programs, claims for payment or approval that are false or fraudulent or making a false statement to avoid, decrease or conceal an obligation to pay money to the federal government;

•the federal Health Insurance Portability and Accountability Act of 1996, or HIPAA, imposes criminal and civil liability for executing a scheme to defraud any healthcare benefit program and also created federal criminal laws that prohibit, among other things, knowingly and willfully falsifying, concealing or covering up a material fact or making any materially false statements in connection with the delivery of or payment for healthcare benefits, items or services;

•HIPAA, as amended by the Health Information Technology for Economic and Clinical Health Act, or HITECH, and their implementing regulations, impose obligations, including mandatory contractual terms, with respect to safeguarding the privacy, security and transmission of individually identifiable health information held by certain healthcare providers, health plans and healthcare clearinghouses, known as covered entities, and individuals and entities that perform services for them that involve individually identifiable health information, known as business associates;

•the federal Physician Payments Sunshine Act created under the Affordable Care Act requires certain manufacturers of drugs, devices, biologics and medical supplies to report annually to the CMS information related to payments and other transfers of value to physicians and teaching hospitals, and ownership and investment interests held by physicians and their immediate family members;

•analogous state and foreign laws and regulations, such as state anti-kickback and false claims laws, may apply to sales or marketing arrangements and claims involving healthcare items or services reimbursed by non-governmental third-party payors, including private insurers; some state laws require pharmaceutical companies to comply with the pharmaceutical industry's voluntary compliance guidelines and the relevant compliance guidance promulgated by the federal government, require drug manufacturers to report information related to payments and other transfers of value to other healthcare providers and healthcare entities, or marketing expenditures; and/or ensure the registration and compliance of sales and medical personnel; and

•state and foreign laws govern the privacy and security of health information in specified circumstances, many of which differ from each other in significant ways and often are not preempted by HIPAA, thus complicating compliance efforts.

The scope and enforcement of each of these laws is uncertain and subject to rapid change in the current environment of healthcare reform, especially in light of the lack of applicable precedent and regulations. Federal and state enforcement bodies have recently increased their scrutiny of interactions between healthcare companies, healthcare providers and other third parties, including charitable foundations, which has led to a number of investigations, prosecutions, convictions and settlements in the healthcare industry. Responding to investigations can be time-and resource-consuming and can divert management's attention from the business. Any such investigation or settlement could increase our costs or otherwise have an adverse effect on our business.

Efforts to ensure that our business arrangements with third parties will comply with applicable healthcare laws and regulations will involve substantial costs. It is possible that governmental authorities will conclude that our business practices may not comply with current or future statutes, regulations or case law involving applicable fraud and abuse or other healthcare laws and regulations. If our operations are found to be in violation of any of these laws or any other governmental regulations that may apply to us, we may be subject to significant civil, criminal and administrative penalties, damages, fines, disgorgement, personal imprisonment, exclusion from government funded healthcare programs, such as Medicare and Medicaid, additional reporting requirements and oversight if we become subject to a corporate integrity agreement or similar agreement to resolve allegations of non-compliance with these laws, and the curtailment or restructuring of our operations. If any physicians or other healthcare providers or entities with whom we expect to do business are found to not be in compliance with applicable laws, they may be subject to criminal, civil or administrative sanctions, including exclusion from government funded healthcare programs.

In addition, the provision of benefits or advantages to physicians to induce or encourage the prescription, recommendation, endorsement, purchase, supply, order or use of medicinal products is also prohibited in the European Union. The provision of benefits or advantages to physicians is governed by the national anti-bribery laws of European Union Member States, such as the U.K. Bribery Act. Infringement of these laws could result in substantial fines and imprisonment. Moreover, payments made to physicians in certain European Union Member States must be publicly disclosed. Agreements with physicians often must also be the subject of prior notification and approval by the physician's employer, his or her competent professional organization and/or the regulatory authorities of the individual European Union Member States. These requirements are provided in the national laws, industry codes or professional codes of conduct, applicable in the European Union Member States. Failure to comply with these requirements could result in reputational risk, public reprimands, administrative penalties, fines or imprisonment.

The collection, use, disclosure, transfer, or other processing of personal data regarding individuals in the European Union, including personal health data, is subject to the EU General Data Protection Regulation, or the GDPR, which became effective on May 25, 2018. The GDPR, which is wide-ranging in scope, imposes several requirements relating to the control over personal data by individuals to whom the personal data relates, the information provided to the individuals, the documentation we must maintain, the security and confidentiality of the personal data, data breach notification and the use of third party processors in connection with the processing of personal data. The GDPR also imposes strict rules on the transfer of personal data out of the European Union, provides an enforcement authority and authorizes the imposition of large penalties for noncompliance, including the potential for fines of up to €20 million or 4% of the annual global revenues of the non-compliant company, whichever is greater. The GDPR requirements apply not only to third-party transactions, but also to transfers of information between us and our subsidiaries such as TxCell, including employee information. The GDPR has increased our responsibility and potential liability in relation to personal data that we process compared to prior European Union law, particularly in light of the TxCell Acquisition, and we may be required to put in place additional mechanisms to ensure compliance with the GDPR, which could divert management's attention and increase our cost of doing business. However, despite our ongoing efforts to bring our practices into compliance with the GDPR, we may not be successful either due to various factors within our control or other factors outside our control. It is also possible that local data protection authorities may have different interpretations of the GDPR, leading to potential inconsistencies amongst various European Union Member States. Any failure or alleged failure (including as a result of deficiencies in our policies, procedures or measures relating to privacy, data security, marketing or communications) by us to comply with laws, regulations, policies, legal or contractual obligations, industry standards or regulatory guidance relating to privacy or data security, may result in governmental investigations and enforcement actions, litigation, fines and penalties or adverse publicity. In addition, new regulation, legislative actions or changes in interpretation of existing laws or regulations regarding data privacy and security (together with applicable industry standards) may increase our costs of doing business. In this regard, we expect that there will continue to be new proposed laws, regulations and industry standards relating to privacy and data protection in the United States, the European Union and other jurisdictions, such as the California Consumer Privacy Act of 2018 that will go into effect beginning January 1, 2020, and we cannot determine the impact

such future laws, regulations and standards will have on our business.

Our employees may engage in misconduct or other improper activities, including noncompliance with regulatory standards and requirements, which could cause significant liability for us and harm our reputation.

We are exposed to the risk of employee fraud or other misconduct, including intentional failures to comply with FDA regulations or similar regulations of comparable foreign regulatory authorities, provide accurate information to the FDA or comparable foreign regulatory authorities, comply with manufacturing standards we have established, comply with federal and state healthcare fraud and abuse laws and regulations and similar laws and regulations established and enforced by comparable foreign regulatory authorities, report financial information or data accurately or disclose unauthorized activities to us. Employee misconduct could also involve the improper use of information obtained in the course of clinical trials, which could result in regulatory sanctions and serious harm to our reputation. It is not always possible to identify and deter employee misconduct, and the precautions we take to detect and prevent this activity may not be effective in controlling unknown or unmanaged risks or losses or in protecting us from governmental investigations or other actions or lawsuits stemming from a failure to be in compliance with such laws or regulations. If any such actions are instituted against us, and we are not successful in defending ourselves or asserting our rights, those actions could have a significant impact on our business and results of operations, including the imposition of significant civil, criminal and

administrative penalties, damages, fines, disgorgement, personal imprisonment, exclusion from government funded healthcare programs, such as Medicare and Medicaid, additional reporting requirements and oversight if we become subject to a corporate integrity agreement or similar agreement to resolve allegations of non-compliance with these laws, and the curtailment or restructuring of our operations..

Product liability lawsuits against us could cause us to incur substantial liabilities and to limit commercialization of any products that we may develop.

We face an inherent risk of product liability exposure related to the testing of our product candidates in human clinical trials and will face an even greater risk if we commercially sell any products that we may develop. Product liability claims may be brought against us by subjects enrolled in our clinical trials, patients, healthcare providers or others using, administering or selling our products. If we cannot successfully defend ourselves against claims that our product candidates or products caused injuries, we could incur substantial liabilities. Regardless of merit or eventual outcome, liability claims may result in:

•decreased demand for any product candidates or products that we may develop;

•termination of clinical trial sites or entire trial programs;

•injury to our reputation and significant negative media attention;

•withdrawal of clinical trial participants;

•significant costs to defend the related litigation;

•substantial monetary awards to trial subjects or patients;

•loss of revenue;

•diversion of management and scientific resources from our business operations; and

•the inability to commercialize any products that we may develop.

We currently hold product liability insurance coverage at a level that we believe is customary for similarly situated companies and adequate to provide us with insurance coverage for foreseeable risks, but which may not be adequate to cover all liabilities that we may incur. Insurance coverage is increasingly expensive. We may not be able to maintain insurance coverage at a reasonable cost or in an amount adequate to satisfy any liability that may arise. We intend to expand our insurance coverage for products to include the sale of commercial products if we obtain regulatory approval for our product candidates in development, but we may be unable to obtain commercially reasonable product liability insurance for any products that receive regulatory approval. Large judgments have been awarded in class action lawsuits based on drugs that had unanticipated side effects. A successful product liability claim or series of claims brought against us, particularly if judgments exceed our insurance coverage, could decrease our cash and adversely affect our business.

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

We currently have limited experience in clinical-scale manufacturing of our product candidates and we rely upon third-party contract manufacturing organizations to manufacture and supply drug product for our preclinical and clinical studies. The manufacture of pharmaceutical products in compliance with the FDA's current good manufacturing practices, or cGMP, requires significant expertise and capital investment, including the development of advanced manufacturing techniques and process controls. Manufacturers of pharmaceutical products often encounter difficulties in production, including difficulties with production costs and yields, quality control, including stability of the product candidate and quality assurance testing, shortages of qualified personnel, as well as compliance with strictly enforced cGMP requirements, other federal and state regulatory requirements and foreign regulations. If our manufacturers were to encounter any of these difficulties or otherwise fail to comply with their obligations to us or under applicable regulations, our ability to provide study biologics in our clinical studies would be jeopardized. Any delay or interruption in the supply of clinical study materials could delay the completion of our clinical studies, increase the costs associated with maintaining our clinical study programs and, depending upon the period of delay, require us to commence new studies at significant additional expense or terminate the studies completely.

All manufacturers of our product candidates must comply with cGMP requirements enforced by the FDA through its facilities inspection program. These requirements include, among other things, quality control, quality assurance and the maintenance of records and documentation. Manufacturers of our product candidates may be unable to comply with these cGMP requirements and with other FDA, state and foreign regulatory requirements. The FDA or similar foreign regulatory agencies may also implement new standards at any time, or change their interpretation and enforcement of existing standards for manufacture, packaging or testing of

products. We have limited control over our manufacturers' compliance with these regulations and standards. Failure to comply with these requirements may result in fines and civil penalties, suspension of production, suspension or delay in product approval, product seizure or recall or withdrawal of product approval. If the safety of any product supplied is compromised due to our manufacturers' failure to adhere to applicable laws or for other reasons, we may not be able to obtain regulatory approval for or successfully commercialize our products and we may be held liable for any injuries sustained as a result. Any of these factors could cause a delay of clinical studies, regulatory submissions, approvals or commercialization of our product candidates, entail higher costs or impair our reputation.

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

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

We are building a manufacturing facility that could support future clinical production of our product candidates. We have no experience as a company manufacturing pharmaceutical products, and there can be no assurance that we will be able to build a compliant manufacturing facility or, if built, we will be able to successfully manufacture any of our product candidates.

We expect to utilize both contract manufacturing organizations, or CMOs, and our own facility to meet our projected needs for clinical supply. We intend to expand our manufacturing capacity by designing and building a manufacturing facility that we plan to initially use to support our clinical supply needs. To meet these objectives we will need to transition manufacturing processes and know-how of our product candidates to our own facility. Transferring manufacturing processes and know-how is complex and involves review and incorporation of both documented and undocumented processes that may have evolved over time. In addition, transferring production to different facilities may require utilization of new or different processes to meet the specific requirements of a given facility. Additional studies may also need to be conducted to support the transfer of certain manufacturing processes and process improvements. We cannot be certain that all relevant know-how and data has been adequately incorporated into the manufacturing process until the completion of studies (and the related evaluations) intended to demonstrate the comparability of material previously produced with that generated by our CMOs. Although some of our employees have experience in the manufacturing of pharmaceutical products from prior employment at other companies, we, as a company, have no prior experience in pharmaceutical product manufacturing, and operating this facility would require us to comply with complex regulations and to continue to hire and retain experienced scientific, quality control, quality assurance and manufacturing personnel. Designing and building a manufacturing facility has been and will continue to be time-consuming and expensive, and we may experience delays or cost overruns. In addition, government approvals would be required for us to operate a manufacturing facility and can be time-consuming to obtain. As a manufacturer of pharmaceutical products, we also would be required to demonstrate and maintain cGMP compliance. These requirements include, among other things, quality control, quality assurance and the maintenance of records and documentation. Furthermore, establishing manufacturing operations may require a reallocation of other resources, particularly the time and attention of our senior management. Even if we are able to

establish our own manufacturing capabilities, we could encounter challenges in operating the manufacturing facility in compliance with cGMP, regulatory or other applicable requirements, resulting in potential negative consequences, including regulatory actions, which could undermine our ability to utilize this facility for our own manufacturing needs. Any failure or delay in the development of our manufacturing capabilities could adversely impact the development of our product candidates.

There are risks associated with manufacturing for clinical and commercial use. Manufacturing biological components at the appropriate scale and quality is complex and difficult.

There are risks associated with manufacturing our product candidates including, among others, cGMP compliance, cost overruns, technical problems with process scale-up, process reproducibility, stability issues, lot consistency, yields and timely availability of raw materials. Even if efficacy and safety data from our clinical trials would otherwise support regulatory approval for a product candidate, there is no assurance that we or any third-party manufacturer will be able to manufacture our product candidates to specifications at levels necessary to support or maintain regulatory approval by the FDA or other regulatory authorities. In addition, we may not be able to manufacture our product candidates in sufficient quantities to meet the requirements for a potential launch or to meet potential future demand. If we or our third-party manufacturers are unable to produce sufficient quantities of the approved

product for commercialization, either on a timely basis or at all, our commercialization efforts would be impaired, which would have a material adverse effect on our business, financial condition, results of operations and growth prospects.

We face uncertainties and risks associated with the manufacture of our product candidates. Our product candidates are biologics and their manufacture involves complex processes, including the development of cell lines or cell systems to produce the biologic, with the challenge of significant variability. Further, there are difficulties in growing large quantities of such cells, consistently and sufficiently isolating certain types of cells and harvesting and purifying the biologic produced by them. The cost to manufacture biologics is generally far higher than traditional small molecule chemical compounds, and the manufacturing process can be difficult to reproduce. There is no guarantee we will be successful in establishing a larger-scale commercial manufacturing process for our pipeline product candidates or obtaining the needed manufacturing capacity. Due to the high cost to manufacture, inherent uncertainty related to manufacturing costs, and uncertainty in our patient population, there is risk that some of our product candidates may not be commercially viable.

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 based on our ZFP technology, 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.

If we are unable to establish sales and marketing capabilities or enter into agreements with third parties to market and sell our product candidates, we may be unable to generate any revenue.

We do not currently have an organization for the sale, marketing and distribution of pharmaceutical products and the cost of establishing and maintaining such an organization may exceed the cost-effectiveness of doing so. In order to market any products that may be approved by the FDA and comparable foreign regulatory authorities, we must build our sales, marketing, managerial and other non-technical capabilities or make arrangements with third parties to perform these services. There are significant risks involved in building and managing a sales organization, including our ability to hire, retain and incentivize qualified individuals, generate sufficient sales leads, provide adequate training to sales and marketing personnel and effectively manage a geographically dispersed sales and marketing team. Any failure or delay in the development of our internal sales, marketing and distribution capabilities would adversely impact the commercialization of any approved products. If we are unable to establish adequate sales, marketing and distribution capabilities, whether independently or with third parties, we may not be able to generate product revenues and may not become profitable. We will be competing with many companies that currently have extensive and well-funded sales and marketing operations. Without an internal commercial organization or the support of a third party to perform sales and marketing functions, we may be unable to compete successfully against these more established companies. If we are not successful in commercializing our current or future product candidates either on our own or through collaborations with one or more third parties, our future product revenue will suffer and we would incur significant additional losses.

We will need to grow the size of our organization, and we may experience difficulties in managing this growth.

As of September 30, 2018, we had 234 full-time employees. We need to grow the size of our organization in order to support our continued development and potential commercialization of our product candidates. In particular, we will need to add substantial numbers of additional personnel and other resources to support our development and potential commercialization of our product candidates. In addition, we may not be able to attract or retain employees with the appropriate levels of experience and to skills to accomplish our objectives. As our development and commercialization plans and strategies continue to develop, or as a result of any future acquisitions, our need for additional managerial, operational, manufacturing, sales, marketing, financial and other resources will increase. Our management, personnel and systems currently in place may not be adequate to support this future growth. Future growth would impose significant added responsibilities on members of management, including:

•managing our preclinical studies and clinical trials effectively;

•identifying, recruiting, maintaining, motivating and integrating additional employees;

•