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# 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 June 30, 2019**
**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)** | **(I.R.S. Employer Identification No.)** |

| **501 Canal Boulevard** | **Richmond** | **California** | **94804** |
|---|---|---|---|
| **(Address of principal executive offices)** | | | **(Zip Code)** |

**(510) 970-6000**
**(Registrant's telephone number, including area code)**

Securities registered pursuant to Section 12(b) of the Act:

| Title of each class | Trading Symbol(s) | Name of each exchange on which registered |
|---|---|---|
| Common Stock, par value $0.01 per share | SGMO | NASDAQ Global Select Market |

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 August 2, 2019, 115,675,077 shares of the issuer's common stock, par value $0.01 per share, were outstanding.

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**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 subsidiaries, including Sangamo Therapeutics France S.A.S (formerly TxCell S.A.).

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.

## 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 that 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 may include, but are not limited to, statements about:

- our strategy;
- anticipated product candidate development and potential commercialization of any resulting products;
- the initiation, scope, rate of progress, enrollment, anticipated results and timing of our preclinical studies and clinical trials and those of our collaborators or strategic partners;
- the therapeutic and commercial potential of, and the ability of Sangamo and our collaborators or strategic partners to advance the development of, product candidates using our zinc finger protein, or ZFP, technology platform, including our ability to effectively deliver our zinc finger nucleases, or ZFNs, and ZFP transcription factors, or ZFP TFs, to produce a clinical benefit;
- the benefits of the acquisition of TxCell S.A., now known as Sangamo Therapeutics France S.A.S.;
- our ability to establish and maintain collaborative, licensing and other similar arrangements;
- anticipated revenues from existing and new collaborations and the timing thereof;
- our research and development and other expenses;
- our ability to obtain adequate preclinical and clinical supplies of our product candidates from current and potential new suppliers and manufacturers;
- the ability of Sangamo and our collaborators or strategic partners to obtain and maintain regulatory approvals for product candidates using our ZFP technology platform;
- our ability to comply with, and the impact of, regulatory requirements, obligations and restrictions on our business;
- our ability to protect our intellectual property and operate our business without infringing upon the intellectual property rights of others, including our ability to obtain rights to the gene transfer technologies required to develop and commercialize our product candidates;
- our estimates regarding the sufficiency of our cash resources and our expenses, capital requirements and need for additional financing, and our ability to obtain additional financing;
- our ability to manage the growth of our business;
- our projected operating and financial performance;
- 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” and similar expressions intended to identify forward-looking statements. These statements reflect our current views with respect to future events, are based on assumptions and are 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 or revise any forward-looking statements to reflect new information or future events or developments. Readers are cautioned not to place undue reliance on the forward-looking statements, which speak only as of the date of this Quarterly Report on Form 10-Q.

**PART I. FINANCIAL INFORMATION**

**ITEM 1. FINANCIAL STATEMENTS**

**SANGAMO THERAPEUTICS, INC.**

**CONDENSED CONSOLIDATED BALANCE SHEETS**
**(Unaudited; in thousands)**

| | June 30, 2019 | December 31, 2018 |
|---|---|---|
| **ASSETS** | | |
| Current assets: | | |
| Cash and cash equivalents | $ 169,222 | $ 140,418 |
| Restricted cash, current portion | 2,000 | — |
| Marketable securities | 271,877 | 259,715 |
| Interest receivable | 766 | 375 |
| Accounts receivable | 12,095 | 4,673 |
| Prepaid expenses and other current assets | 5,848 | 5,340 |
| Total current assets | 461,808 | 410,521 |
| Marketable securities, non-current | 8,450 | — |
| Property and equipment, net | 21,019 | 78,723 |
| Intangible assets | 53,892 | 54,243 |
| Goodwill | 39,795 | 40,044 |
| Operating lease right-of-use assets | 79,435 | — |
| Other non-current assets | 7,582 | 3,364 |
| Non-current restricted cash | 1,500 | 3,500 |
| Total assets | $ 673,481 | $ 590,395 |
| **LIABILITIES AND STOCKHOLDERS' EQUITY** | | |
| Current liabilities: | | |
| Accounts payable and accrued liabilities | $ 22,505 | $ 21,457 |
| Accrued compensation and employee benefits | 8,714 | 9,490 |
| Deferred revenues | 50,826 | 47,564 |
| Total current liabilities | 82,045 | 78,511 |
| Deferred revenues, non-current | 94,189 | 108,273 |
| Long-term portion of lease liabilities | 41,550 | 27,689 |
| Deferred income tax | 6,661 | 6,705 |
| Other non-current liabilities | 3,288 | 1,960 |
| Total liabilities | 227,733 | 223,138 |
| Commitments and contingencies | | |
| Stockholders' equity: | | |
| Preferred stock | — | — |
| Common stock | 1,156 | 1,022 |
| Additional paid-in capital | 1,078,976 | 929,632 |
| Accumulated deficit | (634,233) | (562,696) |
| Accumulated other comprehensive loss | (765) | (1,440) |
| Total Sangamo Therapeutics, Inc. stockholders' equity | 445,134 | 366,518 |
| Non-controlling interest | 614 | 739 |
| Total stockholders' equity | 445,748 | 367,257 |
| Total liabilities and stockholders' equity | $ 673,481 | $ 590,395 |

*See accompanying Notes to Condensed Consolidated Financial Statements.*

**SANGAMO THERAPEUTICS, INC.**

**CONDENSED CONSOLIDATED STATEMENTS OF OPERATIONS**
**(Unaudited; in thousands, except per share amounts)**

| | Three Months Ended June 30, | | Six Months Ended June 30, | |
|---|---|---|---|---|
| | 2019 | 2018 | 2019 | 2018 |
| Revenues | $ 17,548 | $ 21,416 | $ 25,619 | $ 34,053 |
| Operating expenses: | | | | |
| Research and development | 36,455 | 29,255 | 71,305 | 52,802 |
| General and administrative | 14,597 | 11,301 | 31,715 | 21,388 |
| Total operating expenses | 51,052 | 40,556 | 103,020 | 74,190 |
| Loss from operations | (33,504) | (19,140) | (77,401) | (40,137) |
| Interest and other income, net | 3,148 | 2,500 | 4,842 | 3,310 |
| Net loss | (30,356) | (16,640) | (72,559) | (36,827) |
| Net loss attributable to non-controlling interest | (72) | — | (125) | — |
| Net loss to Sangamo Therapeutics, Inc. stockholders | $ (30,284) | $ (16,640) | $ (72,434) | $ (36,827) |
| Basic and diluted net loss per share attributable to Sangamo Therapeutics, Inc. stockholders | $ (0.26) | $ (0.17) | $ (0.67) | $ (0.40) |
| Shares used in computing basic and diluted net loss per share attributable to Sangamo Therapeutics, Inc. stockholders | 114,382 | 97,267 | 108,360 | 91,831 |

*See accompanying Notes to Condensed Consolidated Financial Statements.*

**SANGAMO THERAPEUTICS, INC.**

**CONDENSED CONSOLIDATED STATEMENTS OF COMPREHENSIVE LOSS**
**(Unaudited; in thousands)**

| | Three Months Ended June 30, | | Six Months Ended June 30, | |
|---|---|---|---|---|
| | 2019 | 2018 | 2019 | 2018 |
| Net loss | $ (30,356) | $ (16,640) | $ (72,559) | $ (36,827) |
| Foreign currency translation adjustment | 1,442 | — | (62) | — |
| Change in unrealized gain on available-for-sale securities | 484 | 230 | 737 | 131 |
| Comprehensive loss | (28,430) | (16,410) | (71,884) | (36,696) |
| Comprehensive loss attributable to non-controlling interest | (72) | — | (125) | — |
| Comprehensive loss attributable to Sangamo Therapeutics, Inc. | $ (28,358) | $ (16,410) | $ (71,759) | $ (36,696) |

*See accompanying Notes to Condensed Consolidated Financial Statements.*

**SANGAMO THERAPEUTICS, INC.**

**CONDENSED CONSOLIDATED STATEMENTS OF STOCKHOLDERS' EQUITY**
**(Unaudited; in thousands)**

| | Common Stock | | Additional Paid-in Capital | Accumulated Deficit | Accumulated Other Comprehensive Income (Loss) | Non-Controlling Interest | Total Stockholders' Equity |
|---|---|---|---|---|---|---|---|
| | Shares | Amount | | | | | |
| Balances at December 31, 2018 | 102,188 | $ 1,022 | $ 929,632 | $ (562,696) | $ (1,440) | $ 739 | $ 367,257 |
| Cumulative-effect adjustment of ASC Topic 842 on January 1, 2019 | — | — | — | 897 | — | — | 897 |
| Issuance of common stock upon exercise of stock options and in connection with restricted stock units, net of tax | 141 | 1 | 215 | — | — | — | 216 |
| Issuance costs related to public offering | — | — | (258) | — | — | — | (258) |
| Stock-based compensation | — | — | 4,523 | — | — | — | 4,523 |
| Foreign currency translation adjustment | — | — | — | — | (1,504) | — | (1,504) |
| Net unrealized gain on marketable securities | — | — | — | — | 253 | — | 253 |
| Net loss | — | — | — | (42,150) | — | (53) | (42,203) |
| Balances at March 31, 2019 | 102,329 | 1,023 | 934,112 | (603,949) | (2,691) | 686 | 329,181 |
| Issuance of common stock upon exercise of stock options and in connection with restricted stock units, net of tax | 492 | 5 | 2,439 | — | — | — | 2,444 |
| Issuance of common stock under employee stock purchase plan | 132 | 1 | 1,137 | — | — | — | 1,138 |
| Issuance of common stock under public offering, net of issuance costs | 12,650 | 127 | 136,439 | — | — | — | 136,566 |
| Issuance costs related to TxCell Acquisition | — | — | (18) | — | — | | (18) |
| Stock-based compensation | — | — | 4,867 | — | — | — | 4,867 |
| Foreign currency translation adjustment | — | — | — | — | 1,442 | — | 1,442 |
| Net unrealized gain on marketable securities | — | — | — | — | 484 | — | 484 |
| Net loss | — | — | — | (30,284) | — | (72) | (30,356) |
| Balances at June 30, 2019 | 115,603 | $ 1,156 | $ 1,078,976 | $ (634,233) | $ (765) | $ 614 | $ 445,748 |

*See accompanying Notes to Condensed Consolidated Financial Statements.*

**SANGAMO THERAPEUTICS, INC.**

**CONDENSED CONSOLIDATED STATEMENTS OF STOCKHOLDERS' EQUITY (CONTINUED)**
**(Unaudited; in thousands)**

| | Common Stock | | Additional Paid-in Capital | Accumulated Deficit | Accumulated Other Comprehensive Income (Loss) | Non-Controlling Interest | Total Stockholders' Equity |
|---|---|---|---|---|---|---|---|
| | Shares | Amount | | | | | |
| Balances at December 31, 2017 | 85,598 | $ 856 | $ 682,809 | $ (495,479) | $ (286) | $ — | $ 187,900 |
| Cumulative-effect adjustment of ASC Topic 606 on January 1, 2018 | — | — | — | 1,117 | — | — | 1,117 |
| Issuance of common stock upon exercise of stock options and in connection with restricted stock units, net of tax | 1,443 | 14 | 10,570 | — | — | — | 10,584 |
| Stock-based compensation | — | — | 3,050 | — | — | — | 3,050 |
| Net unrealized loss on marketable securities | — | — | — | — | (99) | — | (99) |
| Net loss | — | — | — | (20,187) | — | — | (20,187) |
| Balances at March 31, 2018 | 87,041 | 870 | 696,429 | (514,549) | (385) | — | 182,365 |
| Issuance of common stock upon exercise of stock options and in connection with restricted stock units, net of tax | 263 | 2 | 1,952 | — | — | — | 1,954 |
| Issuance of common stock under employee stock purchase plan | 163 | 2 | 688 | — | — | — | 690 |
| Issuance of common stock under public offering, net of issuance costs | 14,157 | 142 | 215,614 | — | — | | 215,756 |
| Stock-based compensation | — | — | 3,514 | — | — | — | 3,514 |
| Foreign currency translation adjustment | — | — | — | — | (2) | — | (2) |
| Net unrealized gain on marketable securities | — | — | — | — | 230 | — | 230 |
| Net loss | — | — | — | (16,640) | — | — | (16,640) |
| Balances at June 30, 2018 | 101,624 | $ 1,016 | $ 918,197 | $ (531,189) | $ (157) | $ — | $ 387,867 |

*See accompanying Notes to Condensed Consolidated Financial Statements.*

**SANGAMO THERAPEUTICS, INC.**

**CONDENSED CONSOLIDATED STATEMENTS OF CASH FLOWS**
**(Unaudited; in thousands)**

| | Six Months Ended June 30, | |
|---|---|---|
| | **2019** | **2018** |
| **Operating Activities:** | | |
| Net loss | $ (72,559) | $ (36,827) |
| Adjustments to reconcile net loss to net cash (used in) provided by operating activities: | | |
| Depreciation and amortization | 1,583 | 1,207 |
| Amortization of discount on marketable securities | (2,455) | (1,656) |
| Gain on free shares | (551) | — |
| Stock-based compensation | 9,390 | 6,564 |
| Net loss on lease termination | 218 | — |
| Other | 11 | 465 |
| Net changes in operating assets and liabilities: | | |
| Interest receivable | (391) | (322) |
| Accounts receivable | (7,422) | (1,634) |
| Prepaid expenses and other assets | (5,449) | (3,564) |
| Operating lease right-of-use assets | 1,923 | — |
| Accounts payable and accrued liabilities | 1,811 | 2,733 |
| Accrued compensation and employee benefits | (764) | (1,133) |
| Deferred revenues | (10,821) | 138,474 |
| Long-term portion of lease liabilities | (563) | — |
| Other non-current liabilities | 1,327 | — |
| Net cash (used in) provided by operating activities | (84,712) | 104,307 |
| **Investing Activities:** | | |
| Purchases of marketable securities | (244,306) | (451,240) |
| Maturities of marketable securities | 226,884 | 133,297 |
| Purchases of property and equipment | (9,760) | (5,768) |
| Net cash used in investing activities | (27,182) | (323,711) |
| **Financing Activities:** | | |
| Proceeds from public offering of common stock, net of issuance costs | 136,308 | 215,756 |
| Taxes paid related to net share settlement of equity awards | (296) | (57) |
| Proceeds from issuance of common stock | 4,094 | 13,285 |
| Net cash provided by financing activities | 140,106 | 228,984 |
| Effects of changes in foreign exchange rates | 592 | — |
| Net increase in cash, cash equivalents, and restricted cash | 28,804 | 9,580 |
| Cash, cash equivalents, and restricted cash, beginning of period | 143,918 | 53,326 |
| **Cash, cash equivalents, and restricted cash, end of period** | $ 172,722 | $ 62,906 |
| **Supplemental disclosure of non-cash activities:** | | |
| Property and equipment included in accrued liabilities | $ 1,679 | $ 1,836 |
| Right-of-use assets obtained in exchange for lease obligations | $ 29,671 | $ — |

*See accompanying Notes to Condensed Consolidated Financial Statements.*

**SANGAMO THERAPEUTICS, INC.**

**NOTES TO CONDENSED CONSOLIDATED FINANCIAL STATEMENTS**
**June 30, 2019**
**(Unaudited)**

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

### *Overview*

Sangamo Therapeutics, Inc. ("Sangamo" or the "Company") was incorporated in the State of Delaware in June 1995 and changed its name from Sangamo Biosciences, Inc. in January 2017. Sangamo is focused on the research, development and commercialization of novel therapeutic strategies 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"). Potential applications of Sangamo's technology include development of human therapeutics, plant agriculture and enhancement of pharmaceutical protein production.

Sangamo is currently working on a number of long-term development projects that will 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 funds, research grants and from the issuance of equity or debt securities. Sangamo believes that its available cash, cash equivalents and investments as of June 30, 2019, and expected revenues from collaborations, strategic partnerships and research grants, will be adequate to fund its operations at least through the next twelve months from the date the financial statements are issued. Sangamo will require additional financial resources to complete the development and commercialization of its products including ZFP Therapeutic products. Additional capital may not be available on terms acceptable to the Company, or 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 ZFP Therapeutic products would 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 generally accepted accounting principles in the United States of America ("U.S. GAAP") 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 U.S. GAAP 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 six months ended June 30, 2019 are not necessarily indicative of the results that may be expected for the year ending December 31, 2019. The condensed consolidated balance sheet data at December 31, 2018 was derived from the audited consolidated financial statements included in Sangamo's Annual Report on Form 10-K for the year ended December 31, 2018 (the "2018 Annual Report") as filed with the SEC on March 1, 2019. 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, 2018, included in the 2018 Annual Report.

### *Use of Estimates*

The preparation of financial statements in conformity with U.S. GAAP requires management to make estimates and assumptions that affect the amounts reported in the financial statements and the accompanying notes. On an ongoing basis, management evaluates its estimates including critical accounting policies or estimates related to revenue recognition, clinical trial accruals, fair value of assets and liabilities, including from acquisitions, 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. In March 2019, the Company recorded an adjustment to revenue related to a change in estimate in connection with the hemophilia A collaboration agreement with Pfizer Inc. ("Pfizer") as result of a decision made in a joint steering committee of Pfizer and Sangamo in March 2019 to increase the project scope and related project cost, which resulted in a decrease in the measure of the proportional performance. This adjustment decreased revenue by $3.0 million, increased net loss by $3.0 million and increased the Company's basic net loss per share by $0.03 for the six months ended June 30, 2019.

### *Foreign Currency Translation*

The functional currency of the Company's foreign subsidiaries is primarily the Euro. Assets and liabilities denominated in foreign currencies are translated to U.S. dollars using the exchange rates at the balance sheet date. Foreign currency translation

adjustments are recorded as a component of Accumulated Other Comprehensive Income (Loss) ("AOCI") within stockholders' equity. Revenues and expenses from the Company's foreign subsidiaries are translated using the monthly average exchange rates in effect during the period in which the transactions occur. Foreign currency transaction gains and losses are recorded in interest and other income, net, on the Company's Condensed Consolidated Statements of Operations.

***Reclassifications***

Certain prior period amounts in the accompanying condensed consolidated financial statements have been reclassified to conform to the current period presentation. These reclassifications had no effect on the reported results of operations. The Company reclassified $0.6 million from Intangible assets to Other non-current assets on the Condensed Consolidated Balance Sheet as of December 31, 2018.

***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, deposits in demand money market accounts and commercial paper.

***Marketable Securities***

Sangamo 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 almost identical assets, with the unrealized holding gains and losses included in AOCI.

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, net, which are determined using the specific identification method.

***Concentrations of Risk***

Cash, cash equivalents, and marketable securities consist of financial instruments that potentially subject the Company to a concentration of credit risk to the extent of the fair value recorded in the condensed consolidated balance sheets. The Company invests cash that is not required for immediate operating needs primarily in highly liquid instruments that bear minimal risk. The Company has established guidelines relating to the quality, diversification, and maturities of securities to enable the Company to manage its credit risk. The Company is exposed to credit risk in the event of a default by the financial institutions holding its cash, cash equivalents and investments and issuers of investments to the extent recorded on the condensed consolidated balance sheets.

In April 2019, the Company entered into an Option Agreement (the "Option") with Brammer Bio MA ("Brammer") whereby Brammer granted an option to secure dedicated capacity for Sangamo for manufacturing in Brammer's facilities. The Company paid $3.0 million for the Option, which expires on July 31, 2020, and which is included in other non-current assets on the Condensed Consolidated Balance Sheets. If the Company exercises the option, the $3.0 million will be applied towards future manufacturing services. If the Company does not exercise the option, $1.5 million of the deposit is non-refundable. The remainder will be applied towards future services initiated within five years. In addition, the Company will pay Brammer $2.0 million to assist it in establishing its manufacturing capabilities in Brisbane, CA.

Certain materials and key components that the Company utilizes in its operations are obtained through single suppliers. Since the suppliers of key components and materials must be named in an investigational new drug application filed with the U.S. Food and Drug Administration for a product, significant delays can occur if the qualification of a new supplier is required. If delivery of material from the Company's suppliers were interrupted for any reason, the Company may be unable to supply any of its product candidates for clinical trials.

***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 are stated at their estimated fair values. The counterparties to the agreements relating to the Company's investment securities consist of the U.S. government-sponsored entities and various major corporations and financial institutions with high credit ratings. The free share asset/liability is measured using a binomial-lattice pricing model and is reviewed each reporting period and adjusted, as needed.

***Leases***

The Company determines if an arrangement is or contains a lease at inception by assessing whether the arrangement contains an identified asset and whether it has the right to control the identified asset. Right-of-use ("ROU") assets represent the Company's right to use an underlying asset for the lease term and lease liabilities represent the Company's obligation to make lease payments arising from the lease. Lease liabilities are recognized at the lease commencement date based on the present value of future lease payments over the lease term. ROU assets are based on the measurement of the lease liability and also include any lease payments made prior to or on lease commencement and exclude lease incentives and initial direct costs incurred, as applicable.

As the implicit rate in the Company's leases is generally unknown, the Company uses its incremental borrowing rate based on the information available at the lease commencement date in determining the present value of remaining lease payments. The incremental borrowing rate represents an estimate of the interest rate the Company would incur at lease commencement to borrow an amount equal to the lease payments on a collateralized basis over the term of a lease in a similar economic environment. The Company gives consideration to its credit risk, term of the lease, total lease payments and adjusts for the impacts of collateral, as necessary, when calculating its incremental borrowing rates. The lease terms may include options to extend or terminate the lease when it is reasonably certain the Company will exercise any such options. Rent expense for the Company's operating leases is recognized on a straight-line basis over the lease term.

The Company has elected to not separate lease and non-lease components for its real estate and copier leases and, as a result, accounts for any lease and non-lease components as a single lease component. The Company has also elected to not apply the recognition requirement to any leases with a term of 12 months or less and does not include an option to purchase the underlying asset that the Company is reasonably certain to exercise.

***Revenue Recognition***

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

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

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 research 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 ASC 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. Related costs and expenses under these arrangements have historically approximated the revenues recognized.

For the six months ended June 30, 2019, revenues related to Kite Pharma, Inc. ("Kite"), a wholly-owned subsidiary of Gilead Sciences, Inc., the hemoglobinopathies agreement with Bioverativ Inc., (now Sanofi Genzyme, a global business unit of Sanofi S.A. ("Sanofi")), and the Company's hemophilia A collaboration agreement with Pfizer represented 61%, 18% and 16%, respectively, of the Company's total revenue, excluding the above change in estimate. For the three months ended June 30, 2019, revenues related to Kite, the Company's hemophilia A collaboration agreement with Pfizer and the hemoglobinopathies agreement with Sanofi represented 52%, 26% and 17%, respectively, of the Company's total revenue. During the six months ended June 30, 2018, revenues related to the Company's hemophilia A collaboration agreement with Pfizer, the hemoglobinopathies agreement with Sanofi and the agreement with Kite represented 47%, 26% and 22%, respectively, of the Company's total revenue. For the three months ended June 30, 2018, revenues related to the Company's hemophilia A collaboration agreement with Pfizer, Kite and the hemoglobinopathies agreement with Sanofi represented 38%, 35% and 21%, respectively, of the Company's 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*

*Simplified Disclosure*

In August 2018, the SEC adopted amendments to certain disclosure requirements in Securities Act Release No. 33-10532, *Disclosure Update and Simplification*, as updated. 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. As such, the Company adopted these SEC amendments on November 5, 2018 and has presented the analysis of changes in stockholders' equity in these interim financial statements for June 30, 2019 and 2018 presented in this Quarterly Report on Form 10-Q. The Company's adoption of these SEC amendments had no material effect on the Company's reporting of financial position, results of operations, cash flows or stockholders' equity.

*Accounting for Leases*

In February 2016, the Financial Accounting Standards Board ("FASB") issued ASU 2016-2, *Leases* ("ASC Topic 842"). ASC Topic 842 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 ROU asset and corresponding liability, measured at the present value of the lease payments. On January 1, 2019, the Company adopted ASC Topic 842 using the modified retrospective approach with a cumulative-effect adjustment of $0.9 million reflected as a decrease to the opening balance of accumulated deficit as of the adoption date. Results for the three and six months ended June 30, 2019 are presented under ASC Topic 842. No prior period amounts were adjusted and continue to be reported in accordance with previous lease guidance, ASC Topic 840 — *Leases* ("ASC Topic 840").

ASC Topic 842 provides a number of optional practical expedients in transition. The Company elected the practical expedients to not reassess its prior conclusions about lease identification under the new standard, to not reassess lease classification, and to not reassess initial direct costs. The Company did not elect the practical expedient allowing the use-of-hindsight which would require the Company to reassess the lease term of its leases based on all facts and circumstances through

the effective date and did not elect the practical expedient pertaining to land easements as this is not applicable to the current contract portfolio.

The impact of the adoption of ASC Topic 842 on the accompanying Condensed Consolidated Balance Sheet as of January 1, 2019 was as follows (in thousands):

| | December 31, 2018 | Adjustments Due to the Adoption of ASC Topic 842 | January 1, 2019 |
|---|---|---|---|
| **Assets:** | | | |
| Property and equipment, net | $ 78,723 | $ (62,500) | $ 16,223 |
| Operating lease right-of-use assets | — | 8,753 | 8,753 |
| Prepaid rent | — | 36,025 | 36,025 |
| | | | |
| **Liabilities:** | | | |
| Operating lease liabilities - current[1] | — | 1,408 | 1,408 |
| Deferred rent[1] | 271 | (271) | — |
| Build-to-suit lease obligation[2] | 27,689 | (27,689) | — |
| Operating lease liabilities - long-term[2] | — | 7,933 | 7,933 |
| | | | |
| **Accumulated deficit** | (562,696) | 897 | (561,799) |

(1) Operating lease liabilities – current and deferred rent are included in accounts payable and accrued liabilities on the Condensed Consolidated Balance Sheets.
(2) Build-to-suit lease obligation and operating lease liabilities – long-term are included in long-term portion of lease liabilities on the Condensed Consolidated Balance Sheets.

The adjustments due to the adoption of ASC Topic 842 primarily related to the recognition of operating lease ROU assets and operating lease liabilities for the Company's leases. In addition, the adoption of ASC Topic 842 resulted in a change in accounting of the build-to-suit component of two leases under ASC Topic 840 to operating leases under ASC Topic 842 and as a result the Company derecognized the estimated fair value of the building shells that were included in Property and equipment, net as of December 31, 2018, as the Company had been deemed to own these buildings under ASC Topic 840. For additional discussion of the build-to-suit properties, see "Note 7 – *Property and equipment, net*" to the Company's consolidated financial statements included in its Annual Report on Form 10-K for the year ended December 31, 2018 filed with the SEC on March 1, 2019. For a description of the leases, see "Note 8 – *Commitments and Contingencies – Leases*" in these condensed consolidated financial statements.

*Not yet adopted*

*Collaborative Arrangements*

In November 2018, the FASB issued ASU 2018-18, *Collaborative Arrangements (ASC Topic 808): Clarifying the Interaction between Topic 808 and Topic 606* ("ASC Topic 808"), which clarifies that certain transactions between participants in a collaborative arrangement should be accounted for under ASC Topic 606 when the counterparty is a customer. In addition, ASC Topic 808 precludes an entity from presenting consideration from a transaction in a collaborative arrangement as revenue from contracts with customers if the counterparty is not a customer for that transaction. This guidance will be effective for the Company beginning January 1, 2020. The Company is currently evaluating the impact of the adoption of this standard on its consolidated financial statements.

*Goodwill Impairment Testing*

In January 2017, the FASB issued ASU No. 2017-4, *Intangibles – Goodwill and Other (Topic 350): Simplifying the Test of Goodwill Impairment* ("ASU 2017-4"). The new guidance simplifies the subsequent measurement of goodwill by eliminating Step 2 from the goodwill impairment test. ASU 2017-4 requires goodwill impairment to be measured as the amount by which a reporting unit's carrying amount exceeds its fair value, not to exceed the carrying amount of its goodwill. ASU 2017-4 requires prospective application and is effective for annual periods beginning after December 15, 2019. ASU 2017-4 will require the Company to amend its methodology for determining any goodwill impairment beginning in 2020.

**NOTE 2—FAIR VALUE MEASUREMENTS**

The Company measures certain financial assets and liabilities at fair value on a recurring basis, including cash equivalents, available-for-sale marketable securities and the free share asset/liability. Fair value is determined based on a three-tier hierarchy under the authoritative guidance for fair value measurements and disclosures that prioritizes the inputs used in measuring fair value as follows:

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

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

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

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

| | June 30, 2019 Fair Value Measurements | | | |
|---|---|---|---|---|
| | Total | Level 1 | Level 2 | Level 3 |
| Assets: | | | | |
| Cash equivalents: | | | | |
| Money market funds | $ 71,490 | $ 71,490 | $ — | $ — |
| Commercial paper securities | 30,441 | — | 30,441 | — |
| Total | 101,931 | 71,490 | 30,441 | — |
| Marketable securities: | | | | |
| Commercial paper securities | 170,953 | — | 170,953 | — |
| Corporate debt securities | 71,383 | — | 71,383 | — |
| U.S. government-sponsored entity debt securities | 37,991 | — | 37,991 | — |
| Total | 280,327 | — | 280,327 | — |
| Total cash equivalents and marketable securities | $ 382,258 | $ 71,490 | $ 310,768 | $ — |
| | | | | |
| Free shares asset | $ 361 | $ — | $ — | $ 361 |

| | December 31, 2018 Fair Value Measurements | | | |
|---|---|---|---|---|
| | Total | Level 1 | Level 2 | Level 3 |
| Assets: | | | | |
| Cash equivalents: | | | | |
| Money market funds | $ 103,291 | $ 103,291 | $ — | $ — |
| Total | 103,291 | 103,291 | — | — |
| Marketable securities: | | | | |
| Commercial paper securities | 177,224 | — | 177,224 | — |
| Corporate debt securities | 63,870 | — | 63,870 | — |
| U.S. government-sponsored entity debt securities | 18,621 | — | 18,621 | — |
| Total | 259,715 | — | 259,715 | — |
| Total cash equivalents and marketable securities | $ 363,006 | $ 103,291 | $ 259,715 | $ — |
| Liabilities: | | | | |
| Free shares liability | $ 154 | $ — | $ — | $ 154 |

***Cash Equivalents and Marketable Securities***

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.

***Free Share Asset/Liability***

As a result of the July 20, 2018 Share Purchase Agreement ("SPA") with TxCell S.A., a French *société anonyme* ("TxCell") (see Note 10 — *Acquisition of TxCell S.A.*), the Company entered into arrangements with the holders of approximately 477,000 "free shares" of TxCell pursuant to which the Company has the right to purchase such shares from the holders thereof (a call option) and such holders have the right to sell to the Company such shares from time to time through mid-2021 (a put option). The Company initially recorded a liability of $0.2 million on the acquisition date. The put options were classified within Level 3 of the fair value hierarchy as the Company utilized a binomial-lattice pricing model (the "Monte Carlo simulation model") that involved certain market conditions to estimate the fair value of the options. The assumptions used in this simulation model are reviewed on a quarterly basis and adjusted, as needed. Subsequent changes in the fair value of the free shares are recorded in general and administrative expenses in the Condensed Consolidated Statements of Operations. The number of free shares has not changed since the Acquisition Date. The free shares liability was approximately $0.2 million at December 31, 2018 and the Company recognized a gain due to an increase in the fair value of the free shares of approximately $0.5 million for the six months ended June 30, 2019 bringing the balance to an asset of approximately $0.4 million at June 30, 2019.

| Free Shares valuation assumptions: | June 30, 2019 | December 31, 2018 |
|---|---|---|
| Sangamo Stock Price (USD) | $ 9.65 | $ 11.48 |
| TxCell Stock Price (EUR) | € 2.24 | € 2.58 |
| EUR / USD Exchange Rate | 0.89 | 0.87 |
| Estimated Correlation Sangamo and TxCell Stock Prices | 72.2% | — |
| Sangamo Stock Price (USD) Volatility Estimate | 77.6% | 79.9% |
| TxCell Stock Price (EUR) Volatility Estimate | 76.2% | 8.6% |
| EUR / USD Exchange Rate Volatility Estimate | 7.0% | 7.7% |
| Risk Free Rate and Cost of Debt by Expected Exercise Date | Varies | Varies |

**NOTE 3—CASH AND MARKETABLE SECURITIES**

***Cash, Cash Equivalents and Restricted Cash***

A reconciliation of cash, cash equivalents and restricted cash reported within the Condensed Consolidated Balance Sheets to the amounts reported within the accompanying Condensed Consolidated Statements of Cash Flows was as follows (in thousands):

| | June 30, 2019 | December 31, 2018 | June 30, 2018 | December 31, 2017 |
|---|---|---|---|---|
| Cash and cash equivalents | $ 169,222 | $ 140,418 | $ 59,406 | $ 49,826 |
| Restricted cash included in Restricted cash, current portion | 2,000 | — | — | — |
| Restricted cash included in Non-current restricted cash | 1,500 | 3,500 | 3,500 | 3,500 |
| Cash, cash equivalents and restricted cash as reported within the accompanying Condensed Consolidated Statements of Cash Flows | $ 172,722 | $ 143,918 | $ 62,906 | $ 53,326 |

Restricted cash consists of a letter of credit for $3.5 million established as a deposit for the Brisbane lease.

***Cash Equivalents and Available-for-sale Securities***

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

| | Amortized Cost | Gross Unrealized Gains | Gross Unrealized (Losses) | Estimated Fair Value |
|---|---|---|---|---|
| **June 30, 2019** | | | | |
| Assets | | | | |
| Cash equivalents: | | | | |
| Money market funds | $ 71,490 | $ — | $ — | $ 71,490 |
| Commercial paper securities | 30,439 | 2 | — | 30,441 |
| Total | 101,929 | 2 | — | 101,931 |
| Available-for-sale securities: | | | | |
| Commercial paper securities | 170,598 | 355 | — | 170,953 |
| Corporate debt securities | 71,280 | 108 | (5) | 71,383 |
| U.S. government-sponsored entity debt securities | 37,973 | 18 | — | 37,991 |
| Total | 279,851 | 481 | (5) | 280,327 |
| Total cash equivalents and available-for-sale securities | $ 381,780 | $ 483 | $ (5) | $ 382,258 |
| **December 31, 2018** | | | | |
| Cash equivalents: | | | | |
| Money market funds | $ 103,291 | $ — | $ — | $ 103,291 |
| Total | 103,291 | — | — | 103,291 |
| Available-for-sale securities: | | | | |
| Commercial paper securities | 177,353 | — | (129) | 177,224 |
| Corporate debt securities | 63,981 | — | (111) | 63,870 |
| U.S. government-sponsored entity debt securities | 18,640 | — | (19) | 18,621 |
| Total | 259,974 | — | (259) | 259,715 |
| Total cash equivalents and available-for-sale securities | $ 363,265 | $ — | $ (259) | $ 363,006 |

The fair value of investments available-for-sale by contractual maturity were as follows (in thousands):

| | June 30, 2019 | December 31, 2018 |
|---|---|---|
| Maturing in one year or less | $ 271,877 | $ 259,715 |
| Maturing after one year through five years | 8,450 | — |
| Total | $ 280,327 | $ 259,715 |

The Company had no material realized losses of its available-for-sale securities for the three and six months ended June 30, 2019 or 2018. Sangamo has the intent and ability to hold its investments for a period of time sufficient to allow for any anticipated recovery in market value. No investments were other-than-temporarily impaired at either June 30, 2019 or December 31, 2018.

**NOTE 4—BASIC AND DILUTED NET LOSS PER SHARE**

Basic net loss per share attributable to Sangamo Therapeutics, Inc. stockholders has been computed by dividing net loss attributable to Sangamo Therapeutics, Inc. stockholders by the weighted-average number of shares of common stock outstanding during the period. Diluted net loss per share attributable to Sangamo Therapeutics, Inc. stockholders is calculated by dividing net loss attributable to Sangamo Therapeutics, Inc. stockholders 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 ("RSUs") outstanding and the employee stock purchase plan ("ESPP") shares reserved for issuance, which are all anti-dilutive, were excluded from consideration in the calculation of diluted net loss per share attributable to Sangamo Therapeutics, Inc. stockholders. Stock options and RSUs outstanding and ESPP shares reserved for issuance as of June 30, 2019 and 2018 totaled 10,155,033 and 8,757,528, respectively.

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

***Collaboration Agreements***

***Kite Pharma, Inc.***

In February 2018, the Company entered into a global collaboration and license agreement with Kite for the research, development and commercialization of potential engineered cell therapies for cancer. In this collaboration, Sangamo is working together with Kite on a research program under which the companies are designing ZFNs and adeno-associated viral vectors ("AAVs") to disrupt and insert certain genes in T-cells and natural killer cells ("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 and has announced that they expect to initiate a clinical trial evaluating KITE-037, an allogeneic anti-CD19 CAR-T cell therapy, in 2020. 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 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 Company concluded the transaction price under this agreement is $185.9 million and 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. Further, the Company concluded the 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 the 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 June 30, 2019 and December 31, 2018, the Company had deferred revenue of $119.1 million and $131.5 million, respectively, related to this agreement.

Revenues recognized under the agreement for the three and six months ended June 30, 2019 and 2018 were as follows (in thousands):

| | Three Months Ended June 30, | | Six Months Ended June 30, | |
|---|---|---|---|---|
| | 2019 | 2018 | 2019 | 2018 |
| Revenue related to Kite agreement: | | | | |
| Recognition of upfront fee | $ 6,227 | $ 5,953 | $ 12,386 | $ 5,953 |
| Research services | 2,833 | 1,562 | 4,986 | 1,562 |
| Total | $ 9,060 | $ 7,515 | $ 17,372 | $ 7,515 |

***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 for 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 originally 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 of the on-going services 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 June 30, 2019 and December 31, 2018, the Company had deferred revenue of $14.3 million and $10.0 million, respectively, related to this agreement.

Revenues recognized under the agreement for the three and six months ended June 30, 2019 and 2018 were as follows (in thousands):

| | Three Months Ended June 30, | | Six Months Ended June 30, | |
|---|---|---|---|---|
| | 2019 | 2018 | 2019 | 2018 |
| Recognition of upfront fee related to Pfizer SB-525 agreement | $ 4,635 | $ 8,183 | $ 1,595 | $ 15,841 |

In March 2019, the Company received new data results in the hemophilia A collaboration agreement with Pfizer, and expansion of patients for the ongoing trial. As a result, the estimated project cost increased and the proportional performance was updated based on the actual services delivered to Pfizer as a percentage of the updated project cost as of March 31, 2019. The increase in project cost resulted in a decrease in the measure of the proportional cumulative performance. During the six months ended June 30, 2019, the Company recognized $1.6 million in revenues related to the Pfizer SB-525 agreement which were net of the approximately $3.0 million reduction in revenues recorded in the three months ended March 31, 2019 related to the updated estimated project cost.

*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 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 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 of the on-going services, over 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 June 30, 2019 and December 31, 2018, the Company had deferred revenue of $8.7 million and $9.8 million, respectively, related to this agreement.

Revenues recognized under the agreement for the three and six months ended June 30, 2019 and 2018 were as follows (in thousands):

| | Three Months Ended June 30, | | Six Months Ended June 30, | |
|---|---|---|---|---|
| | 2019 | 2018 | 2019 | 2018 |
| Recognition of upfront fee related to Pfizer C9ORF72 agreement | $ 455 | $ 617 | $ 1,070 | $ 1,076 |

***Sanofi Genzyme***

In January 2014, the Company entered into an exclusive worldwide collaboration and license agreement to develop therapeutics for hemoglobinopathies, focused on beta thalassemia and sickle cell disease ("SCD"). The agreement was originally signed with Biogen MA Inc., who subsequently assigned it to Bioverativ Inc., which was later acquired by Sanofi. 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, Sanofi 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, Sanofi has the right to step in and take over any of the Company's remaining activities. Furthermore, the Company has an option to co-promote in the U.S. any licensed products to treat beta thalassemia and SCD developed under the agreement, and Sanofi will compensate the Company for such co-promotion activities. Subject to the terms of the agreement, the Company has granted Sanofi 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 Sanofi 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. Sanofi 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 Sanofi agreement.

The agreement may be terminated by (i) the Company or Sanofi for the uncured material breach of the other party, (ii) the Company or Sanofi for the bankruptcy or other insolvency proceeding of the other party; (iii) Sanofi, upon 180 days' advance written notice to the Company and (iv) Sanofi, 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 the 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 Sanofi 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 of the ongoing services 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 June 30, 2019 and December 31, 2018, the Company had deferred revenue of $2.9 million and $4.6 million, respectively, related to this agreement.

Revenues recognized under the agreement for the three and six months ended June 30, 2019 and 2018 were as follows (in thousands):

| | Three Months Ended June 30, | | Six Months Ended June 30, | |
|---|---|---|---|---|
| | 2019 | 2018 | 2019 | 2018 |
| Revenue related to Sanofi agreement: | | | | |
| Recognition of upfront fee | $ 901 | $ 1,184 | $ 1,654 | $ 2,338 |
| Research services | 2,158 | 3,332 | 3,355 | 6,560 |
| Total | $ 3,059 | $ 4,516 | $ 5,009 | $ 8,898 |

***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 June 30, 2019 and December 31, 2018, the Company had received $3.0 million and $1.7 million, respectively, 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 June 30, 2019 and December 31, 2018, the $3.3 million and $1.8 million, respectively, including interest, related to

this award are recorded as a loan in other long-term liabilities on the accompanying Condensed Consolidated Balance Sheets as the Company does not expect to repay these amounts with the next 12 months.

**NOTE 6—INCOME TAXES**

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

**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 six months ended June 30, 2019 and 2018 (in thousands):

| | Three Months Ended June 30, | | Six Months Ended June 30, | |
|---|---|---|---|---|
| | 2019 | 2018 | 2019 | 2018 |
| Research and development | $ 2,763 | $ 2,120 | $ 5,061 | $ 3,879 |
| General and administrative | 2,104 | 1,394 | 4,329 | 2,685 |
| Total stock-based compensation expense | $ 4,867 | $ 3,514 | $ 9,390 | $ 6,564 |

**NOTE 8—COMMITMENTS AND CONTINGENCIES**

***Leases***

Sangamo occupies approximately 87,700 square feet of office and research and development laboratory facilities in Brisbane, CA pursuant to a lease that expires in May 2029. Sangamo also leases approximately 37,900 square feet of office and laboratory space in Richmond, CA through August 2026. The Company leases approximately 7,700 square feet of additional research and office space located in Richmond, CA pursuant to a lease that expires in December 2019. In addition, the Company leases two properties in Valbonne, France. The first lease is for approximately 14,036 square feet of research and office space that expires in June 2025. The second lease, which commenced on April 1, 2019, is for approximately 6,800 square feet of office space and expires in March 2028.

Certain of these leases also include renewal options at the election of the Company to renew or extend the lease for an additional five to ten years. These optional periods have not been considered in the determination of the ROU assets or lease liabilities associated with these leases as the Company did not consider it reasonably certain it would exercise the options.

With respect to the Brisbane lease, the commencement date for approximately 35,080 square feet of the office space occurred in January 2019 while the commencement date for the remaining approximately 52,620 square feet occurred in June 2019. The Company has the right to make tenant improvements, including the addition of laboratory space, with a lease incentive allowance of $6.8 million on the first portion of the space occupied and $10.2 million on the portion of the lease that commenced in June 2019. This lease includes two renewal options at the election of the Company to extend the lease for an additional five years each. These optional periods have not been considered in the determination of the ROU assets or lease liabilities associated with these leases as the Company did not consider it reasonably certain it would exercise the options.

The Company performed evaluations of its contracts and determined each of its identified leases are operating leases. For the three and six months ended June 30, 2019, the Company incurred $1.6 million and $2.8 million, respectively, of lease costs included in operating expenses in the Condensed Consolidated Statements of Operations in relation to these operating leases. Variable lease expense was $0.4 million and $0.6 million for the three and six months ended June 30, 2019, respectively, and was not included in the measurement of the Company's operating ROU assets and lease liabilities. The variable expense consists primarily of the Company's proportionate share of operating expenses, property taxes and insurance and is classified as lease expense due to the Company's election to not separate lease and non-lease components.

Cash paid for amounts included in the measurement of operating lease liabilities for the six months ended June 30, 2019 was $1.1 million and was included in net cash used in operating activities in the Company's Condensed Consolidated Statements of Cash Flows.

As of June 30, 2019, the maturities of the Company's operating lease liabilities were as follows (in thousands):

| | Total |
|---|---|
| Six months ending December 31, 2019 | $ 2,135 |
| 2020 | 6,065 |
| 2021 | 6,107 |
| 2022 | 6,175 |
| 2023 | 6,253 |
| Thereafter | 31,969 |
| Total lease payments | 58,704 |
| Less: | |
| Imputed interest | (14,669) |
| Total | $ 44,035 |
| | |
| Reported as of June 30, 2019: | |
| Operating lease liabilities - current (included in Accounts payable and accrued liabilities on the Condensed Consolidated Balance Sheet) | $ 2,485 |
| Operating lease liabilities - long-term | 41,550 |
| Total | $ 44,035 |

As of June 30, 2019, the weighted-average remaining lease term is 9.3 years and the weighted-average incremental borrowing rate used to determine the operating lease liability was 6.9% for the Company's operating leases.

The Company does not have any financing leases.

***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**

***Common Stock***

In April 2019, Sangamo completed an underwritten public offering of its common stock, in which the Company sold an aggregate of 12.7 million shares of its common stock at a public offering price of $11.50 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 $136.3 million.

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.

***At-the-Market Offering Agreement***

In May 2017, the Company entered into an amended and restated "at-the-market" offering program sales agreement with Cowen and Company, LLC ("Cowen"), pursuant to which the Company may issue and sell from time to time up to $75.0 million of the Company's common stock through Cowen as the sales agent (the "ATM Agreement"). 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. As of June 30, 2019, the Company has not sold any common stock under the ATM Agreement and the full $75.0 million remained available for sale, subject to certain conditions as specified in the agreement.

**NOTE 10—ACQUISITION OF TXCELL S.A.**

On July 20, 2018, Sangamo entered into several agreements with TxCell S.A. ("TxCell"), a French publicly-listed company specialized in the development of cellular immunotherapy products based on regulator T-cells ("Tregs") to treat severe autoimmune and inflammatory diseases, and certain of its shareholders, with the goal of eventually acquiring 100% of TxCell's share capital.

Under a Share Purchase Agreement ("SPA") signed with certain shareholders of TxCell, the Company acquired 13,519,036 ordinary shares of TxCell ("TxCell Ordinary Shares"), representing approximately 53% of the outstanding share capital and voting rights of TxCell, (the "Block Transaction") at a price of €2.58 per share. The Block Transaction closed on October 1, 2018 (the "Acquisition Date").

Additionally, the Company and TxCell entered into a Tender Offer Agreement ("TOA"), pursuant to which the Company agreed to acquire 11,981,867 TxCell Ordinary Shares not acquired as part of the Block Transaction following a public tender offer (the "Tender Offer") at a price of €2.58 per share. The Tender Offer closed on November 23, 2018.

Following the Block Transaction and the Tender Offer, the Company owns 98.2% of TxCell Ordinary Shares.

In addition, the Company also entered into arrangements with the holders of approximately 477,000 "free shares" of TxCell pursuant to which the Company has the right to purchase such shares from the holders thereof (a call option) and such holders have the right to sell to the Company such shares from time to time through mid-2021 (a put option) (collectively the "Free Shares Options") so as to increase the Company's ownership of TxCell to 100% should all free shares be acquired following through the exercise of either the call or the put options.

The purchase price for each such free share acquired by the Company upon exercise of a Free Shares Option will be based on the performance of the Company's stock price from the announcement of the transactions contemplated by the SPA and TOA through the Acquisition Date. At the Acquisition Date, the Free Shares Options purchase price was valued at €2.58 per share or approximately $2.99 per share using an exchange rate of $1.16. If the Company's stock price increases during that time period, the Free Shares Options purchase price per share will proportionately increase. If the Company's stock price decreases, the Free Shares Options purchase price will proportionately decrease, the minimum purchase price of €2.58 per share only applies to vested Free Shares Options that are not yet transferable shares, subject to certain exceptions.

At the Acquisition Date, the fair value of the Free Shares Options was estimated to be a liability of $0.2 million based on an option pricing method. The value was included in the purchase consideration and is recorded as a non-controlling interest on the Condensed Consolidated Balance Sheets. The fair value of the Free Shares Options will vary based on future changes in the Company's stock price during the option period. The Company assesses this fair value on a quarterly basis with changes in fair value being recognized in operations. The fair value of the Free Shares Options was estimated to be an asset of $0.4 million as of June 30, 2019.

In September 2018, the Company provided TxCell with a $5.2 million loan (the "TxCell Loan") that was deemed to be part of the purchase consideration for accounting purposes. The TxCell Loan, together with $40.5 million cash paid to acquire the TxCell Ordinary Shares and the $0.2 million estimated fair value of the Free Shares Options, comprise the aggregate purchase consideration of $45.9 million as of the Acquisition Date.

Management estimated the fair value of tangible and intangible assets and liabilities in accordance with the applicable accounting guidance for business combinations and utilized the services of third-party valuation consultants. Balances subject to adjustment primarily include the valuations of acquired assets (tangible and intangible), liabilities assumed, as well as tax-related matters. During the measurement period, the Company may record adjustments to the provisional amounts recognized. There were no adjustments to the provisional values subsequent to the Acquisition Date.

The acquisition of TxCell was accounted for as a business combination in accordance with ASC Topic 805, *Business Combinations*. The operating results of TxCell after the Acquisition Date have been included in the Company's Condensed Consolidated Statements of Operations. In June 2019, TxCell became a *société par actions simplifiée* (S.A.S.) and was renamed "Sangamo Therapeutics France."

***Fair Value Estimate of Assets Acquired and Liabilities Assumed***

Under ASC Topic 805, an acquirer recognizes and consolidates assets acquired, liabilities assumed, and any non-controlling interest at 100% of their fair values as of the acquisition date (regardless of the acquirer's percentage ownership in the acquiree). As goodwill is calculated as a residual, all goodwill of the acquired business, not just the acquirer's share, is recognized under this "full-goodwill" approach. Recognized goodwill is allocated between the controlling and non-controlling interests. Although this allocation is not presented separately on the acquirer's balance sheet, it is necessary so that a goodwill impairment charge recognized in a period following the business combination by an acquirer is appropriately allocated between controlling and non-controlling interests. There were no goodwill impairments during the six months ended June 30, 2019 or during 2018 and, as noted below, substantially all of the non-controlling interest on the Acquisition Date was subsequently acquired by the Company and, accordingly, substantially all of the goodwill is allocated to the Company as of June 30, 2019 and December 31, 2018.

The following table summarizes the estimated fair value of the net assets acquired as of the Acquisition Date (in thousands):

| | October 1, 2018 |
|---|---|
| Consideration transferred | $ 45,911 |
| Fair value of non-controlling interest | 35,829 |
| Fair value of TxCell | $ 81,740 |
| | |
| Cash | $ 4,779 |
| Current assets | 2,427 |
| Property and equipment | 1,857 |
| IPR&D | 55,019 |
| Other assets | 155 |
| Current liabilities | (9,761) |
| Assumed debt liabilities | (4,933) |
| Deferred tax liability, net | (6,798) |
| Fair value of net identifiable assets acquired | 42,745 |
| Goodwill | 38,995 |
| Total fair value of net assets acquired | $ 81,740 |

***Consideration Transferred***

Consideration transferred as of October 1, 2018 consisted of the 13,519,036 TxCell Ordinary Shares acquired by the Company on the Acquisition Date of approximately $2.99 per share, the $5.2 million TxCell Loan and approximately $0.2 million for the fair value of the Free Shares Options.

***Non-controlling Interest***

The fair value of the non-controlling interest at the Acquisition Date was based on the $2.99 acquisition price per share for the 11,981,867 Ordinary Shares that were not purchased by the Company on the Acquisition Date.

On November 1, 2018, pursuant to the TOA, the Company commenced a cash tender offer (the "Offer") to acquire all of the TxCell Ordinary Shares not held by the Company for the same per share price paid in the Block Transaction. Following the completion of the Offer on November 23, 2018, the Company initiated compulsory squeeze-out procedures applicable to French public companies to acquire the remaining TxCell Ordinary Shares, other than the free shares that were subject to the Free Shares Options. Subsequent to the Acquisition Date and through December 31, 2018, the Company acquired 11,528,635 TxCell Ordinary Shares which, when aggregated with the 13,519,036 Ordinary Shares acquired at the Acquisition Date, resulted in the Company owning 98.2% of all TxCell Ordinary Shares as of December 31, 2018. The 11,528,635 shares acquired subsequent to the Acquisition Date were acquired for total consideration of approximately $33.9 million, or $2.94 per share. As of December 31, 2018, the aggregate purchase consideration was approximately $80.4 million through the completion of this purchase, with approximately 453,000 Ordinary Shares (vested free shares), which remain outstanding and are subject to purchase by the Company as noted above, with an estimated fair value of approximately $0.6 million as of June 30, 2019.

Non-controlling interest as of June 30, 2019 was as follows (in thousands):

| | |
|---|---|
| Non-controlling interest at January 1, 2018 | $ — |
| Non-controlling interest at Acquisition Date | 35,829 |
| Shares acquired post acquisition | (34,516) |
| Non-controlling interest of acquired entity | 1,313 |
| Foreign currency effect | (19) |
| Loss attributable to non-controlling interest | (555) |
| Non-controlling interest at December 31, 2018 | 739 |
| Loss attributable to non-controlling interest | (125) |
| Non-controlling interest at June 30, 2019 | $ 614 |

## 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," "intend," "plan," "will" 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. Actual results could differ materially from those set forth in such forward-looking statements as a result of, but not limited to the "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, 2018, or the 2018 Annual Report, as filed with the Securities and Exchange Commission, or SEC, on March 1, 2019.

**Overview**

We are a clinical stage biotechnology company focused on translating ground-breaking science into genomic medicines with the potential to transform patients' lives using our platform technologies in gene therapy, *ex vivo* gene-edited cell therapy, *in vivo* genome editing and gene regulation.

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 gene therapy and genome editing products by forward integrating into manufacturing, development and commercial operations. In other therapeutic areas we intend to partner with biopharmaceutical companies to develop products as appropriate. Decisions to partner product candidates or not will be based on the best way to bring new medicines to patients and on an evaluation of our capacity to bring such products to commercial stage rapidly and efficiently on our own. For our proprietary clinical development programs, we are focused on three therapeutic areas: inherited metabolic diseases, or IMDs, central nervous system diseases and inflammatory and autoimmune diseases.

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 development 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.

We have a substantial intellectual property position including the design, selection, manufacture, composition and use of engineered ZFPs, CAR-Tregs and cell therapies to support our research and development activities. We continue to license and file new patent applications that strengthen our patent portfolio. We believe that our intellectual property position is a critical element in our ability to research, develop, manufacture and commercialize gene therapy, *ex vivo* gene-edited cell therapy, *in vivo* genome editing and gene regulation products and services.

*Gene therapy programs*

We are conducting the Phase 1/2 Alta study, an open-label, ascending-dose clinical trial to evaluate investigational SB-525 gene therapy for severe hemophilia A. SB-525 is being developed under global collaboration with Pfizer Inc., or Pfizer, for the research, development and commercialization of gene therapy product candidates for hemophilia A. 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.

In July 2019, we and Pfizer announced updated initial data from 10 patients treated in the Alta study. Across the four dosage cohorts evaluated, patients demonstrated a dose-dependent increase in Factor VIII, or FVIII, levels, and a dose-dependent reduction in the use of FVIII replacement therapy was also observed, with patients in the highest dose cohort not requiring factor replacement therapy after initial use of prophylactic factor and experiencing no bleeding events as of the data cut-off date. For the four patients in the highest dose (3e13 vg/kg) cohort, FVIII activity data were available through 24, 19, 6, and 4 weeks of follow-up, respectively. The first two patients treated in the 3e13 vg/kg cohort (Patients 7 and 8) remained in the normal range, as measured using a chromogenic assay, through 24 and 19 weeks of follow-up, respectively. The next two patients in the 3e13 vg/

kg cohort (Patients 9 and 10), with 6 and 4 weeks of follow-up, respectively, demonstrated rapid FVIII activity kinetics that appear consistent with Patients 7 and 8 at similar early time points. SB-525 was generally well-tolerated, with one patient (treated with the 3e13 vg/kg dose) reporting a treatment-related serious adverse event of hypotension and fever, which occurred following vector infusion and resolved with treatment within 24 hours of completion of vector infusion. The fifth patient in the 3e13 vg/kg cohort (Patient 11) was treated in July 2019.

Based on the accumulating results from the Alta study, the U.S. Food and Drug Administration, or FDA, has granted regenerative medicine advanced therapy, or RMAT, designation for SB-525 gene therapy to treat severe hemophilia A. RMAT designation is granted to regenerative medicine therapies intended to treat, modify, reverse, or cure a serious condition, for which preliminary clinical evidence indicates that the medicine has the potential to address an unmet medical need. The RMAT designation includes all the benefits of the fast track and breakthrough therapy designation programs, including early interactions with the FDA.

We are currently working with Pfizer on plans to advance SB-525 to a registrational study. Pfizer will assume responsibility for SB-525 late-stage development and manufacturing and we have initiated the transfer of the SB-525 manufacturing process to Pfizer.

We are also evaluating our wholly-owned investigational ST-920 gene therapy for Fabry disease, an inherited metabolic disease. An investigational new drug application, or IND, was accepted by the FDA in February 2019. We have activated the first clinical site for a Phase 1/2 clinical trial and expect to treat the first subject in the study by the end of 2019.

*Ex vivo gene edited cell therapy programs*

We are conducting the Phase 1/2 THALES study, an open-label, single arm clinical trial to evaluate the safety and efficacy of ST-400 in up to six subjects with beta thalassemia. ST-400 is an *ex vivo* gene-edited beta thalassemia cell therapy that involves gene editing of a patient's own hematopoietic stem progenitor cells using non-viral delivery of ZFN technology. ST-400 is being developed in collaboration with Sanofi Genzyme, or Sanofi, to research, develop and commercialize therapeutic gene-edited cell therapy products in hemoglobinopathies, including beta thalassemia and sickle cell disease, or SCD. Sanofi is responsible for subsequent development, manufacturing and commercialization of licensed products. The collaboration includes a related program evaluating BIVV003 for the treatment of SCD. BIVV003 uses the same technology as ST-400 and is currently being evaluated in a Phase 1/2 clinical trial conducted by Sanofi.

In April 2019, we announced early preliminary data from the first patient enrolled in the study. Recruitment of this study is ongoing, with four of six patients enrolled. We expect to present preliminary data from the first patients enrolled in the study in the fourth quarter of 2019. Until that time, we are not planning to report additional clinical data from the program. More complete results will become available and presented once enrollment is complete and patients have been followed for a longer period.

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. In this collaboration, we are working together with Kite on a research program under which we are designing ZFNs and adeno-associated viral vectors, or AAVs, to disrupt and insert certain genes in T-cells and natural killer cells, 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 and has announced that they expect to initiate a clinical trial evaluating KITE-037, an allogeneic anti-CD19 CAR-T cell therapy, in 2020.

In the fourth quarter of 2018, we completed our acquisition of 98.2% of the outstanding share capital and voting rights of TxCell S.A., or TxCell, which was renamed Sangamo Therapeutics France in June 2019. With the acquisition of TxCell, or the TxCell Acquisition, we believe we can now accelerate our research and development of innovative, personalized T-cell immunotherapies for the treatment of inflammatory and autoimmune diseases with high unmet medical need. In this regard, we expect the TxCell Acquisition will accelerate our entry into 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 evaluating the potential of CAR-Tregs in solid organ transplantation as well as a range of autoimmune diseases, such as multiple sclerosis, rheumatoid arthritis, inflammatory bowel diseases and 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. In 2019, we anticipate submitting a clinical trial application in Europe for TX-200, an autologous CAR-Treg cell therapy for the prevention of solid organ transplant rejection.

*In vivo genome editing and gene regulation programs*

We have three proprietary *in vivo* genome editing programs being evaluated in Phase 1/2 clinical trials: SB-913 (Mucopolysaccharidosis type II, or MPS II), SB-318 (MPS I), and SB-FIX (hemophilia B). In April 2019, we announced that we expect that no additional patients will be treated with first-generation ZFNs in the SB-913, SB-318 and SB-FIX clinical programs

given that clinical benefit has not been demonstrated in analyses conducted to date in the ongoing clinical trials and the expected near-term clinical development of second-generation ZFNs.

We are planning a new clinical trial for SB-913 to treat MPS III to evaluate second-generation ZFNs and other potential modifications to enhance the *in vivo* delivery of the ZFNs. *In vitro* preclinical data presented last year showed three potential advantages of second-generation ZFNs for use in the clinic: (1) improvements in efficiency and potency due to structural modifications to the ZFN architecture and expression vector; (2) the ability to function equally well in the patients with a single nucleotide polymorphism, or SNP, in the target locus in the albumin gene (~20% of the population); and (3) improvements in specificity.

We expect to initiate this next clinical trial by year end 2020. We expect to use data from the new study evaluating second generation ZFNs to make a Phase III decision for the SB-913 program and to define the next steps for the SB-318 and SB-FIX programs.

We also have several preclinical programs evaluating our zinc finger protein transcription factor, or ZFP TF, gene regulation technology. ZFP TFs act at the DNA level to selectively repress or activate the expression of specific genes to achieve a desired therapeutic effect. Gene regulation differs from other genome editing approaches as it is designed to enable precise, robust, and long-term repression of a selected gene following a single administration of AAV and does not cut or modify the target DNA.

In March and April 2019, we presented new preclinical data describing the effects of tau-targeted ZFP TFs, delivered with AAVs in the mouse and nonhuman primate, or NHP, brain. Intrahippocampal ZFP TF delivery to adult mice resulted in more than 80% tau reduction, and intravenous ZFP TF administration reduced tau levels by 50-70% across the entire mouse brain. AAV ZFP TFs targeting tau were administered to the adult NHP hippocampus using real-time MRI-guided stereotaxic infusion. The lowering of tau in the hippocampus and entorhinal cortex of NHP was correlated with the transgene expression levels. The treatment was well tolerated for the duration of the study. We believe that together, these preclinical data from mice and NHPs highlight the potential for a single administration of ZFP TF to lower tau as a treatment for tauopathies, including Alzheimer's disease.

In December 2017, we entered into a research collaboration and license agreement with 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.

Our revenues have consisted primarily of revenues from our corporate partners, contractual payments from strategic partners for research services and milestones and research grant funding. We expect revenues to continue to fluctuate from period to period and there can be no assurance that new collaborations or partner funding will continue beyond their initial terms or that we are able to meet the milestones specified in these agreements.

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

We expect to continue to devote substantial resources to research and development in the future and expect research and development expenses to increase in the next several years if we are successful in advancing our gene therapy and our genome editing programs in the clinic and, if we are able, to progress our earlier stage product candidates into clinical trials. Pursuant to the terms of the agreements with Kite and Sanofi, certain expenses related to research and development activities will be reimbursed to us. The reimbursement funds to be received from Kite and Sanofi will be recognized as revenue as the costs are incurred and collection is reasonably assured.

***Comparability***

We adopted Accounting Standards Codification Topic 842—*Leases*, or ASC Topic 842, on January 1, 2019, resulting in changes to our accounting policy for leases. We used the modified retrospective approach and recognized the cumulative effect of initially applying ASC Topic 842 as an adjustment to the opening balances of the lease related accounts and accumulated deficit at January 1, 2019. 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 and details on lease related accounts impacted by ASC Topic 842.

***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 in estimate for revenue reversal related to our Pfizer agreement as described in Note 1, Item 1 of this Quarterly Report on Form 10-Q, and for the change to our accounting policy for leases as a result of adopting ASC Topic 842, there have been no significant changes in our critical accounting policies and estimates disclosed in our 2018 Annual Report, as filed with the SEC on March 1, 2019.

**Results of Operations for the Three and Six Months Ended June 30, 2019 and 2018**

***Revenues***

| | Three Months Ended June 30, (in thousands, except percentage values) | | | | Six Months Ended June 30, (in thousands, except percentage values) | | | |
|---|---|---|---|---|---|---|---|---|
| | 2019 | 2018 | Change | % | 2019 | 2018 | Change | % |
| Revenues | $ 17,548 | $ 21,416 | $ (3,868) | (18%) | $ 25,619 | $ 34,053 | $ (8,434) | (25%) |

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 Sanofi as we continue to recognize in revenues upfront and milestone payments received under such agreements over time.

The decrease of $3.9 million in revenues for the three months ended June 30, 2019, compared to the same period in 2018, was primarily due to a decrease of $3.7 million in revenues related to our agreements with Pfizer due to a change in estimate as a result of the expansion of the project scope in 2019, and a $1.5 million decrease in revenues related to Sanofi, partially offset by an increase of $1.5 million in revenue related to our agreement with Kite, which took effect in April 2018.

The decrease of $8.4 million in revenues for the six months ended June 30, 2019, compared to the same period in 2018, was primarily attributable to a decrease of $14.2 million in revenues related to the hemophilia A Pfizer Agreement due to a change in estimate and a $3.9 million decrease in revenues related to our agreement with Sanofi, partially offset by an increase of $9.9 million in revenue related to our agreement with Kite, which took effect in April 2018.

***Operating Expenses***

| | Three Months Ended June 30, (in thousands, except percentage values) | | | | Six Months Ended June 30, (in thousands, except percentage values) | | | |
|---|---|---|---|---|---|---|---|---|
| | 2019 | 2018 | Change | % | 2019 | 2018 | Change | % |
| Operating expenses: | | | | | | | | |
| Research and development | $ 36,455 | $ 29,255 | $ 7,200 | 25% | $ 71,305 | $ 52,802 | $ 18,503 | 35% |
| General and administrative | 14,597 | 11,301 | 3,296 | 29% | 31,715 | 21,388 | 10,327 | 48% |
| Total operating expenses | $ 51,052 | $ 40,556 | $ 10,496 | 26% | $ 103,020 | $ 74,190 | $ 28,830 | 39% |

*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 clinical supply 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 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. Overall increases in the current period include activities attributed to TxCell, which was acquired on October 1, 2018.

The increase of $7.2 million in research and development expenses for the three months ended June 30, 2019, compared to the same period in 2018, was primarily driven by a $4.3 million increase in compensation costs due to headcount growth in our development and technical operations teams to support clinical development trials, a $1.3 million increase in lab supplies, and a $1.2 million increase in facility expense primarily related to our Brisbane facility.

The increase of $18.5 million in research and development expenses for the six months ended June 30, 2019, compared to the same period in 2018, was primarily driven by a $9.6 million increase in compensation cost due to headcount growth in our development and technical operations teams to support clinical development trials, $2.5 million increase in research, preclinical

and clinical expenses, $2.2 million increase in manufacturing related cost as our programs move into the clinic, $2.0 million increase in lab supply expense, and $1.8 million increase in facility expense primarily related to our Brisbane facility.

*General and Administrative Expenses*

General and administrative expenses consist primarily of salaries and personnel related expenses for executive, finance and administrative personnel, stock-based compensation expenses, professional fees, allocated facilities expenses, patent prosecution expenses and other general corporate expenses. As we continue to build out our product portfolio and advance our product candidates into and through the clinic, we expect the growth of our business to require increased general and administrative expenses. Overall increases in the current period include activities attributed to TxCell, which was acquired on October 1, 2018.

The increase of $3.3 million in general and administrative expenses for the three months ended June 30, 2019, compared to the same period in 2018, was primarily due to $3.4 million higher compensation costs due to headcount growth and $0.8 million increased facility expense primarily related to our new Brisbane facility, partially offset by a decrease of $1.0 million in consulting and professional fees, which in 2018 included costs to support the TxCell Acquisition.

The increase of $10.3 million in general and administrative expenses for the six months ended June 30, 2019, compared to the same period in 2018, was primarily due to increases of $6.3 million in compensation related costs due to headcount growth, $0.9 million in legal expense, $1.1 million in consultant expenses, and $1.8 million in facility expense primarily related to our new Brisbane facility. The increases were primarily due to the growth of our business to support the continued advancement of our product candidates into clinical trials.

*Interest and other income, net*

The increases of $0.6 million and $1.5 million in interest and other income, net, for the three and six months ended June 30, 2019 and 2018, respectively, were primarily due to higher interest income resulting from our treasury strategy.

**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. Our most significant use of capital pertains to funding our preclinical and clinical research and development programs, as well as employee compensation.

As of June 30, 2019, we had cash, cash equivalents, marketable securities and interest receivable totaling $450.3 million compared to $400.5 million as of December 31, 2018, with the increase primarily attributable to the proceeds from the underwritten public offering completed in April 2019, partially offset by operating expenditures. Our most significant use of capital pertains to our employee compensation 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 U.S. government-sponsored entity debt securities, corporate debt securities, commercial paper securities and money market funds. Cash in excess of immediate requirements is invested in accordance with our investment policy with a view toward capital preservation and liquidity.

In April 2019, we completed an underwritten public offering of our common stock, in which we sold an aggregate of 12.7 million shares of our common stock at a public offering price of $11.50 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 $136.3 million.

In May 2017, we entered into an amended and restated sales agreement with Cowen and Company, LLC, or Cowen, 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, or the ATM Facility. 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. We have not sold any common stock under the ATM Facility. As of June 30, 2019, the full $75.0 million provided for under the ATM Facility remained available for sale, subject to certain conditions as specified in the agreement.

Since the beginning of 2017, we have received significant amounts of capital as upfront payments under the following collaboration arrangements: $70.0 million received in May 2017 from Pfizer under our hemophilia A agreement, $12.0 million received in January 2018 from Pfizer under our C9ORF72 agreement, and $150.0 million received in April 2018 under our collaboration agreement with Kite. Our collaboration agreements provide for the payment of development, regulatory, and commercial milestones. For more information see Note 5 — Major Customers, Partnerships and Strategic Alliances in the condensed consolidated financial statements of this Quarterly Report on Form 10-Q.

***Cash Flows***

*Operating activities*

Net cash used in operating activities was $84.7 million for the six months ended June 30, 2019 primarily reflecting our net loss of $72.6 million, a decrease in deferred revenues of $10.8 million, an increase in accounts receivable of $7.4 million and an increase in prepaid expenses and other assets of $5.4 million, partially offset by stock-based compensation of $9.4 million.

Net cash provided by operating activities of $104.3 million for the six months ended June 30, 2018 primarily reflected the increase in deferred revenue due to the $150.0 million upfront license payment from Kite, partially offset by the net loss for the period as well as an increase in prepaid expenses and increased business activities.

*Investing activities*

Net cash used in investing activities for the six months ended June 30, 2019 and 2018, was $27.2 million and $323.7 million, respectively. The decrease in net cash used in the six months ended June 30, 2019, compared to the same period in 2018, was due primarily to a net decrease in purchases and maturities of marketable securities.

*Financing activities*

Net cash provided by financing activities for the six months ended June 30, 2019 was $140.1 million primarily related to our April 2019 underwritten public offering of our common stock, which generated net proceeds of approximately $136.3 million, with the remainder primarily related to the $4.1 million from the issuance of common stock upon exercise of stock options.

Net cash provided by financing activities for the six months ended June 30, 2018 was $229.0 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 $13.3 million from the issuance of common stock upon exercise of stock options.

***Operating Capital and Capital Expenditure Requirements***

We anticipate continuing to incur operating losses for at least the next several years. While we expect our rate of cash usage to increase in the future, in particular to support our product development endeavors, we believe that the available cash resources, as well as funds received from corporate collaborators, strategic partners and research grants will enable us to maintain our currently planned operations through at least the next twelve months from the date the financial statements are issued. Future capital requirements will be substantial and if our capital resources are insufficient to meet future capital requirements, we will need to raise additional capital to fund our operations through equity or debt financing. We regularly consider fund raising opportunities and may decide, from time to time, to raise capital based on various factors, including market conditions and our plans of operation. Additional capital may not be available on terms acceptable to us, or at all. If adequate funds are not available, or if the terms of potential funding sources are unfavorable, our business and our ability to advance our product candidate pipeline would be harmed. Furthermore, any sales of additional equity securities, including any sales under our ATM Facility, 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; 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 2018 Annual Report and there have been no material changes outside the ordinary course of business in the previously disclosed contractual commitments during the six months ended

June 30, 2019. See Note 1 to our condensed consolidated financial statements appearing elsewhere in this Quarterly Report on Form 10-Q for a discussion of the Option Agreement with Brammer Bio MA, which may increase our contractual commitments in the future.

## 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. government-sponsored entity debt securities, corporate debt securities, commercial paper securities and money market funds. 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 June 30, 2019 have not changed materially from those discussed in Item 7A of our 2018 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 June 30, 2019. Based on that evaluation, as of June 30, 2019, 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.

### Changes 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 June 30, 2019 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. This Form 10-Q contains forward-looking information based on our current expectations. Because our actual results may differ materially from any forward-looking statements made by or on our behalf, this section includes a discussion of important factors that could affect our actual future results, including, but not limited to, our revenues, expenses, net loss and net loss per share. You should carefully consider the information described in the following risk factors, together with the other information appearing elsewhere in this report, before making an investment decision regarding our common stock.

**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, and we are therefore substantially dependent on the results of clinical trials of our lead therapeutic programs. Our failure to enroll sufficient patients to conduct these clinical trials, demonstrate safety or obtain positive clinical trial results, or our inability to meet the expected timeline of these 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 and complete 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 have initiated clinical sites in a Phase 1/2 clinical trial of ST-920, an investigational gene therapy product candidate for Fabry disease, and we expect to treat the first subject by year end 2019. 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 TX-200, 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-913 and SB-318, the endpoints needed to support regulatory approvals may be different than originally anticipated. For example, in order to support regulatory approval for SB-913 and SB-318, 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 assay we used 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 February 2019, we announced interim results of the CHAMPIONS Study. A newly developed sensitive quantitative assay (lower limit of quantification of 0.78 nmol/hour/mL) was used to measure plasma IDS activity for these interim results. Small increases in IDS enzyme activity compared to baseline were recorded in the two patients receiving the mid-dose and in one patient receiving the high-dose. At 24 weeks post-dosing, these measurements remained within the expected range for baseline values (less than 10 nmol/hour/mL, as compared to the normal range, which is estimated at greater than 82 nmol/hour/mL). While the newly developed, more sensitive assay was able to detect IDS at lower levels, there can be no guarantee that we will be able to continue to be able to detect IDS in patients or otherwise show direct evidence of efficacy or gene editing. Moreover, these interim results cast doubt with regard to whether there will be evidence of a clinical benefit, and it is possible that we may never see a clinical benefit to patients treated with SB-913. This may delay or preclude continued development and/or any regulatory approvals for SB-913. In April 2019, we provided an update on the SB-318, SB-913 and SB-FIX trials, announcing that no additional patients are expected to be treated with first-generation ZFNs given that clinical benefit has not been demonstrated in analyses conducted to date in the ongoing clinical trials and the expected near-term clinical development of second-generation ZFNs. We are planning a new clinical trial to evaluate second-generation ZFNs and other potential modifications to enhance the *in vivo* delivery of the ZFNs for SB-913 to treat MPS II, which is planned to begin by year end 2020. While we expect to use data from this study to make a Phase III decision for the SB-913 program in 2020 and to define the next steps for the SB-318 and SB-FIX programs, there can be no assurance that we will be able to effectively deliver second-generation ZFNs to produce a clinical benefit to patients treated with SB-913, SB-318 and SB-FIX or any of our other product candidates.

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, initial or interim data from clinical trials should be considered carefully and with caution since the final data may be materially different from the preliminary, initial or interim 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. From time to time, we have and may in the future publish or report preliminary, initial or interim data from our clinical trials, such as the preliminary, initial and interim data we have announced from the CHAMPIONS Study (SB-913), the Alta Study (SB-525), the EMPOWERS Study (SB-318) and the THALES Study (SB-400, which involved very early data from the first dosed patient). Preliminary, initial or interim data from our clinical trials and those of our partners 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. In this regard, such data may show initial evidence of clinical benefit but as patients continue to be followed and more patient data becomes available, there is a risk that any therapeutic effects will not be durable in patients and/or will decrease over time or cease entirely. Preliminary, initial or interim data also remain subject to audit and verification procedures that may result

in the final data being materially different from such preliminary, initial or interim data. As a result, preliminary, initial or interim 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. Moreover, the interim results recently announced for SB-913 and SB318 cast doubt with regard to whether there will be evidence of a clinical benefit of either product candidate. In April 2019, we provided an update on the SB-318, SB-913 and SB-FIX trials, announcing that no additional patients are expected to be treated with first-generation ZFNs given that clinical benefit has not been demonstrated in analyses conducted to date in the ongoing clinical trials and the expected near-term clinical development of second-generation ZFNs. We are planning a new clinical trial to evaluate second-generation ZFNs and other potential modifications to enhance the *in vivo* delivery of the ZFNs for SB-913 to treat MPS II, which is planned to begin by year end 2020. While we expect to use data from this study to make a Phase III decision for the SB-913 program in 2020 and to define the next steps for the SB-318 and SB-FIX programs, there can be no assurance that we will be able to effectively deliver second-generation ZFNs to produce a clinical benefit to patients treated with SB-913, SB-318 and SB-FIX or any of our other product candidates. Furthermore, these programs (other than TX-200 that we acquired through the acquisition of TxCell, or 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 to FDA and IRB oversight, under guidelines promulgated by the National Institutes of Health, or NIH, gene therapy clinical trials are also subject to review and oversight by an institutional biosafety committee, or IBC, a local institutional committee that reviews and oversees research utilizing recombinant or synthetic nucleic acid molecules at that institution. The IBC assesses the safety of the research and identifies any potential risk to public health or the environment. While the NIH guidelines are not mandatory unless the research in question is being conducted at or sponsored by institutions receiving NIH funding of recombinant or synthetic nucleic acid molecule research, many companies and other institutions not otherwise subject to the NIH guidelines voluntarily follow them. Although the FDA decides whether individual gene therapy protocols may proceed, the review process and determinations of other reviewing bodies can impede or delay the initiation of a clinical trial, even if the FDA has reviewed the trial and approved its initiation.

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 IBC and NIH 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 the TxCell Acquisition, we acquired the rights, among others, to TxCell's first CAR-Treg product candidate, TX-200, 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 TX-200 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 TX-200, 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.

In April 2019, we provided an update on the SB-318, SB-913 and SB-FIX trials, announcing that no additional patients are expected to be treated with first-generation ZFNs given that clinical benefit has not been demonstrated in analyses conducted to date in the ongoing clinical trials and the expected near-term clinical development of second-generation ZFNs. We are planning a new clinical trial to evaluate second-generation ZFNs and other potential modifications to enhance the *in vivo* delivery of the ZFNs for SB-913 to treat MPS II, which is planned to begin by year end 2020. While we expect to use data from this study to make a Phase III decision for the SB-913 program in 2020 and to define the next steps for the SB-318 and SB-FIX programs, there can be no assurance that we will be able to effectively deliver second-generation ZFNs to produce a clinical benefit to patients treated with SB-913, SB-318 and SB-FIX or any of our other product candidates.

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 infused the first patient in the Phase 1/2 clinical trial evaluating ST-400 for the treatment of beta thalassemia in the first quarter of 2019. Moreover, we had recently begun to enroll patients into the Phase 1/2 clinical trials evaluating SB-FIX for the treatment of hemophilia B, SB-318 for the treatment of MPS I and SB-913 for MPS II, none of which are enrolling additional patients due to our decision to plan a new clinical trial to evaluate second-generation ZFNs and other potential modifications to enhance the *in vivo* delivery of the ZFNs for SB-913 to treat MPS II, which we expect to begin by year end 2020. If we are not able to enroll the necessary number of patients in a timely manner, we may not be able to complete these clinical trials. 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 EMA'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 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.

***Regenerative Medicine Advanced Therapy, or RMAT, designation, may not lead to a faster development or regulatory review or approval process and it does not increase the likelihood that SB-525 will receive marketing approval.***

We have received Regenerative Medicine Advanced Therapy, or RMAT, designation for SB-525 to treat severe hemophilia A. RMAT designation is intended to expedite review of a cell therapy, therapeutic tissue engineering product, human cell and tissue product, or any combination product using such therapies or products, intended to treat, modify, reverse, or cure a serious or life-threatening disease or condition and for which preliminary clinical evidence indicates the potential to address unmet medical needs for such a disease or condition. RMAT designation provides potential benefits that include more frequent meetings with the FDA to discuss the development plan for the product candidate, and eligibility for rolling review and priority review of the related BLA. Products granted RMAT designation may also be eligible for accelerated approval on the basis of a surrogate or intermediate endpoint reasonably likely to predict long-term clinical benefit, or reliance upon data obtained from a meaningful number of sites, including through expansion to additional sites. There is no assurance that we will be able to obtain additional RMAT designations for any of our other product candidates. RMAT designation does not change the FDA's standards for product approval, and there is no assurance that such designation will result in expedited review or approval or that the approved indication will not be narrower than the indication covered by the designation. Additionally, RMAT designation can be revoked if the criteria for eligibility cease to be met as clinical data emerges.

***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 Sanofi for our beta thalassemia and sickle cell disease product candidates.

If we are unable to find additional partners or if the partners 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 that 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 using ZFNs or ZFP TFs 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 clinical benefit.

In February 2019, we announced our development of second generation, potentially more potent ZFN constructs designed to increase editing efficiency. *In vitro* data of these second-generation ZFNs were reviewed by the FDA. The *in vitro* data showed three potential advantages for use in the clinic: (1) a five to thirty-fold improvement in efficiency and potency due to structural changes; (2) the ability to function equally well in the patients who have a single nucleotide polymorphism in the target locus in the albumin gene (approximately 20% of the population); and (3) improvements in specificity. The second-generation

ZFNs are being manufactured and we are planning a new clinical trial to evaluate second-generation ZFNs for SB-913 to treat MPS II, which is planned to begin by year end 2020; however, there can be no assurances that we will be able to effectively deliver this second-generation ZFN to produce a clinical benefit to patients treated with SB-913, SB-318 and SB-FIX or any of our other product candidates. Additional data from our *in vivo* genome editing programs will be assessed before potential integration plans for the second-generation ZFNs are finalized.

***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 payors 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 changed 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% as of 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, there have been two signed 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 that repealed, 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, there was a signed 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, amended 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 December 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. On December 14, 2018, a Texas U.S. District Court Judge ruled that the Affordable Care Act is unconstitutional in its entirety because the "individual mandate" was repealed by the U.S. Congress as part of the Tax Cuts and Jobs Act of 2017 Act. While the Texas U.S. District Court Judge, as well as the current administration and CMS, have stated that the ruling will have no immediate effect pending appeal of the decision, it is unclear how this decision, subsequent appeals, and other efforts to repeal and replace the Affordable Care Act will impact 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 current administration's budget proposal for fiscal year 2019. Additionally, the current 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, or the HHS, has begun the process of soliciting feedback on some of these measures and, at the same time, is immediately implementing others under its existing authority. Although a number of these, and other potential, proposed measures will require additional authorization to become effective. Congress and the executive branch 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, marketing expenditures; or drug pricing; and/or ensure the registration and compliance of sales 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 UK 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 will 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 will be required for us to operate a manufacturing facility and can be time-consuming to obtain. As a manufacturer of pharmaceutical products, we also will 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 will 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 February 15, 2019, we had 302 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 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;
- managing our internal development efforts effectively while complying with our contractual obligations to licensors, licensees, contractors and other third parties;
- improving our managerial, development, operational, information technology, and finance systems; and
- expanding our facilities.

As our operations expand, we will also need to manage additional relationships with various strategic partners, suppliers and other third parties. Our future financial performance and our ability to commercialize our product candidates and to compete effectively will depend, in part, on our ability to manage any future growth effectively. To that end, we must be able to manage our development efforts and preclinical studies and clinical trials effectively and hire, train and integrate additional management, research and development, manufacturing, administrative and sales and marketing personnel. Our failure to accomplish any of these tasks could prevent us from successfully growing our company.

**Risks Relating to our Industry**

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

Any products that we or our collaborators or strategic partners develop by using our ZFP technology platform will enter into highly competitive markets. Even if we are able to generate products that are safe and effective for their intended use, competing technologies may prove to be more effective or less expensive, which, to the extent these competing technologies achieve market acceptance, will limit our revenue opportunities. In some cases, competing technologies have proven to be effective and less expensive. Competing technologies may include other methods of regulating gene expression or modifying genes. ZFNs and ZFP TFs have broad application in the life sciences industry and compete with a broad array of new technologies and approaches being applied to genetic research by many companies. Competing proprietary technologies with our product development focus include but are not limited to:

- For genome editing and gene therapy products:
  - recombinant proteins;
  - other gene therapy/cDNAs;

- antisense;
- siRNA and microRNA approaches, exon skipping;
- small molecule drugs;
- monoclonal antibodies;
- CRISPR/Cas technology; and
- TALE proteins, meganucleases, and MegaTALs.

- Our non-therapeutic applications compete against similar technologies:
  - For protein production: gene amplification, CRISPR/Cas technology, TALE technology, insulator technology, and mini-chromosomes;
  - For target validation: antisense, siRNA, TALE technology and CRISPR/Cas technology;
  - For plant agriculture: recombination approaches, mutagenesis approaches, TALE technology, CRISPR/Cas technology, mini-chromosomes; and
  - For transgenic animals: somatic nuclear transfer, embryonic stem cell, TALE, CRISPR/Cas technology and transposase technologies.

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

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

These organizations also compete with us to:

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

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

***Our product candidates are based on novel technologies, which makes it difficult to predict the timing and costs of development and of subsequently obtaining regulatory approval.***

We have concentrated our research and development efforts on genome editing, gene therapy, gene regulation and cell therapy. The regulatory approval process for novel product candidates such as ours is unclear and may be lengthier and more expensive than the process for other, better-known or more extensively studied product candidates.

Adverse developments in clinical trials of gene therapy products conducted by others may cause the FDA or other oversight bodies to change the requirements for approval of our product candidates.

These regulatory review committees and advisory groups, and any new guidelines they promulgate, may lengthen the regulatory review process, require us to perform additional preclinical studies or clinical trials, increase our development costs, lead to changes in regulatory positions and interpretations, delay or prevent approval and commercialization of our current or future product candidates or lead to significant post-approval limitations or restrictions. As we advance our product candidates, we will be required to consult with these regulatory and advisory groups and comply with applicable guidelines. If we fail to do so, we may be required to delay or discontinue development of our product candidates. These additional processes may result in a review and approval process that is longer than we otherwise would have expected. Delay or failure to obtain, or unexpected costs in obtaining, the regulatory approval necessary to bring a potential product to market could decrease our ability to generate sufficient product revenue, and our business, financial condition, results of operations and prospects would be harmed. Even if our product candidates are approved, we expect that the FDA will require us to submit follow-up data regarding our clinical trial subjects for a number of years after any approval. If this follow-up data shows negative long-term safety or efficacy outcomes for these patients, the FDA may revoke its approval or change the label of our products in a manner that could have an adverse impact on our business.

In addition, adverse developments in clinical trials of gene therapy or cell therapy products conducted by others may cause the FDA or other oversight bodies to change the requirements for approval of our product candidates. The FDA and EMA have only very recent and limited experience in the approval of *in vivo* gene therapy products. As a result, it is difficult to determine how long it will take or how much it will cost to obtain regulatory approvals for our product candidates.

***Our gene therapy approach utilizes vectors derived from viruses, which may be perceived as unsafe or may result in unforeseen adverse events. Negative public opinion and increased regulatory scrutiny of gene therapy may damage public***

***perception of the safety of our product candidates and adversely affect our ability to conduct our business or obtain regulatory approvals for our product candidates.***

Gene therapy remains a novel technology, with only one *in vivo* gene therapy product approved for a genetic disease to date in the United States and only two *in vivo* gene therapy products for genetic diseases approved to date in the European Union. Public perception may be influenced by claims that gene therapy is unsafe, and gene therapy may not gain the acceptance of the public or the medical community. In particular, our success will depend upon physicians who specialize in the treatment of genetic diseases targeted by our product candidates, prescribing treatments that involve the use of our product candidates in lieu of, or in addition to, existing treatments with which they are familiar and for which greater clinical data may be available. More restrictive government regulations or negative public opinion would have an adverse effect on our business, financial condition, results of operations and prospects and may delay or impair the development and commercialization of our product candidates or demand for any products we may develop. For example, earlier gene therapy trials led to several well-publicized adverse events, including cases of leukemia and death seen in other trials using other vectors. Serious adverse events in our clinical trials, or other clinical trials involving gene therapy products or our competitors' products, even if not ultimately attributable to the relevant product candidates, and the resulting publicity, could result in increased government regulation, unfavorable public perception, potential regulatory delays in the testing or approval of our product candidates, stricter labeling requirements for those product candidates that are approved and a decrease in demand for any such product candidates.

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

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

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

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

**Risks Relating to our Finances**

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

We have generated operating losses since we began operations in 1995. Our net losses for the years ended December 31, 2018, 2017 and 2016 were $68.9 million, $54.6 million and $71.7 million, respectively. The extent of our future losses and the timing of profitability are uncertain, and we expect to incur losses for the foreseeable future. We have been engaged in developing our ZFP technology since inception, which has and will continue to require significant research and development expenditures. To date, we have generated our funding from issuance of equity securities, revenues derived from collaboration agreements, other strategic partnerships in non-therapeutic applications of our technology, federal government research grants and grants awarded by research foundations. As of June 30, 2019 we had an accumulated deficit of $634.2 million. Since our initial public offering in 2000, we have generated an aggregate of approximately $794.2 million in gross proceeds from the sale of our equity securities. We expect to continue to incur additional operating losses for the next several years as we continue to advance our product candidates. If the time required to generate significant product revenues and achieve profitability is longer than we currently anticipate or if we are unable to generate liquidity through equity financing or other sources of funding, we may be forced to curtail or suspend our operations.

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

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

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

We began operations in 1995, are in the early phases of product development for the most advanced candidates in our therapeutics pipeline, and we have incurred significant losses since inception. To date, our revenues have been generated from collaboration agreements, other collaborations in non-therapeutic applications of our technology, federal government research grants and grants awarded by research foundations. Our focus on higher-value therapeutic product development and related collaboration requires us to incur substantial expenses associated with product development. In addition, the preclinical or clinical failure of any single product may have a significant effect on the actual or perceived value of our stock. Our business is subject to all of the risks inherent in the development of a new technology, which includes the need to:

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

***Comprehensive U.S. tax reform legislation could increase the tax burden on our orphan drug programs and adversely affect our business and financial condition.***

The U.S. government enacted comprehensive tax legislation in 2017 that included significant changes to the taxation of business entities. These changes included, among others, (i) a permanent reduction to the corporate income tax rate from a top marginal rate of 35% to a flat rate of 21%, (ii) a partial limitation on the deductibility of business interest expense and net operating loss carryforwards, (iii) a shift of the U.S. taxation of multinational corporations from a tax on worldwide income to a territorial system (along with certain rules designed to prevent erosion of the U.S. income tax base) and (iv) a one-time tax on accumulated offshore earnings held in cash and illiquid assets, with the latter taxed at a lower rate. Further, the comprehensive tax legislation, among other things, reduced the orphan drug tax credit from 50% to 25% of qualifying expenditures. When and if we become profitable, this reduction in tax credits may result in an increased federal income tax burden on our orphan drug programs as it may cause us to pay federal income taxes earlier under the revised tax law than under the prior law and, despite being partially off-set by a reduction in the corporate tax rate as described above, may increase our total federal tax liability attributable to such programs.

Notwithstanding the reduction in the corporate income tax rate, the overall impact of this comprehensive tax legislation resulted in an overall reduction in our deferred tax assets, and our business and financial condition could still be adversely affected as additional guidance and regulations are issued with respect to the original tax law change. In addition, it is uncertain if and to what extent various states will conform to this comprehensive tax legislation. The impact of this comprehensive tax legislation on holders of our common stock is also uncertain and could be adverse. Investors should consult with their legal and tax advisors with respect to this comprehensive tax legislation and the potential tax consequences of investing in or holding our common stock.

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

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

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

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

In addition, conflicts could arise between us and our collaborators resulting from disputes regarding our or our collaborators' or strategic partners' performance under the applicable agreement, including disputes arising from alleged breaches of our agreements with our collaborators and strategic partners. For example, we have certain confidentiality obligations to our collaborators and strategic partners under our agreements with them, and it is possible that, in connection with the data security incident we disclosed in April 2018, we could be subject to claims that we have breached our confidentiality obligations, which could result in damages payable by us and/or the affected collaborator or strategic partner seeking to terminate its agreement with us.

Any of these developments could harm our product development efforts and otherwise adversely affect our business and prospects.

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

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

For example, under our agreements with Kite, Pfizer and Sanofi, they have control and broad discretion over all or certain aspects of the clinical development and commercialization of any product developed under the agreement, and we will have little, if any, influence on how these programs will be conducted. Our lack of control over the clinical development in such agreements could cause delays or other difficulties in the development and commercialization of our product candidates, which may prevent us from completing the intended IND filings in a timely fashion and receiving any milestone, royalty payments and other benefits under the agreement. In addition, under their respective agreements, our third-party collaborators have certain rights to terminate the agreements by providing us with advance notices, therefore, the actual milestone payments that we may receive under these agreements may be substantially lower than the full amounts provided for under these agreements.

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

Our collaborators or strategic partners may adopt alternative technologies, which could decrease the marketability of ZFP technology. Additionally, because many of our collaborators or strategic partners are likely to be working on more than one development project, they could choose to shift their resources to projects other than those they are working on with us. If they do so, this would delay our ability to test our technology and would delay or terminate the development of potential products based on our ZFP technology. Further, our collaborators and strategic partners may elect not to develop products arising out of our collaborative and strategic partnering arrangements or to devote sufficient resources to the development, manufacturing, marketing or sale of these products. If they terminate the collaborative relationship with us, we will be required to seek the support of other partners or collaborators. We may not have sufficient resources and expertise to develop these programs by ourselves, and we may not be able to identify a suitable partner or negotiate a favorable collaboration agreement to allow us to continue the development of these programs. If any of these events occur, we may not be able to develop our technologies or commercialize our products.

***If the licensed products under our non-therapeutic license agreements are not successfully commercialized, or our third-party licensees terminate our agreements, our ability to generate revenue under these license agreements may be limited.***

We have a number of collaboration agreements with third parties whereby we licensed our ZFP technologies to develop products in non-therapeutic fields, such as laboratory research reagents, protein pharmaceuticals, and, transgenic animals, as well as plant agriculture.

We cannot be certain that we or our collaboration partners will succeed in the development of commercially viable products in these non-therapeutic fields of use, and there is no guarantee that we or our collaboration partners will achieve the milestones set forth in the respective license agreements. To the extent we or our collaboration partners do not succeed in developing and commercializing products or if we or our collaboration partners fail to achieve such milestones, our revenues and benefits under the license agreements will be limited. In the event our third party licensees decide to terminate the license agreements, our ability to generate revenue under such license agreements will cease.

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

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

**Risks Relating to our Intellectual Property**

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

Our commercial success may depend in part on obtaining and enforcing patent protection for our technology and successfully defending any of our patents that may be challenged. Obtaining and enforcing pharmaceutical and biotechnology patents is costly, time consuming and complex, and we may not be able to file and prosecute all necessary or desirable patent applications, or maintain, enforce and license any patents that may issue from such patent applications, at a reasonable cost or in a timely manner. It is also possible that we will fail to identify patentable aspects of our research and development output before it is too late to obtain patent protection. The patent positions of pharmaceutical and biotechnology companies can be highly uncertain and can involve complex legal and factual questions. No consistent policy regarding the breadth of claims allowed in biotechnology patents has emerged to date. Accordingly, we cannot predict the breadth of claims that may issue from any patent applications that we own or license, nor are we able to predict whether any third-party patents might issue with claims that are relevant to our product candidates or technologies.

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

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

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

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

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

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

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

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

***If we are unable to obtain or protect intellectual property rights related to our product candidates, we may not be able to compete effectively in the intended markets.***

We rely upon a combination of patents, trade secret protection and confidentiality agreements to protect the intellectual property related to our product candidates. The strength of patents in the biotechnology and pharmaceutical field involves complex legal and scientific questions and can be uncertain. The patent applications that we own or in-license may fail to result in issued patents with claims that cover our product candidates in the United States or in other foreign countries. There is no assurance that all of the potentially relevant prior art relating to our patents and patent applications has been found, which can invalidate a patent or prevent a patent from issuing from a pending patent application. Even if patents do successfully issue and even if such patents cover our product candidates, third parties may challenge their validity, enforceability or scope, which may result in such patents being narrowed or invalidated. Furthermore, even if they are unchallenged, our patents and patent applications may not adequately protect our intellectual property, provide exclusivity for our product candidates or prevent others from designing around our claims. Our competitors may be able to circumvent our patents by developing similar or alternative product candidates in a non-infringing manner. Any of these outcomes could impair our ability to prevent competition from third parties, which may have an adverse impact on our business.

If the patent applications we hold or have in-licensed with respect to our programs or product candidates fail to issue, if their breadth or strength of protection is threatened, or if they fail to provide meaningful exclusivity for our product candidates, it could dissuade companies from collaborating with us to develop product candidates, and threaten our ability to commercialize, future products. We have filed several patent applications covering our product candidates recently. We cannot offer any assurances about which, if any, patents will issue, the breadth of any such patent or whether any issued patents will be found invalid and unenforceable or will be threatened by third parties. Any successful challenge to these patents or any other patents owned by or licensed to us could deprive us of rights necessary for the successful commercialization of any product candidates that we may develop. Further, if we encounter delays in regulatory approvals, the period of time during which we could market a product candidate under patent protection could be reduced. Since patent applications in the United States and most other countries are confidential for a period of time after filing, and some remain so until issued, we cannot be certain that we were the first to file any patent application related to a product candidate. Furthermore, if third parties have filed such patent applications, an interference or derivation proceeding in the United States can be initiated by a third party to determine who was the first to invent any of the subject matter covered by the patent claims of our applications.

***Patent terms may be inadequate to protect our competitive position on our product candidates for an adequate amount of time.***

Patents have a limited lifespan. In the United States, the natural expiration of a patent is generally 20 years from its earliest U.S. non-provisional filing date or from the filing date of the corresponding international application. Various extensions

may be available. However, the life of a patent, and the protection it affords, is limited. Even if patents covering our product candidates are obtained, once the patent life has expired for a product, we may be open to competition from generic medications. Given the amount of time required for the development, testing and regulatory review of new product candidates, patents protecting such candidates might expire before or shortly after such candidates are commercialized. As a result, our owned and licensed patent portfolio may not provide us with sufficient rights to exclude others from commercializing products similar or identical to ours.

***If we are unable to protect the confidentiality of our trade secrets, the value of our technology could be materially adversely affected and our business would be harmed.***

We rely on trade secret protection and confidentiality agreements to protect proprietary know-how that is not patentable or that we elect not to patent, processes for which patents are difficult to enforce and any other elements of our product candidate discovery and development processes that involve proprietary know-how, information or technology that is not covered by patents. However, trade secrets can be difficult to protect. We seek to protect our proprietary technology and processes, in part, by entering into confidentiality agreements with our employees, consultants, scientific advisors and contractors. We also seek to preserve the integrity and confidentiality of our data and trade secrets by maintaining physical security of our premises and physical and electronic security of our information technology systems. While we have confidence in these individuals, organizations and systems, agreements or security measures have been and may in the future be breached, and we may not have adequate remedies for any breach. See also the risk factor titled, "*Significant disruptions of our information technology systems or data security incidents could result in significant financial, legal, regulatory, business and reputational harm to us*." In addition, our trade secrets may otherwise become known or be independently discovered by competitors.

Although we expect all of our employees and consultants to assign their inventions to us, and all of our employees, consultants, advisors and any third parties who have access to our proprietary know-how, information or technology to enter into confidentiality agreements, we cannot provide any assurances that all such agreements have been duly executed or that our trade secrets and other confidential proprietary information will not be disclosed or that competitors will not otherwise gain access to our trade secrets or independently develop substantially equivalent information and techniques. Misappropriation or unauthorized disclosure of our trade secrets could impair our competitive position and may have a material adverse effect on our business. Additionally, if the steps taken to maintain our trade secrets are deemed inadequate, we may have insufficient recourse against third parties for misappropriating the trade secret. In addition, others may independently discover our trade secrets and proprietary information. For example, the FDA, as part of its Transparency Initiative, is currently considering whether to make additional information publicly available on a routine basis, including information that we may consider to be trade secrets or other proprietary information, and it is not clear at the present time how the FDA's disclosure policies may change in the future, if at all.

Further, the laws of some foreign countries do not protect proprietary rights to the same extent or in the same manner as the laws of the United States. As a result, we may encounter significant problems in protecting and defending our intellectual property both in the United States and abroad. If we are unable to prevent material disclosure of the non-patented intellectual property related to our technologies to third parties, and there is no guarantee that we will have any such enforceable trade secret protection, we may not be able to establish or maintain a competitive advantage in our market, which could materially adversely affect our business, results of operations and financial condition.

***Third-party claims of intellectual property infringement may prevent or delay our development and commercialization efforts.***

Our commercial success depends in part on our avoiding infringement of the patents and proprietary rights of third parties. There is a substantial amount of litigation, both within and outside the United States, involving patent and other intellectual property rights in the biotechnology and pharmaceutical industries, including patent infringement lawsuits, interferences, oppositions, *ex part*e reexaminations, post-grant review, and *inter partes* review proceedings before the U.S. Patent and Trademark Office, or U.S. PTO, and corresponding foreign patent offices. Numerous U.S. and foreign issued patents and pending patent applications, which are owned by third parties, exist in the fields in which we are pursuing development candidates. As the biotechnology and pharmaceutical industries expand and more patents are issued, the risk increases that our product candidates may be subject to claims of infringement of the patent rights of third parties.

Third parties may assert that we are employing their proprietary technology without authorization, and such parties may be able to sustain the costs of complex patent litigation more effectively than we can because they have substantially greater resources. There may be third-party patents or patent applications with claims to materials, formulations, methods of manufacture or methods for treatment related to the use or manufacture of our product candidates. For example, we are aware of certain patents held by a third party related to certain vector manufacturing methods that are currently being used in certain of our product candidates. We have not yet finalized the commercial scale manufacturing process for any of our product candidates. If our commercial scale manufacturing process utilizes these vector manufacturing methods, and if these third-party patents are in force at the time of commercialization, we may need to use or develop a non-infringing manufacturing method or seek a license to these patents. In any event, if any third-party patents were held by a court of competent jurisdiction to cover the manufacturing methods

of any of our product candidates, any molecules formed during the manufacturing process or any final product itself, the holders of any such patents may be able to block our ability to commercialize such product candidate unless we obtained a license under the applicable patents, or until such patents expire. Similarly, if any third-party patents were held by a court of competent jurisdiction to cover aspects of our formulations, processes for manufacture or methods of use, including combination therapy, the holders of any such patents may be able to block our ability to develop and commercialize the applicable product candidate unless we obtained a license, or until such patents expires. In either case, such a license may not be available on commercially reasonable terms or at all. The inability to obtain required licenses on reasonable commercial terms, if at all, could delay or prevent the preclinical evaluation, drug development collaborations, clinical testing, and/or commercialization of the affected product candidates. Moreover, because patent applications can take many years to issue, there may be currently pending patent applications which may later result in issued patents that our product candidates may infringe. In addition, third parties may obtain patents in the future and claim that use of our technologies infringes upon these patents.

Parties making claims against us may obtain injunctive or other equitable relief, which could effectively block our ability to further develop and commercialize one or more of our product candidates. Defense of these claims, regardless of their merit, would involve substantial litigation expense and would be a substantial diversion of employee resources from our business. In the event of a successful claim of infringement against us, we may have to pay substantial damages, including treble damages and attorneys' fees for willful infringement, pay royalties, redesign our infringing products or obtain one or more licenses from third parties, which may be impossible or require substantial time and monetary expenditure. Furthermore, because of the substantial amount of discovery required in connection with intellectual property litigation or administrative proceedings, there is a risk that some of our confidential information could be compromised by disclosure. In addition, any uncertainties resulting from the initiation and continuation of any litigation could have a material adverse effect on our ability to raise additional funds or otherwise have a material adverse effect on our business, results of operations, financial condition and prospects.

***We may not be successful in obtaining or maintaining necessary rights to gene or cell therapy product components and processes for our development pipeline through acquisitions and in-licenses.***

Presently, we believe we have rights to the intellectual property, through licenses from third parties and under patents that we own, to develop our gene and cell therapy product candidates. Because our programs may involve additional product candidates, such as TX-200 and potential future CAR-Treg therapies that may require the use of proprietary rights held by third parties, the growth of our business will likely depend in part on our ability to acquire, in-license or use these proprietary rights. In addition, our product candidates may require specific formulations to work effectively and efficiently and these rights may be held by others. We may be unable to acquire or in-license any compositions, methods of use, processes or other third-party intellectual property rights from third parties that we identify on commercially reasonable terms, if at all. The licensing and acquisition of third-party intellectual property rights is a competitive area, and a number of more established companies are also pursuing strategies to license or acquire third-party intellectual property rights that we may consider attractive. These established companies may have a competitive advantage over us due to their size, cash resources and greater clinical development and commercialization capabilities.

For example, we sometimes collaborate with U.S. and foreign academic institutions to accelerate our preclinical research or development under written agreements with these institutions. Typically, these institutions provide us with an option to negotiate a license to any of the institution's rights in technology resulting from the collaboration. Regardless of such right of first negotiation for intellectual property, we may be unable to negotiate a license within the specified time frame or under terms that are acceptable to us. If we are unable to do so, the institution may offer the intellectual property rights to other parties, potentially blocking our ability to pursue our program.

In addition, companies that perceive us to be a competitor may be unwilling to assign or license rights to us. We also may be unable to license or acquire third-party intellectual property rights on terms that would allow us to make an appropriate return on our investment. If we are unable to successfully obtain rights to required third-party intellectual property rights, our business, financial condition and prospects for growth could suffer.

***If we fail to comply with our obligations in the agreements under which we license intellectual property rights from third parties or otherwise experience disruptions to our business relationships with our licensors, we could lose license rights that are important to our business.***

We are a party to a number of intellectual property license agreements that are important to our business and expect to enter into additional license agreements in the future. Our existing license agreements impose, and we expect that future license agreements will impose, various diligence, milestone, royalty and other obligations on us. If we fail to comply with our obligations under these agreements, or we are subject to a bankruptcy, the licensor may have the right to terminate the license, in which event we would not be able to market products covered by the license.

We may need to obtain licenses from third parties to advance our research or allow commercialization of our product candidates, and we have done so from time to time. We may fail to obtain any of these licenses at a reasonable cost or on

reasonable terms, if at all. In that event, we may be required to expend significant time and resources to develop or license replacement technology. If we are unable to do so, we may be unable to develop or commercialize the affected product candidates, which could harm our business significantly. We cannot provide any assurances that third-party patents do not exist that might be enforced against our current product candidates or future products, resulting in either an injunction prohibiting our sales, or, with respect to our sales, an obligation on our part to pay royalties and/or other forms of compensation to third parties.

In many cases, patent prosecution of our in-licensed technology is controlled solely by the licensor. If our licensors fail to obtain and maintain patent or other protection for the proprietary intellectual property we license from them, we could lose our rights to the intellectual property or our exclusivity with respect to those rights, and our competitors could market competing products using the intellectual property. In certain cases, we control the prosecution of patents resulting from licensed technology. In the event we breach any of our obligations related to such prosecution, we may incur significant liability to our licensing partners. Licensing of intellectual property is of critical importance to our business and involves complex legal, business and scientific issues and is complicated by the rapid pace of scientific discovery in our industry. Disputes may arise regarding intellectual property subject to a licensing agreement, including:

- the scope of rights granted under the license agreement and other interpretation-related issues;
- the extent to which our technology and processes infringe on intellectual property of the licensor that is not subject to the licensing agreement;
- the sublicensing of patent and other rights under our collaborative development relationships;
- our diligence obligations under the license agreement and what activities satisfy those diligence obligations;
- the ownership of inventions and know-how resulting from the joint creation or use of intellectual property by our licensors and us and our partners; and
- the priority of invention of patented technology.

If disputes over intellectual property that we have licensed prevent or impair our ability to maintain our current licensing arrangements on acceptable terms, we may be unable to successfully develop and commercialize the affected product candidates.

In addition, the agreements under which we currently license intellectual property or technology from third parties are complex, and certain provisions in such agreements may be susceptible to multiple interpretations. The resolution of any contract interpretation disagreement that may arise could narrow what we believe to be the scope of our rights to the relevant intellectual property or technology, or increase what we believe to be our financial or other obligations under the relevant agreement, either of which could have a material adverse effect on our business, financial condition, results of operations, and prospects. If disputes over intellectual property that we have licensed prevent or impair our ability to maintain our current licensing arrangements on acceptable terms, we may be unable to successfully develop and commercialize the affected product candidates, which could have a material adverse effect on our business, financial conditions, results of operations, and prospects. As an example, TxCell has exclusively licensed the right to the CAR for use in TX-200 from the University of British Columbia, or UBC. Should UBC terminate this license agreement, we may have to develop or acquire the appropriate CAR which would extend our anticipated development timeline and add expense, and which could result in our failure to realize the anticipated benefits of the TxCell Acquisition.

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

Competitors may infringe our patents or the patents of our licensors. To counter infringement or unauthorized use, we may be required to file infringement claims, which can be expensive and time-consuming. In addition, in an infringement proceeding, a court may decide that a patent of ours or our licensors is not valid, is unenforceable, in whole or in part, or may refuse to stop the other party from using the technology at issue on the grounds that our patents do not cover the technology in question. An adverse result in any litigation or defense proceedings could put one or more of our patents at risk of being invalidated, held unenforceable or interpreted narrowly and could put our patent applications at risk of not issuing. Moreover, if we or one of our licensing partners initiated legal proceedings against a third party to enforce a patent covering one of our product candidates, the defendant could counterclaim that the patent covering our product candidate is invalid and/or unenforceable. In patent litigation in the United States, defendant counterclaims alleging invalidity and/or unenforceability are commonplace. Grounds for a validity challenge could be an alleged failure to meet any of several statutory requirements, including lack of novelty, obviousness or non-enablement. Grounds for an unenforceability assertion could be an allegation that someone connected with prosecution of the patent withheld relevant information from the U.S. PTO, or made a misleading statement, during prosecution. Third parties may also raise similar claims before administrative bodies in the United States or abroad, even outside the context of litigation. Such mechanisms include re-examination, post grant review, *inter partes* review and equivalent proceedings in foreign jurisdictions (e.g., opposition proceedings). Such proceedings could result in revocation or amendment to our patents in such a way that they no longer cover our product candidate. For example, TxCell has exclusively licensed the rights to technology related to redirected Treg cells from the Yeda Research and Development Company, or Yeda. A patent included in this exclusive license agreement with Yeda was granted in Europe in July 2016. Subsequent to this grant, the patent was opposed by several parties in May 2017 and revoked in November 2018. The outcome following legal assertions of invalidity and

unenforceability is unpredictable. With respect to the validity question, for example, we cannot be certain that there is no invalidating prior art, of which we and the patent examiner were unaware during prosecution. If a defendant were to prevail on a legal assertion of invalidity and/or unenforceability, we would lose at least part, and perhaps all, of the patent protection on our product candidates. Such a loss of patent protection could have a material adverse impact on our business.

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

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

***We may be subject to claims that our employees, consultants or independent contractors have wrongfully used or disclosed confidential information of third parties or that our employees have wrongfully used or disclosed alleged trade secrets of their former employers.***

We employ individuals who were previously employed at universities or other biotechnology or pharmaceutical companies, including our competitors or potential competitors. Although we try to ensure that our employees, consultants and independent contractors do not use the proprietary information or know-how of others in their work for us, we may be subject to claims that we or our employees, consultants or independent contractors have inadvertently or otherwise used or disclosed intellectual property, including trade secrets or other proprietary information, of any of our employee's former employer or other third parties. Litigation may be necessary to defend against these claims. If we fail in defending any such claims, in addition to paying monetary damages, we may lose valuable intellectual property rights or personnel, which could adversely impact our business. Even if we are successful in defending against such claims, litigation could result in substantial costs and be a distraction to management and other employees.

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

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

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

Periodic maintenance fees, renewal fees, annuity fees and various other governmental fees on patents and/or applications will be due to be paid to the U.S. PTO and various governmental patent agencies outside of the United States in several stages over the lifetime of the patents and/or applications. We have systems in place to remind us to pay these fees, and we employ an outside firm and rely on our outside counsel to pay these fees due to non-U.S. patent agencies. The U.S. PTO and various non-U.S. governmental patent agencies require compliance with a number of procedural, documentary, fee payment and other similar provisions during the patent application process. We employ professionals to help us comply, and in many cases, an inadvertent lapse can be cured by payment of a late fee or by other means in accordance with the applicable rules. However, there are situations in which non-compliance can result in abandonment or lapse of the patent or patent application, resulting in partial or complete loss of patent rights in the relevant jurisdiction. In such an event, our competitors might be able to enter the market and this circumstance would have a material adverse effect on our business.

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

As is the case with other biotechnology companies, our success is heavily dependent on intellectual property, particularly patents. Obtaining and enforcing patents in the biotechnology industry involve both technological and legal complexity, and is therefore costly, time-consuming and inherently uncertain. Changes in either the patent laws or interpretation of the patent laws in the United States or in other jurisdictions in which we seek patent protection could increase the uncertainties and costs surrounding the prosecution of patent applications and the enforcement or defense of issued patents. The United States enacted the Leahy-Smith America Invents Act, or the America Invents Act, which includes a number of significant changes that affect the way patent applications will be prosecuted and also may affect patent litigation. These include allowing third party submission of prior art to the U.S. PTO during patent prosecution and additional procedures to attack the validity of a patent by U.S. PTO administered post-grant proceedings, including post-grant review, *inter partes* review, and derivation proceedings. Because of a lower evidentiary standard in U.S. PTO proceedings compared to the evidentiary standard in United States federal courts necessary to invalidate a patent claim, a third party could potentially provide evidence in a U.S. PTO proceeding sufficient for the U.S. PTO to hold a claim invalid even though the same evidence would be insufficient to invalidate the claim if first presented in a district court action. In addition, the challenged patents are not accorded the presumption of validity as they are in Federal District Court. Accordingly, a third party may attempt to use the U.S. PTO procedures to invalidate our patent claims that would not have been invalidated if first challenged by the third party as a defendant in a district court action. Therefore, the America Invents Act and its implementation could increase the uncertainties and costs surrounding the prosecution of our owned or in-licensed patent applications and the enforcement or defense of our owned or in-licensed issued patents, all of which could have a material adverse effect on our business, financial condition, results of operations, and prospects. Moreover, recent U.S. Supreme Court rulings have narrowed the scope of patent protection available in certain circumstances and weakened the rights of patent owners in certain situations. In addition to increasing uncertainty with regard to our ability to obtain patents in the future, this combination of events has created uncertainty with respect to the value of patents, once obtained. Depending on decisions by the U.S. Congress, the federal courts, the U.S. PTO, and similar legislative, judicial and regulatory bodies in other jurisdictions, the laws and regulations governing patents could change in unpredictable ways that would weaken our ability to obtain new patents or to enforce our existing patents and patents that we might obtain in the future.

***We may not be able to protect our intellectual property rights throughout the world.***

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

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

**Risks Relating to our Business Operations**

***Significant disruptions of our information technology systems or data security incidents could result in significant financial, legal, regulatory, business and reputational harm to us.***

We are increasingly dependent on information technology systems and infrastructure, including mobile technologies, to operate our business. In the ordinary course of our business, we collect, store, process and transmit large amounts of sensitive information, including intellectual property, proprietary business information, personal information and other confidential information. It is critical that we do so in a secure manner to maintain the confidentiality, integrity and availability of such

sensitive information. We have also outsourced elements of our operations (including elements of our information technology infrastructure) to third parties, and as a result, we manage a number of third-party vendors who may or could have access to our computer networks or our confidential information. In addition, many of those third parties in turn subcontract or outsource some of their responsibilities to third parties. As a result, our information technology systems, including the functions of third parties that are involved or have access to those systems, is very large and complex. While all information technology operations are inherently vulnerable to inadvertent or intentional security breaches, incidents, attacks and exposures, the size, complexity, accessibility and distributed nature of our information technology systems, and the large amounts of sensitive information stored on those systems, make such systems potentially vulnerable to unintentional or malicious, internal and external attacks on our technology environment. Potential vulnerabilities can be exploited from inadvertent or intentional actions of our employees, third-party vendors, business partners, or by malicious third parties. Attacks of this nature are increasing in their frequency, levels of persistence, sophistication and intensity, and are being conducted by sophisticated and organized groups and individuals with a wide range of motives (including, but not limited to, industrial espionage) and expertise, including organized criminal groups, "hacktivists," nation states and others. In addition to the extraction of sensitive information, such attacks could include the deployment of harmful malware, ransomware, denial-of-service attacks, social engineering and other means to affect service reliability and threaten the confidentiality, integrity and availability of information. In addition, the prevalent use of mobile devices increases the risk of data security incidents.

Significant disruptions of our, our third-party vendors' and/or business partners' information technology systems or other similar data security incidents could adversely affect our business operations and/or result in the loss, misappropriation, and/or unauthorized access, use or disclosure of, or the prevention of access to, sensitive information, which could result in financial, legal, regulatory, business and reputational harm to us. For example, in April 2018, we announced a data security incident involving the compromise of a then senior executive's company email account. Upon learning of the incident on March 28, 2018, external network security experts were promptly engaged, and the incident response team worked diligently to investigate the incident. We also promptly notified federal law enforcement of the incident. The investigation concluded that the incident was limited to the compromise of the then senior executive's company email account for approximately 11 weeks. The investigation did not reveal any evidence that our network or other information technology systems were otherwise compromised in connection with the incident or that the incident resulted in the disclosure of or access to personal information about patients or other individuals besides the holder of the company email account that was affected. However, proprietary, confidential and other sensitive information of ours and that of other entities was accessed and may have been compromised as a result of the incident. Unforeseen developments related to this incident could occur, which could have a further adverse impact on us. We do not maintain cyber liability insurance and will therefore have no coverage for any losses resulting from this data security incident. Any litigation or regulatory review arising from this incident could result in significant legal exposure to us. In addition, information technology system disruptions, whether from attacks on our technology environment or from computer viruses, natural disasters, terrorism, war and telecommunication and electrical failures, could result in a material disruption of our development programs and our business operations. For example, the loss of clinical trial data from completed or future clinical trials could result in delays in our regulatory approval efforts and significantly increase our costs to recover or reproduce the data.

While we aware of the company email incident described above, there is no way of knowing with certainty whether we have experienced any other data security incidents that have not been discovered. While we have no reason to believe this to be the case, attackers have become very sophisticated in the way they conceal access to systems, and many companies that have been attacked are not aware that they have been attacked. Any event, including the company email incident described above, that leads to unauthorized access, use or disclosure of personal information, including but not limited to personal information regarding our patients or employees, could disrupt our business, harm our reputation, compel us to comply with applicable federal and/or state breach notification laws and foreign law equivalents, subject us to time consuming, distracting and expensive litigation, regulatory investigation and oversight, mandatory corrective action, require us to verify the correctness of database contents, or otherwise subject us to liability under laws, regulations and contractual obligations, including those that protect the privacy and security of personal information. This could result in increased costs to us, and result in significant legal and financial exposure and/or reputational harm. In addition, any failure or perceived failure by us or our vendors or business partners to comply with our privacy, confidentiality or data security-related legal or other obligations to third parties, or any further security incidents or other inappropriate access events that result in the unauthorized access, release or transfer of sensitive information, which could include personally identifiable information, may result in governmental investigations, enforcement actions, regulatory fines, litigation, or public statements against us by advocacy groups or others, and could cause third parties, including clinical sites, regulators or current and potential partners, to lose trust in us or we could be subject to claims by third parties that we have breached our privacy- or confidentiality-related obligations, which could materially and adversely affect our business and prospects. In addition, failure to maintain effective internal accounting controls related to security breaches and cybersecurity in general could impact our ability to produce timely and accurate financial statements and subject us to regulatory scrutiny. Moreover, data security incidents and other inappropriate access can be difficult to detect, and any delay in identifying them may lead to increased harm of the type described above. While we have implemented security measures intended to protect our information

technology systems and infrastructure, there can be no assurance that such measures will successfully prevent service interruptions or further security incidents.

***We may not realize the anticipated benefits of the TxCell Acquisition or be able to successfully integrate the acquired TxCell operations.***

The TxCell Acquisition involves numerous uncertainties and risks, and has required, and will continue to require, significant efforts and expenditures, including with respect to integrating the acquired TxCell operations with our operations. We may not be able to accomplish this integration process smoothly or successfully. The integration of certain of the acquired TxCell operations will take time and will require the dedication of significant management resources, which may temporarily distract our management's attention from the routine business of the combined company. In any event, we may encounter unexpected difficulties, or incur unexpected costs, in connection with our transition activities and integration efforts, which include:

- the potential disruption of our historical core business;
- the risk that our relative lack of historical experience in CAR-Treg development and developing product candidates and technology for immunological diseases will not allow us to advance the development of CAR-Treg therapies, including TX-200, on the timeframes we expect, or at all;
- the strain on, and need to continue to expand, our existing operational, technical, financial and administrative infrastructure;
- the difficulties in assimilating employees and corporate cultures;
- the difficulties in effectively managing transition and integration activities given the distance between our headquarters and U.S.-based management team and TxCell's offices in France;
- the failure to retain key managers and other personnel, including the employees from the acquired TxCell business who might experience uncertainty about their future roles with us;
- the challenges in controlling additional costs and expenses in connection with and as a result of the TxCell Acquisition;
- the diversion of our management's attention to integration of operations and corporate and administrative infrastructures; and
- any unanticipated liabilities for activities of or related to TxCell or its operations, technologies or product candidates.

If any of these factors impairs our ability to integrate successfully, we may be required to spend time or money on integration activities that otherwise would be spent on the development and expansion of our business. If we fail to integrate or otherwise manage the acquired TxCell business successfully and in a timely manner, the combined company's potential to achieve the anticipated long-term strategic benefits of the TxCell Acquisition could be compromised and resulting operating inefficiencies could increase costs and expenses more than we planned, could negatively impact the market price of our common stock and could otherwise distract us from execution of our strategy. Failure to maintain effective financial controls and reporting systems and procedures could also adversely affect our ability to produce timely and accurate financial statements. In addition, while we intend to avail ourselves of the French tax credit for certain research and development related expenses, we may not receive the anticipated amount and we may also be required to make corrective actions upon any audit by the French tax authority with respect to such tax credit.

In any event, there can be no assurance that we will integrate or otherwise manage the acquired TxCell business successfully or otherwise do so without experiencing operating inefficiencies or control deficiencies. In addition, because the historical business operations of TxCell differ from our historical business operations, and the combined company has a different business mix than our historical business, we face different operational risks and challenges and the complexity of our company has increased. Significant management time and effort is required to effectively manage the increased complexity of our company following the TxCell Acquisition, and our failure to successfully do so could have a material adverse effect on our business, financial condition, results of operations and growth prospects.

***If we are unable to acquire 100% of the equity interests of TxCell, our business, financial condition and results of operations could be adversely affected.***

Although we completed the TxCell Acquisition, we may not be able to acquire the remaining ordinary shares of TxCell for some period of time, if ever. As of February 15, 2019, we have acquired a total of 25,047,671 ordinary shares of TxCell, representing approximately 98.2% of the outstanding share capital and voting rights of TxCell. Until such time, if ever, that we acquire 100% of the equity interests of TxCell, we will need to consider the rights of, and duties owed to, the minority shareholders of TxCell under French law when making future decisions that might impact TxCell, its business or its operations, which could adversely affect our business and our ability to realize the anticipated benefits of the TxCell Acquisition.

***We plan to continue to operate the acquired TxCell business in France, which may expose us to unanticipated costs or events.***

TxCell's historical operations have been based in France and we plan to continue to operate the acquired TxCell business in France. Our operation of the acquired TxCell business in France involves significant risks, including:

- difficulty hiring and retaining appropriate personnel due to intense competition for such limited resources;
- disruptions in relations with our employees, including legacy TxCell employees; and
- compliance with regulatory requirements, including local French employment regulations and organized labor in France.

In addition, as a result of our operations in France, we have become more exposed to fluctuations in currency exchange rates between the Euro and the U.S. dollar. Given the volatility of currency exchange rates, there is no assurance that we will be able to effectively manage currency transaction and/or conversion risks. To date, we have not entered into derivative instruments to offset the impact of foreign exchange fluctuations, which fluctuations could have a material adverse effect on our financial condition and results of operations. In any event, difficulties resulting from these and other risks related to our anticipated operations in France could expose us to increased expenses, impair our development efforts, adversely affect our financial condition and results of operations, and harm our competitive position.

***We are also exposed to general risks associated with our operations outside of the United States, which could adversely affect our business.***

In addition to our French operations as a result of the TxCell Acquisition, we also have operations and conduct business in other countries outside the United States, and have a UK subsidiary. We may plan to expand these activities or in to additional countries in the future. Consequently, we are, and will continue to be, subject to risks inherent with operating in foreign countries, in addition to those specific risks associated with TxCell, which include:

- the increased complexity and costs inherent in managing international operations, including in geographically disparate locations;
- diverse regulatory, financial and legal requirements, and any future changes to such requirements, in one or more countries where we are located or do business;
- differing payor reimbursement regimes, governmental payors or patient self-pay systems and price controls;
- adverse tax consequences, including changes in applicable tax laws and regulations;
- applicable trade laws, tariffs, export quotas, custom duties or other trade restrictions, and any changes to them;
- economic weakness, including inflation, or political or economic instability in particular foreign economies and markets;
- compliance with tax, employment, immigration and labor laws for employees living or traveling abroad;
- foreign currency fluctuations, which could result in increased operating expenses or reduced revenues, and other obligations incident to doing business or operating in another country;
- liabilities for activities of, or related to, our international operations;
- challenges inherent in efficiently managing employees in diverse geographies, including the need to adapt systems, policies, benefits and compliance programs to differing labor and other regulations;
- workforce uncertainty in countries where labor unrest is more common than in the United States; and
- laws and regulations relating to data security and the unauthorized use of, or access to, commercial and personal information.

***The expected withdrawal of the United Kingdom from the European Union, commonly referred to as "Brexit," may adversely impact our ability to obtain regulatory approvals of our product candidates in the European Union and may require us to incur additional expenses in order to develop, manufacture and commercialize our product candidates in the European Union.***

In June 2016, UK voters approved a referendum to withdraw from the European Union, commonly referred to as "Brexit." Pursuant to Article 50 of the Treaty on European Union, the United Kingdom will cease to be a European Union Member State either on the effective date of a withdrawal agreement (which requires UK parliamentary approval) or, failing that, two years following the United Kingdom's notification of its intention to leave the European Union, unless extended. Although the United Kingdom formally notified the European Council of its intention to leave the European Union in March 2017,several extensions have been granted (most recently until October 31, 2019). Given that no formal withdrawal agreements have been agreed and there have been several extensions granted, the United Kingdom has yet to formally leave the European Union, and it is uncertain as to when it will occur, if ever.

Brexit could adversely affect European or worldwide political, regulatory, economic or market conditions and could contribute to instability in global political institutions, regulatory agencies and financial markets. If any of these outcomes occur, we may be forced to restrict or delay efforts to seek regulatory approval in the United Kingdom or the European Union for our

product candidates, or incur significant additional expenses to operate our business, which could significantly and materially harm or delay our ability to generate revenues or achieve profitability of our business. Because a significant proportion of the regulatory framework in the United Kingdom applicable to our business and our product candidates is derived from European Union directives and regulations, Brexit could materially impact the regulatory regime with respect to the development, manufacture, importation, approval and commercialization of our product candidates in the United Kingdom or the European Union. In the near term, there is a risk of disrupted import and export processes due to a lack of administrative processing capacity by the respective UK and European Union customs agencies that may delay time-sensitive shipments and may negatively impact our product supply chain. Any further changes in international trade, tariff and import/export regulations as a result of Brexit or otherwise may impose unexpected duty costs or other non-tariff barriers on us. It is also possible that Brexit may negatively affect our ability to attract and retain employees, particularly those from the European Union.

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

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

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

Our success depends on our continued ability to attract, retain, and motivate highly qualified management and scientific personnel and our ability to develop and maintain important relationships with leading research and academic institutions and scientists. Competition for skilled and qualified personnel and academic and other research collaborations is intense. If we lose the services of personnel with the necessary skills, including the members of our senior management team, it could significantly impede the achievement of our research and development objectives. In addition, we expect to rely on the experience and expertise of TxCell's historical management team and other key personnel in the development of TX-200 and potential future CAR-Treg therapies. If we were to lose the services of a significant portion or key individuals of this team, such development and our business could be adversely affected. Moreover, if we fail to negotiate additional acceptable collaborations with academic and other research institutions and scientists, or if our existing collaborations are unsuccessful, our development programs may be delayed or may not succeed.

***Third parties on which we rely and we may be adversely affected by natural disasters and our business continuity and disaster recovery plans may not adequately protect us from a serious disaster.***

Natural disasters could severely disrupt our operations and have a material adverse effect on our business, financial condition, results of operations and prospects. If a natural disaster, power outage or other event occurred that prevented us from using all or a significant portion of our headquarters, that damaged critical infrastructure or that otherwise disrupted operations, it may be difficult or, in certain cases, impossible for us to continue our business for a substantial period of time. The disaster recovery and business continuity plans we have in place currently are limited and may not prove adequate in the event of a serious disaster or similar event. We may incur substantial expenses as a result of the limited nature of our disaster recovery and business continuity plans, which could have a material adverse effect on our business, financial condition, results of operations and prospects.

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

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

Our stock price has been volatile and may continue to be volatile, which could cause stockholders to incur substantial losses. An active public market for our common stock may not be sustained, and the market price of our common stock may continue to be highly volatile. The market price of our common stock has fluctuated significantly in response to various factors, some of which are beyond our control, including but not limited to the following:

- announcements by us or collaborators providing updates on the progress or development status of product candidates;
- data from clinical trials;

- initiation or termination of clinical trials;
- changes in market valuations of similar companies;
- overall market and economic conditions, including the equity markets for emerging biotechnology companies;
- deviations in our results of operations from the guidance given by us;
- announcements by us or our competitors of new or enhanced products, technologies or services or significant contracts, acquisitions, strategic relationships, joint ventures or capital commitments;
- announcement of changes in business and operations by our collaborators and partners, or changes in our existing collaboration agreements;
- regulatory developments;
- changes, by one or more of our security analysts, in recommendations, ratings or coverage of our stock;
- additions or departures of key personnel;
- future sales of our common stock or other securities by us, management or directors, liquidation of institutional funds that comprised large holdings of our stock; and decreases in our cash balances.

***Actual or potential sales of our common stock by our employees, including our executive officers, pursuant to pre-arranged stock trading plans could cause our stock price to fall or prevent it from increasing for numerous reasons, and actual or potential sales by such persons could be viewed negatively by other investors.***

In accordance with the guidelines specified under Rule 10b5-1 of the Securities Exchange Act of 1934, as amended, and our policies regarding stock transactions, a number of our employees, including executive officers and members of our board of directors, have adopted and may continue to adopt stock trading plans pursuant to which they have arranged to sell shares of our common stock from time to time in the future. Generally, sales under such plans by our executive officers and directors require public filings. Actual or potential sales of our common stock by such persons could cause the price of our common stock to fall or prevent it from increasing for numerous reasons.

***Future sales and issuances of our common stock or rights to purchase common stock, including pursuant to our equity incentive plans, could result in additional dilution of the percentage ownership of our stockholders and could cause our stock price to fall.***

Additional capital will be needed in the future to continue our planned operations. To the extent we raise additional capital by issuing equity securities, our stockholders may experience substantial dilution. We may sell common stock, convertible securities or other equity securities in one or more transactions at prices and in a manner we determine from time to time. If we sell common stock, convertible securities or other equity securities in more than one transaction, investors may be materially diluted by subsequent sales. These sales may also result in material dilution to our existing stockholders, and new investors could gain rights superior to our existing stockholders.

***Our stock price is also influenced by public perception of gene therapy and government regulation of potential products.***

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

***If securities or industry analysts do not publish research or publish inaccurate or unfavorable research about our business, our stock price and trading volume could decline.***

The trading market for our common stock will depend in part on the research and reports that securities or industry analysts publish about us or our business. In the event securities or industry analysts who cover us downgrade our stock or publish inaccurate or unfavorable research about our business, our stock price would likely decline. If one or more of these analysts cease coverage of our company or fail to publish reports on us regularly, demand for our stock could decrease, which might cause our stock price and trading volume to decline.

***We do not intend to pay dividends on our common stock so any returns will be limited to the value of our stock.***

We have never declared or paid any cash dividends on our common stock. We currently anticipate that we will retain future earnings for the development, operation and expansion of our business and do not anticipate declaring or paying any cash dividends for the foreseeable future. Any return to stockholders will therefore be limited to the appreciation of their stock.

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

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

Similarly, our authorized but unissued common stock is available for future issuance without stockholder approval. Our certificate of incorporation further provides that stockholders may not take action by written consent.

In addition, our amended and restated bylaws:

- establish advance notice requirements for nominations for election to the board of directors or proposing matters that can be acted upon at stockholders' meetings; and
- prohibit stockholders from calling a special meeting of stockholders.

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

***Our amended and restated bylaws provide that a state or federal court located within the State of Delaware will be the exclusive forum for the adjudication of certain disputes, which could limit our stockholders' ability to obtain a favorable judicial forum for disputes with us or our directors, officers, or employees.***

Our amended and restated bylaws provide that a state or federal court located within the State of Delaware is the sole and exclusive forum for:

- any derivative action or proceeding brought on our behalf;
- any action asserting a claim of breach of a fiduciary duty owed by any director, officer or other employee or stockholder of Sangamo to us or our stockholders;
- any action asserting a claim arising pursuant to any provision of the General Corporation Law of the State of Delaware, our charter or our bylaws, as to which the General Corporation Law of the State of Delaware confers jurisdiction on the Court of Chancery of the State of Delaware; and
- any action asserting a claim governed by the internal affairs doctrine.

This provision further provides that any person or entity that acquires any interest in shares of our capital stock will be deemed to have notice of and consented to the provisions of such provision.

This provision may limit a stockholder's ability to bring a claim in a judicial forum that it finds favorable for disputes with us or our directors, officers, or other employees, which may discourage lawsuits against us and our directors, officers, and other employees. If a court were to find this provision to be inapplicable or unenforceable in an action, we may incur additional costs associated with resolving the dispute in other jurisdictions, which could seriously harm our business.

## ITEM 2. UNREGISTERED SALES OF EQUITY SECURITIES AND USE OF PROCEEDS

None.

## ITEM 3. DEFAULTS UPON SENIOR SECURITIES

Not applicable.

**ITEM 4. MINE SAFEY DISCLOSURES**

Not applicable.

**ITEM 5. OTHER INFORMATION**

None.

**ITEM 6. EXHIBITS**

(a) Exhibits:

| | | |
|---|---|---|
| 3.1 | | Composite copy of Seventh Amended and Restated Certificate of Incorporation of Sangamo Therapeutics, Inc., as amended (incorporated by reference to Exhibit 3.1 to the Registrant's Quarterly Report on Form 10-Q (File No. 000-30171), filed with the SEC on August 9, 2017). |
| 3.2 | | Third Amended and Restated Bylaws of Sangamo Therapeutics, Inc. (incorporated by reference to Exhibit 3.1 to the Registrant's Current Report on Form 8-K (File No. 000-30171), filed with the SEC on June 15, 2018). |
| 31.1 | | Rule 13a — 14(a) Certification of Principal Executive Officer. |
| 31.2 | | Rule 13a — 14(a) Certification of Principal Financial Officer. |
| 32.1 | * | Certifications Pursuant to 18 U.S.C. Section 1350. |
| 101.INS | | XBRL Instance Document - the instance document does not appear in the Interactive Data File because its XBRL tags are embedded within the Inline XBRL document. |
| 101.SCH | | XBRL Taxonomy Extension Schema Document |
| 101.CAL | | XBRL Taxonomy Extension Calculation Linkbase Document |
| 101.DEF | | XBRL Taxonomy Extension Definition Linkbase Document |
| 101.LAB | | XBRL Taxonomy Extension Label Linkbase Document |
| 101.PRE | | XBRL Taxonomy Extension Presentation Linkbase Document |
| 104 | | The cover page from Sangamo's Quarterly Report on Form 10-Q for the three months ended June 30, 2019, formatted in Inline XBRL. |

* The certifications attached as Exhibit 32.1 accompany this Quarterly Report on Form 10-Q pursuant to 18 U.S.C. Section 1350, as adopted pursuant to Section 906 of the Sarbanes-Oxley Act of 2002, and shall not be deemed "filed" by the Registrant for purposes of Section 18 of the Securities Exchange Act of 1934, as amended.

**SIGNATURES**

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

Dated: August 7, 2019

SANGAMO THERAPEUTICS, INC.

/s/ ALEXANDER D. MACRAE

**Alexander D. Macrae**
**President and Chief Executive Officer**
**(Principal Executive Officer)**

/s/ PRATHYUSHA DURAIBABU

**Prathyusha Duraibabu**
**Vice President, Finance**
**(Principal Accounting Officer)**

**Exhibit 31.1**

**CERTIFICATION**

I, Alexander D. Macrae, certify that:

1. I have reviewed this quarterly report on Form 10-Q of Sangamo Therapeutics, Inc.;

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

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

4. The registrant's other certifying officer(s) and I are responsible for establishing and maintaining disclosure controls and procedures (as defined in Exchange Act Rules 13a-15(e) and 15d-15(e)) and internal control over financial reporting (as defined in Exchange Act Rules 13a-15(f) and 15d-15(f)) for the registrant and have:

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

   (b) Designed such internal control over financial reporting or caused such internal control over financial reporting to be designed under our supervision, to provide reasonable assurance regarding the reliability of financial reporting and the preparation of financial statements for external purposes in accordance with generally accepted accounting principles;

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

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

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

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

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

Date: August 7, 2019

/s/ ALEXANDER D. MACRAE

Alexander D. Macrae
President and Chief Executive Officer
(Principal Executive Officer)

**Exhibit 31.2**

**CERTIFICATION**

I, Stéphane Boissel, certify that:

1. I have reviewed this quarterly report on Form 10-Q of Sangamo Therapeutics, Inc.;

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

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

4. The registrant's other certifying officer(s) and I are responsible for establishing and maintaining disclosure controls and procedures (as defined in Exchange Act Rules 13a-15(e) and 15d-15(e)) and internal control over financial reporting (as defined in Exchange Act Rules 13a-15(f) and 15d-15(f)) for the registrant and have:

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

   (b) Designed such internal control over financial reporting or caused such internal control over financial reporting to be designed under our supervision, to provide reasonable assurance regarding the reliability of financial reporting and the preparation of financial statements for external purposes in accordance with generally accepted accounting principles;

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

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

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

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

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

Date: August 7, 2019

/s/ STÉPHANE BOISSEL

Stéphane Boissel

Executive Vice President, Corporate Strategy and Interim Chief Financial Officer

(Principal Financial Officer)

**Exhibit 32.1**

**Certifications Pursuant to 18 U.S.C. §1350, as Adopted**
**Pursuant to §906 of the Sarbanes-Oxley Act of 2002**

Pursuant to the requirement set forth in Rule 13a-14(b) of the Securities Exchange Act of 1934, as amended, (the "Exchange Act") and Section 1350 of Chapter 63 of Title 18 of the United States Code (18 U.S.C. §1350), each of the undersigned hereby certifies in his capacity as an officer of Sangamo Therapeutics, Inc. (the "Company"), that, to the best of his knowledge:

(1) the Company's Quarterly Report on Form 10-Q for the period ended June 30, 2019, to which this Certification is attached as Exhibit 32.1 (the "Report") fully complies with the requirements of Section 13(a) or Section 15(d) of the Exchange Act; and

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

/s/ ALEXANDER D. MACRAE

Alexander D. Macrae
President and Chief Executive Officer
(Principal Executive Officer)

Date: August 7, 2019

/s/ STÉPHANE BOISSEL

Stéphane Boissel
Executive Vice President, Corporate Strategy and Interim Chief Financial Officer
(Principal Financial Officer)

Date: August 7, 2019

*This certification accompanies the Quarterly Report on Form 10-Q to which it relates, is not deemed filed with the Securities and Exchange Commission and is not to be incorporated by reference into any filing of Sangamo Therapeutics, Inc. under the Securities Act of 1933, as amended, or the Exchange Act (whether made before or after the date of the Form 10-Q), irrespective of any general incorporation language contained in such filing. A signed original of this written statement required by Section 906 of the Sarbanes-Oxley Act of 2002 has been provided to Sangamo Therapeutics, Inc. and will be retained by Sangamo Therapeutics, Inc. and furnished to the Securities and Exchange Commission or its staff upon request.*